

IDERA PHARMACEUTICALS, INC.

Form 10-Q

May 09, 2016

Table of Contents

**UNITED STATES**

**SECURITIES AND EXCHANGE COMMISSION**

**Washington, D.C. 20549**

**FORM 10-Q**

x **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 transition period from \_\_\_\_\_ to \_\_\_\_\_.**

**Commission File Number: 001-31918**

**IDERA PHARMACEUTICALS, INC.**

**(Exact name of registrant as specified in its charter)**

**Delaware**  
**(State or other jurisdiction of**  
**incorporation or organization)**

**04-3072298**  
**(I.R.S. Employer**  
**Identification No.)**

**167 Sidney Street**

**Cambridge, Massachusetts**  
**(Address of principal executive offices)**

**02139**  
**(Zip code)**

**(617) 679-5500**

**(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. (Check one):

Large accelerated filer  Accelerated filer

Non-accelerated filer  (Do not check if a smaller reporting company) 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

**Common Stock, par value \$.001 per share**  
**Class**

**121,315,950**  
**Outstanding as of April 15, 2016**

**Table of Contents**

**IDERA PHARMACEUTICALS, INC.**

**FORM 10-Q**

**INDEX**

	<b>Page</b>
<b><u>PART I FINANCIAL INFORMATION</u></b>	
<u>Item 1 Financial Statements Unaudited</u>	1
<u>Condensed Balance Sheets as of March 31, 2016 and December 31, 2015</u>	1
<u>Condensed Statements of Operations and Comprehensive Loss for the Three Months Ended March 31, 2016 and 2015</u>	2
<u>Condensed Statements of Cash Flows for the Three Months Ended March 31, 2016 and 2015</u>	3
<u>Notes to Condensed Financial Statements</u>	4
<u>Item 2 Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	12
<u>Item 3 Quantitative and Qualitative Disclosures About Market Risk</u>	20
<u>Item 4 Controls and Procedures</u>	20
<b><u>PART II OTHER INFORMATION</u></b>	
<u>Item 1A Risk Factors</u>	22
<u>Item 6 Exhibits</u>	44
<u>Signatures</u>	45

IMO<sup>®</sup> and Idera<sup>®</sup> are our trademarks. All other trademarks and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners.

**Table of Contents**

**FORWARD-LOOKING STATEMENTS**

This Quarterly Report on Form 10-Q contains 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, or the Exchange Act. All statements, other than statements of historical fact, included or incorporated in this report regarding our strategy, future operations, clinical trials, collaborations, intellectual property, cash resources, financial position, future revenues, projected costs, prospects, plans, and objectives of management are forward-looking statements. The words believes, anticipates, estimates, plans, expects, intends, may, could, should, potential, will, and would and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We cannot guarantee that we actually will achieve the plans, intentions or expectations disclosed in our forward-looking statements and you should not place undue reliance on our forward-looking statements. There are a number of important factors that could cause our actual results to differ materially from those indicated or implied by forward-looking statements. These important factors include those set forth below under Part II, Item 1A Risk Factors. These factors and the other cautionary statements made in this Quarterly Report on Form 10-Q should be read as being applicable to all related forward-looking statements whenever they appear in this Quarterly Report on Form 10-Q. In addition, any forward-looking statements represent our estimates only as of the date that this Quarterly Report on Form 10-Q is filed with the Securities and Exchange Commission, or the SEC, and should not be relied upon as representing our estimates as of any subsequent date. We do not assume any obligation to update any forward-looking statements. We disclaim any intention or obligation to update or revise any forward-looking statement, whether as a result of new information, future events or otherwise.

**Table of Contents****PART I FINANCIAL INFORMATION****ITEM 1. FINANCIAL STATEMENTS.****IDERA PHARMACEUTICALS, INC.****CONDENSED BALANCE SHEETS****(UNAUDITED)**

<b>(In thousands, except per share amounts)</b>	<b>March 31, 2016</b>	<b>December 31, 2015</b>
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 21,879	\$ 26,586
Short-term investments	40,511	33,574
Prepaid expenses and other current assets	3,129	3,082
Total current assets	65,519	63,242
Long-term investments	11,732	26,997
Property and equipment, net	1,727	1,692
Restricted cash and other assets	342	345
Total assets	\$ 79,320	\$ 92,276
<b>LIABILITIES AND STOCKHOLDERS EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 958	\$ 1,169
Accrued expenses	2,797	4,274
Current portion of note payable	269	261
Current portion of deferred revenue	1,111	1,111
Total current liabilities	5,135	6,815
Deferred revenue, net of current portion	985	1,262
Note payable, net of current portion	431	501
Other liabilities	91	116
Total liabilities	6,642	8,694
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.01 par value, Authorized 5,000 shares		
Series A convertible preferred stock, Designated 1,500 shares; Issued and outstanding 1 share		
Common stock, \$0.001 par value, Authorized 280,000 shares; Issued and outstanding 121,300 and 121,265 shares at March 31, 2016 and December 31, 2015, respectively	121	121

Edgar Filing: IDERA PHARMACEUTICALS, INC. - Form 10-Q

Additional paid-in capital	585,461	583,676
Accumulated deficit	(512,904)	(500,081)
Accumulated other comprehensive income (loss)		(134)
Total stockholders' equity	72,678	83,582
Total liabilities and stockholders' equity	\$ 79,320	\$ 92,276

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

Table of Contents

**IDERA PHARMACEUTICALS, INC.**  
**CONDENSED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**  
**(UNAUDITED)**

<b>(In thousands, except per share amounts)</b>	<b>Three Months Ended March 31</b>	
	<b>2016</b>	<b>2015</b>
Alliance revenue	\$ 294	\$ 34
Operating expenses:		
Research and development	9,296	8,720
General and administrative	3,916	3,837
Total operating expenses	13,212	12,557
Loss from operations	(12,918)	(12,523)
Other income (expense):		
Interest income	120	41
Interest expense	(23)	(27)
Foreign currency exchange (loss) gain	(2)	28
Net loss	\$ (12,823)	\$ (12,481)
Basic and diluted net loss per common share (Note 13)	\$ (0.11)	\$ (0.12)
Shares used in computing basic and diluted net loss per common share	121,284	105,067
Net loss	\$ (12,823)	\$ (12,481)
Other comprehensive income:		
Unrealized gain on available-for-sale securities	134	18
Other comprehensive income	134	18
Comprehensive loss	\$ (12,689)	\$ (12,463)

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

Table of Contents

**IDERA PHARMACEUTICALS, INC.**  
**CONDENSED STATEMENTS OF CASH FLOWS**  
**(UNAUDITED)**

(In thousands)	<b>Three Months Ended March 31,</b>	
	<b>2016</b>	<b>2015</b>
<b>Cash Flows from Operating Activities:</b>		
Net loss	\$ (12,823)	\$ (12,481)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	1,716	1,346
Depreciation and amortization expense	157	101
Accretion of premiums and discounts on investments	195	86
Issuance of common stock for services rendered	32	19
Non-employee stock option expense		240
Gain on sale of assets	3	
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(46)	(532)
Accounts payable, accrued expenses, and other liabilities	(1,812)	(1,488)
Deferred revenue	(277)	
Net cash used in operating activities	(12,855)	(12,709)
<b>Cash Flows from Investing Activities:</b>		
Purchases of available-for-sale securities	(2,946)	(3,674)
Proceeds from maturity of available-for-sale securities	9,239	6,602
Proceeds from sale of available-for-sale securities	1,974	999
Purchases of property and equipment	(90)	(105)
Net cash provided by investing activities	8,177	3,822
<b>Cash Flows from Financing Activities:</b>		
Proceeds from equity financings, net of issuance costs		81,070
Proceeds from exercise of common stock options and employee stock purchases	36	121
Payments on note payable	(63)	
Payments on capital lease	(2)	(2)
Net cash (used in) provided by financing activities	(29)	81,189
Net (decrease) increase in cash and cash equivalents	(4,707)	72,302
Cash and cash equivalents, beginning of period	26,586	19,971
Cash and cash equivalents, end of period	\$ 21,879	\$ 92,273



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

**Table of Contents**

**IDERA PHARMACEUTICALS, INC.**

**NOTES TO CONDENSED FINANCIAL STATEMENTS**

March 31, 2016

**(UNAUDITED)**

(1) Organization

Idera Pharmaceuticals, Inc. ( Idera or the Company ) is a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics for oncology and rare diseases. The Company uses two distinct proprietary drug discovery technology platforms to design and develop drug candidates: its Toll-like receptor ( TLR ) targeting technology and its third-generation antisense ( 3GA ) technology. The Company developed these platforms based on its scientific expertise and pioneering work with synthetic oligonucleotides as therapeutic agents. Using its TLR targeting technology, the Company designs synthetic oligonucleotide-based drug candidates to modulate the activity of specific TLRs. In addition, using its 3GA technology, the Company is developing drug candidates to turn off the messenger RNA, or mRNA, associated with disease causing genes. The Company believes that its 3GA technology may potentially reduce the immunotoxicity and increase the potency of earlier generation antisense and RNA interference, or RNAi, technologies.

The Company is focused on the development of drug candidates for oncology and rare diseases characterized by small, well-defined patient populations with serious unmet medical needs. The Company plans to explore potential collaborative alliances to support late-stage development and commercialization of some of its drug candidates.

The Company is developing IMO-8400, an antagonist of TLR7, TLR8 and TLR9, for the treatment of certain B-cell lymphomas in which the MYD88 L265P oncogenic mutation is present. Oncogenic mutations are changes in the DNA of tumor cells that promote the survival and proliferation of tumor cells. MYD88 is an adaptor protein in the TLR signaling pathway that mediates TLR signaling. The Company's pipeline of drug candidates includes IMO-2125, a TLR9 agonist that may have potential applications as an immune therapy for the treatment of cancer. The Company has selected dermatomyositis as the first non-cancer rare disease for application of IMO-8400. The Company selected this indication for development based on the reported role of TLRs in the pathogenesis of the disease state, clinical feasibility, including ease of patient identification and availability of endpoints for regulatory approval, and commercial potential.

At March 31, 2016, the Company had an accumulated deficit of \$512,904,000. The Company expects to incur substantial operating losses in future periods. The Company does not expect to generate significant product revenue, sales-based milestones or royalties until the Company successfully completes development and obtains marketing approval for drug candidates, either alone or in collaborations with third parties, which the Company expects will take a number of years. In order to commercialize its drug candidates, the Company needs to complete clinical development and comply with comprehensive regulatory requirements.

The Company is subject to a number of risks and uncertainties similar to those of other companies of the same size within the biotechnology industry, such as uncertainty of clinical trial outcomes, uncertainty of additional funding, and history of operating losses.

(2) New Accounting Pronouncements Recently Issued

In May 2014, the Financial Accounting Standards Board (the FASB ) issued Accounting Standards Update ( ASU ) No. 2014-09, Revenue from Contracts with Customers (Topic 606), which was amended by ASU No. 2015-14. ASU No. 2014-09, as amended by ASU No. 2015-14, requires an entity to recognize revenue from the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. In particular, this ASU addresses contracts with more than one performance obligation, as well as the accounting for some costs to obtain or fulfill a contract with a customer, and provides for additional disclosures with respect to revenues and cash flows arising from contracts with customers. This ASU will be effective for fiscal years beginning after December 15, 2017, including interim periods within that fiscal year. Early adoption of this ASU is permitted only for fiscal years beginning after December 15, 2016, including interim periods within that fiscal year. The Company is currently evaluating the effect that the adoption of this ASU will have on its financial statements.

In August 2014, the FASB issued ASU No. 2014-15, Disclosure of Uncertainties about an Entity s Ability to Continue as a Going Concern. ASU 2014-15 amends FASB ASC 205-40, Presentation of Financial Statements Going Concern, by providing guidance on determining when and how reporting entities must disclose going-concern uncertainties in their financial statements, including requiring management to perform interim and annual assessments of an entity s ability to continue as a going concern within

## **Table of Contents**

one year of the date of issuance of the entity's financial statements and providing certain disclosures if there is substantial doubt about the entity's ability to continue as a going concern. ASU 2014-15 will be effective for fiscal years ending after December 15, 2016 and for interim periods thereafter. Early adoption of ASU 2014-15 is permitted. The Company is currently evaluating ASU 2014-15 and has not yet adopted it. As described in Liquidity and Capital Resources elsewhere in this Form 10-Q, we believe that, based on our current operating plan, our existing cash, cash equivalents and investments will enable us to fund our operations into the third quarter of 2017.

In January 2016, the FASB issued ASU No. 2016-01, Recognition and Measurement of Financial Assets and Financial Liabilities. The amendments in ASU 2016-01 address certain aspects of recognition, measurement, presentation and disclosure of financial instruments. ASU 2016-01 will be effective for fiscal years beginning after December 15, 2017, including interim periods within those fiscal years. Early adoption of some of the amendments included in ASU 2016-01 for financial statements of fiscal years or interim periods that have not yet been issued is permitted as of the beginning of the fiscal year of adoption. The Company is currently evaluating the effect that the adoption of ASU 2016-01 will have on its financial statements.

In February 2016, the FASB issued ASU No. 2016-02, Leases. The amendments in ASU 2016-02 will require organizations that lease assets, with lease terms of more than 12 months, to recognize on the balance sheet the assets and liabilities for the rights and obligations created by those leases. Consistent with current U.S. Generally Accepted Accounting Principles ( U.S. GAAP ), the recognition, measurement, and presentation of expenses and cash flows arising from a lease by a lessee primarily will depend on its classification as a finance or operating lease. However, unlike current U.S. GAAP which requires only capital leases to be recognized on the balance sheet, ASU No. 2016-02 will require both types of leases to be recognized on the balance sheet. ASU 2016-02 will be effective for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years. Early adoption is permitted. The Company is currently evaluating the effect that the adoption of ASU 2016-02 will have on its financial statements.

In March 2016, the FASB issued ASU No. 2016-06, Derivatives and Hedging (Topic 815) Contingent Put and Call Options in Debt Instruments. ASU 2016-06 amends FASB ASC 815-15 to clarify what steps are required when assessing whether the economic characteristics and risks of call (put) options are clearly and closely related to the economic characteristics and risks of their debt hosts, which is one of the criteria for bifurcating an embedded derivative. ASU 2016-06 will be effective for fiscal years beginning after December 15, 2016, including interim periods within those fiscal years. An entity should apply the amendments in ASU No. 2016-06 on a modified retrospective basis to existing debt instruments as of the beginning of the fiscal year for which the amendments are effective. Early adoption is permitted, including adoption in an interim period. The Company is currently evaluating the effect that the adoption of ASU 2016-06 will have on its financial statements.

In March 2016, the FASB issued ASU No. 2016-09, Compensation - Stock Compensation (Topic 718). ASU 2016-09 will require organizations to recognize all income tax effects of awards in the income statement when the awards vest or are settled. ASU 2016-09 will also allow organizations to repurchase more shares from employees than they could previously purchase for tax withholding purposes without triggering liability accounting and to make a policy election to account for forfeitures as they occur. ASU 2016-09 will be effective for fiscal years beginning after December 15, 2016, and interim periods within those fiscal years. Early adoption is permitted in any interim or annual period. The Company is currently evaluating the effect that the adoption of ASU 2016-09 will have on its financial statements.

In April 2016, the FASB issued ASU No. 2016-10, Revenue from Contracts with Customers (Topic 606). ASU 2016-10 amends ASC 606 to clarify two aspects of ASC 606, identifying performance obligations and the licensing implementation guidance, while retaining the related principles of those areas. The amendments in ASU 2016-10 do not change the core principle of the guidance in ASC 606. The amendments in ASU No. 2016-10 affect the guidance

in ASU 2014-09, Revenue from Contracts with Customers (Topic 606), which is not yet effective. The effective date and transition requirements for the amendments in ASU No. 2016-10 are the same as the effective date and transition requirements in ASC 606 and any other Topic amended by Update 2014-09. ASU 2015-14, Revenue from Contracts with Customers (Topic 606): Deferral of the Effective Date, defers the effective date of ASU 2014-09 by one year. The Company is currently evaluating the effect that the adoption of ASU 2016-10 will have on its financial statements.

### (3) Unaudited Interim Financial Statements

The accompanying unaudited financial statements included herein have been prepared by the Company in accordance with U.S. GAAP for interim financial information and pursuant to the rules and regulations of the Securities and Exchange Commission (the SEC). Accordingly, certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. GAAP have been condensed or omitted pursuant to such rules and regulations. In the opinion of management, all adjustments, consisting of normal recurring adjustments, and disclosures considered necessary for a fair presentation of interim period results have been included. Interim results for the three months ended March 31, 2016 are not necessarily indicative of results that may be expected for the year ending December 31, 2016. For further information, refer to the financial statements and footnotes thereto included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2015, which was filed with the SEC on March 10, 2016.

**Table of Contents****(4) Financial Instruments**

The fair value of the Company's financial instruments is determined and disclosed in accordance with the three-tier fair value hierarchy specified in Note 6, Fair Value of Assets and Liabilities. The Company is required to disclose the estimated fair values of its financial instruments. The Company's financial instruments consist of cash, cash equivalents, available-for-sale investments, receivables and a note payable. The estimated fair values of these financial instruments approximate their carrying values as of March 31, 2016 and December 31, 2015. As of March 31, 2016 and December 31, 2015, the Company did not have any derivatives, hedging instruments or other similar financial instruments except for the note issued under the Company's loan and security agreement, which is discussed in Note 5(a) to the financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2015, including put and call features which the Company determined are clearly and closely associated with the debt host and do not require bifurcation as a derivative liability, or the fair value of the feature is immaterial.

**(5) Cash and Cash Equivalents**

The Company considers all highly liquid investments with maturities of 90 days or less when purchased to be cash equivalents. Cash and cash equivalents at March 31, 2016 consisted of cash, commercial paper and money market funds. Cash and cash equivalents at December 31, 2015 consisted of cash and money market funds.

**(6) Fair Value of Assets and Liabilities**

The Company measures fair value at the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date using assumptions that market participants would use in pricing the asset or liability (the inputs) into a three-tier fair value hierarchy. This fair value hierarchy gives the highest priority (Level 1) to quoted prices in active markets for identical assets or liabilities and the lowest priority (Level 3) to unobservable inputs in which little or no market data exists, requiring companies to develop their own assumptions. Observable inputs that do not meet the criteria of Level 1, and include quoted prices for similar assets or liabilities in active markets or quoted prices for identical assets and liabilities in markets that are not active, are categorized as Level 2. Level 3 inputs are those that reflect the Company's estimates about the assumptions market participants would use in pricing the asset or liability, based on the best information available in the circumstances. Valuation techniques for assets and liabilities measured using Level 3 inputs may include unobservable inputs such as projections, estimates and management's interpretation of current market data. These unobservable Level 3 inputs are only utilized to the extent that observable inputs are not available or cost-effective to obtain. The Company applies ASU No. 2011-04, Fair Value Measurement (Topic 820), in its fair value measurements and disclosures.

The table below presents the assets and liabilities measured and recorded in the financial statements at fair value on a recurring basis at March 31, 2016 and December 31, 2015 categorized by the level of inputs used in the valuation of each asset and liability.

<b>(In thousands)</b>	<b>Total</b>	<b>Quoted Prices in Active Markets for Identical Assets or Liabilities</b>	<b>Significant Other Observable Inputs (Level 2)</b>	<b>Significant Unobservable Inputs (Level 3)</b>
-----------------------	--------------	--	--	--

**(Level 1)****March 31, 2016**

Assets					
Money market funds		\$ 20,189	\$ 20,189	\$	\$
Other cash equivalents	commercial paper	1,500			1,500
Short-term investments	corporate bonds	33,979			33,979
Short-term investments	municipal bonds	6,532			6,532
Long-term investments	corporate bonds	4,509			4,509
Long-term investments	municipal bonds	7,223			7,223
<b>Total Assets</b>		<b>\$ 73,932</b>	<b>\$ 20,189</b>	<b>\$ 53,743</b>	<b>\$</b>
<b>Total Liabilities</b>		<b>\$</b>	<b>\$</b>	<b>\$</b>	<b>\$</b>

**December 31, 2015**

Assets					
Money market funds		\$ 26,056	\$ 26,056	\$	\$
Short-term investments	commercial paper	3,974			3,974
Short-term investments	corporate bonds	24,575			24,575
Short-term investments	municipal bonds	5,025			5,025
Long-term investments	corporate bonds	21,186			21,186
Long-term investments	municipal bonds	5,811			5,811
<b>Total Assets</b>		<b>\$ 86,627</b>	<b>\$ 26,056</b>	<b>\$ 60,571</b>	<b>\$</b>
<b>Total Liabilities</b>		<b>\$</b>	<b>\$</b>	<b>\$</b>	<b>\$</b>

**Table of Contents**

The Level 1 assets consist of money market funds, which are actively traded daily. The Level 2 assets consist of corporate bond, commercial paper and municipal bond investments whose fair value may not represent actual transactions of identical securities. The fair value of corporate and municipal bonds is generally determined from quoted market prices received from pricing services based upon quoted prices from active markets and/or other significant observable market transactions at fair value. The fair value of commercial paper is generally determined based on the relationship between the investment's discount rate and the discount rates of the same issuer's commercial paper available in the market which may not be actively traded daily. Since these fair values may not be based upon actual transactions of identical securities, they are classified as Level 2. Since any investments are classified as available-for-sale securities, any unrealized gains or losses are recorded in accumulated other comprehensive income or loss within stockholders' equity on the balance sheet. The Company did not elect to measure any other financial assets or liabilities at fair value at March 31, 2016 or December 31, 2015.

**(7) Investments**

The Company's available-for-sale investments at fair value consisted of the following at March 31, 2016 and December 31, 2015:

		<b>March 31, 2016</b>			<b>Estimated Fair Value</b>
		<b>Cost</b>	<b>Gross Unrealized (Losses)</b>	<b>Gross Unrealized Gains</b>	
		<b>(In thousands)</b>			
Short-term investments	corporate bonds	\$ 34,006	\$ (29)	\$ 2	\$ 33,979
Short-term investments	municipal bonds	6,532			6,532
<b>Total short-term investments</b>		<b>40,538</b>	<b>(29)</b>	<b>2</b>	<b>40,511</b>
Long-term investments	corporate bonds	4,505	(1)	5	4,509
Long-term investments	municipal bonds	7,200		23	7,223
<b>Total long-term investments</b>		<b>11,705</b>	<b>(1)</b>	<b>28</b>	<b>11,732</b>
<b>Total investments</b>		<b>\$ 52,243</b>	<b>\$ (30)</b>	<b>\$ 30</b>	<b>\$ 52,243</b>

		<b>December 31, 2015</b>			<b>Estimated Fair Value</b>
		<b>Cost</b>	<b>Gross Unrealized (Losses)</b>	<b>Gross Unrealized Gains</b>	
		<b>(In thousands)</b>			
Short-term investments	commercial paper	\$ 3,973	\$	\$ 1	\$ 3,974
Short-term investments	corporate bonds	24,600	(25)		24,575
Short-term investments	municipal bonds	5,025			5,025
<b>Total short-term investments</b>		<b>33,598</b>	<b>(25)</b>	<b>1</b>	<b>33,574</b>
Long-term investments	corporate bonds	21,289	(103)		21,186
Long-term investments	municipal bonds	5,818	(9)	2	5,811



Edgar Filing: IDERA PHARMACEUTICALS, INC. - Form 10-Q

Total long-term investments	27,107	(112)	2	26,997
Total investments	\$ 60,705	\$ (137)	\$ 3	\$ 60,571

7

**Table of Contents**

The Company had no realized gains or losses from available-for-sale securities in the three months ended March 31, 2016 and 2015. There were no losses or other-than-temporary declines in value included in Interest income on the Company's condensed statements of operations and comprehensive loss for any securities for the three months ended March 31, 2016 and 2015. The Company had no auction rate securities as of March 31, 2016 and December 31, 2015. See Note 4, Financial Instruments, and Note 6, Fair Value of Assets and Liabilities for additional information related to the Company's investments.

**(8) Property and Equipment**

At March 31, 2016 and December 31, 2015, net property and equipment at cost consisted of the following:

<b>(In thousands)</b>	<b>March 31, 2016</b>	<b>December 31, 2015</b>
Leasehold improvements	\$ 636	\$ 603
Laboratory equipment and other	4,680	4,543
<b>Total property and equipment, at cost</b>	<b>5,316</b>	<b>5,146</b>
Less: accumulated depreciation	3,589	3,454
<b>Property and equipment, net</b>	<b>\$ 1,727</b>	<b>\$ 1,692</b>

Depreciation and amortization expense on property and equipment was approximately \$151,000 and \$93,000 in the three months ended March 31, 2016 and 2015, respectively. There were \$99,000 and \$36,000 in non-cash property additions in the three months ended March 31, 2016 and 2015, respectively.

**(9) Restricted Cash**

As part of the Company's lease arrangement for its office and laboratory facility in Cambridge, Massachusetts, the Company is required to restrict cash held in a certificate of deposit securing a line of credit for the lessor. As of March 31, 2016 and December 31, 2015, the restricted cash amounted to \$311,000 held in certificates of deposit securing a line of credit for the lessor.

**(10) Comprehensive Loss**

Comprehensive income (loss) is defined as the change in equity of a business enterprise during a period from transactions and other events and circumstances from non-owner sources. Comprehensive loss for the three months ended March 31, 2016 and 2015 is comprised of reported net loss and any change in net unrealized gains and losses on investments during each period, which is included in accumulated other comprehensive income (loss) on the accompanying balance sheets. The Company applies ASU No. 2011-05, Comprehensive Income, by presenting the components of net income and other comprehensive income as one continuous statement.

The following table includes the changes in the accumulated balance of the component of other comprehensive income (loss) for the three months ended March 31, 2016 and 2015:

<b>(In thousands)</b>	<b>Three Months Ended March 31, 2016</b>	<b>Three Months Ended March 31, 2015</b>
Accumulated unrealized loss on available-for-sale securities at beginning of period	\$ (134)	\$ (17)
Change during the period	134	18
Accumulated unrealized income on available-for-sale securities at end of period	\$	\$ 1

**Table of Contents**

(11) Collaboration with GlaxoSmithKline Intellectual Property Development Limited

On November 20, 2015, the Company entered into a collaboration and license agreement with GlaxoSmithKline Intellectual Property Development Limited ( GSK ) to license, research, develop and commercialize pharmaceutical compounds from the Company's 3GA technology for the treatment of selected targets in renal disease (the GSK Agreement ). The initial collaboration term is currently anticipated to last between two and four years. In connection with the GSK Agreement, GSK identified an initial target for Idera to attempt to identify a potential population of development candidates to address such target under a mutually agreed upon research plan, currently estimated to take 27 months to complete. From the population of identified development candidates, GSK may designate one development candidate in its sole discretion to move forward into clinical development. Once GSK designates a development candidate, GSK would be solely responsible for the development and commercialization activities for that designated development candidate.

At any time during the first two years of the GSK Agreement, GSK has the option to select up to two additional targets, for further research under mutually agreed upon research plans. GSK may then designate one development candidate for each additional target, at which time GSK would have sole responsibility to develop and commercialize each such designated development candidate.

In accordance with the GSK Agreement, a Joint Steering Committee (JSC) was formed with equal representation from Idera and GSK. The responsibilities of the JSC, include, but are not limited to monitoring the progress of the collaboration, reviewing research plans and dealing with disputes that may arise amongst the parties. If a dispute cannot be resolved by the JSC, GSK has final decision making authority.

Under the terms of the GSK Agreement, the Company received a \$2,500,000 upfront, non-refundable, non-creditable cash payment upon the execution of the GSK Agreement. The Company is eligible to receive up to approximately \$100,000,000 in license, research, clinical development and commercialization milestone payments. Approximately \$9,000,000 of these milestone payments are payable by GSK upon the identification of additional targets, the completion of current and future research plans and the designation of development candidates. Approximately \$89,000,000 is payable by GSK upon the achievement of clinical milestones and commercial milestones. In addition, the Company is eligible to receive royalty payments on sales upon commercialization at varying rates of up to five percent on annual net sales, as defined in the GSK Agreement.

*Accounting Analysis*

The Company evaluated the GSK Agreement in accordance with the provisions of ASC 605-25. The GSK Agreement contains the following initial deliverables: (i) a collaboration license for Idera's proprietary technology related to the initial target (the Collaboration License ), (ii) research services (the Research Services ), and (iii) participation in a Joint Steering Committee (the JSC Deliverable ).

The Company has determined that the options to choose up to two additional targets and to purchase additional collaboration licenses for the Company's proprietary technology related to each additional target are substantive options. GSK is not contractually obligated to exercise the options. Moreover, as a result of the uncertain outcome of the research activities, there is significant uncertainty as to whether GSK will decide to exercise its options for any additional targets. Consequently, the Company is at risk with regard to whether GSK will exercise the options. The Company has determined that the options to choose up to two additional targets and to purchase additional collaboration licenses for the Company's proprietary technology related to each additional target are not priced at a significant and incremental discount.

The Company has concluded that the Collaboration License does not qualify for separation from the Research Services. As it relates to the assessment of standalone value, the Company has determined that GSK cannot fully exploit the value of the Collaboration License without receipt of the Research Services from the Company. The Research Services involve unique skills and specialized expertise, particularly as it relates to the Company's proprietary technology, which is not available in the marketplace. Accordingly, GSK must obtain the Research Services from the Company which significantly limits the ability for GSK to utilize the Collaboration License for its intended purpose on a standalone basis. Therefore, the Collaboration License does not have standalone value from the Research Services. As a result, the Collaboration License and the Research Services have been combined as a single unit of accounting (the R&D Services Unit of Accounting). The Company has concluded that the JSC Deliverable identified at the inception of the arrangement has standalone value from the other deliverables noted based on its nature. Factors considered in this determination included, among other things, the capabilities of the collaboration partner, whether any other vendor sells the item separately, whether the value of the deliverable is dependent on the other elements in the arrangement, whether there are other vendors that can provide the items and if the customer could use the item for its intended purpose without the other deliverables in the arrangement.

## **Table of Contents**

Therefore, the Company has identified two units of accounting in connection with its initial deliverables under the GSK Agreement as follows: (i) R&D Services Unit of Accounting, and (ii) JSC Deliverable.

Allocable arrangement consideration at inception of the GSK Agreement is comprised of the up-front payment of \$2.5 million, which was allocated to the R&D Services Unit of Accounting. No amount was allocated to the JSC Deliverable because the related best estimate of selling price was determined to be de minimus. The \$2.5 million was recorded as deferred revenue in the Company's balance sheet and is being recognized as revenue on a straight line basis as the Research Services are delivered over the estimated 27 month research plan period.

The Company has determined that certain of the payments that may be received under the GSK Agreement are related to substantive milestones. Factors considered in the evaluation of the milestones included the degree of risk associated with the achievement of the milestone, the level of effort and investment required, whether the milestone consideration was reasonable relative to the deliverables and whether the milestone was earned at least in part based on the Company's performance. Revenues from substantive milestones, if they are nonrefundable and non-creditable, are recognized as revenue upon successful achievement of the related milestone, including the successful completion of research plans. Clinical and commercial milestones are deemed non-substantive as they are based solely on GSK's performance. Non-substantive milestones will be recognized when achieved to the extent the Company has no remaining performance obligations under the arrangement. Payments received in connection with GSK's identification of additional targets will be recognized as revenue as the research services are delivered under the related research plans.

The Company will recognize royalty revenue in the period of sale of the related product(s), based on the underlying contract terms, provided that the reported sales are reliably measurable and the Company has no remaining performance obligations, assuming all other revenue recognition criteria are met.

The Company recognized as revenue approximately \$277,000 of deferred revenue related to the GSK Agreement during the three months ended March 31, 2016. This revenue is classified as alliance revenue in the accompanying statements of operations and comprehensive loss. There was approximately \$2,096,000 of deferred revenue related to the GSK Agreement at March 31, 2016, including approximately \$1,111,000 classified as current portion of deferred revenue in the accompanying balance sheet.

### **(12) Stock-Based Compensation**

The Company recognizes all stock-based payments to employees and directors as expense in the statements of operations and comprehensive loss based on their fair values. The Company records compensation expense over an award's requisite service period, or vesting period, based on the award's fair value at the date of grant. The Company's policy is to charge the fair value of stock options as an expense, adjusted for forfeitures, on a straight-line basis over the vesting period, which is generally four years for employees and three years for directors.

The Company recorded charges of \$1,716,000 and \$1,346,000 in its statements of operations and comprehensive loss for the three months ended March 31, 2016 and 2015, respectively, for stock-based compensation expense attributable to stock-based payments made to employees and directors. The fair value of each option award is estimated on the date of grant using the Black-Scholes option-pricing model. The following weighted average assumptions apply to the options to purchase 2,733,750 and 969,500 shares of common stock granted to employees and directors during the three months ended March 31, 2016 and 2015, respectively:

	<b>Three Months Ended</b>	
	<b>March 31,</b>	
	<b>2016</b>	<b>2015</b>
Average risk free interest rate	1.5%	1.2%
Expected dividend yield		
Expected lives (years)	4.1	4.2
Expected volatility	93.0%	92.0%
Weighted average grant date fair value of options granted during the period (per share)	\$ 1.90	\$ 2.98
Weighted average exercise price of options granted during the period (per share)	\$ 2.86	\$ 4.50

**Table of Contents**

The expected lives and the expected volatility of the options granted during the three months ended March 31, 2016 and 2015 are based on historical experience. All options granted during the three months ended March 31, 2016 and 2015 were granted at exercise prices equal to the fair market value of the common stock on the dates of grant.

**(13) Net Loss per Common Share**

For the three months ended March 31, 2016 and 2015, basic and diluted net loss per common share is computed using the weighted average number of shares of common stock outstanding during the period. Diluted net loss per common share is the same as basic net loss per common share as the effects of the Company's potential common stock equivalents are antidilutive. Total antidilutive securities were 73,346,318 and 74,950,998 for the three months ended March 31, 2016 and 2015, respectively, and consist of stock options, preferred stock and warrants.

**(14) Common Stock Option Exercises and Employee Stock Purchases**

The Company issued common stock to employees as a result of stock option exercises and employee stock purchases as follows during the three months ended March 31, 2016 and 2015:

<b>(In thousands)</b>	<b>Three Months Ended March 31, 2016</b>		<b>Three Months Ended March 31, 2015</b>	
	<b>Shares</b>	<b>Proceeds</b>	<b>Shares</b>	<b>Proceeds</b>
Stock option exercises		\$	25	\$ 106
Employee stock purchases	24	36	6	15
<b>Total</b>	<b>24</b>	<b>\$ 36</b>	<b>31</b>	<b>\$ 121</b>

**(15) Related Party Transactions**

The Company issued 10,690 and 3,927 shares of common stock in lieu of director board and committee fees of approximately \$32,000 and \$19,000 pursuant to the Company's director compensation program during the three months ended March 31, 2016 and 2015, respectively. The number of shares issued was calculated based on the market closing price of the Company's common stock on the issuance date.

See also Note 16, Financing for additional information on related party transactions.

**(16) Financing**

On February 19, 2015, the Company closed a follow-on underwritten public offering, in which it sold 23,000,000 shares of common stock at a price to the public of \$3.75 per share for aggregate gross proceeds of \$86.3 million. The net proceeds to the Company from the offering, after deducting underwriters' discounts and commissions and other offering costs and expenses, were \$80.6 million. Investment funds affiliated with Baker Bros. Advisors LP, one of our principal stockholders, and two members of the Company's board of directors purchased 5,333,333 shares in this offering at the \$3.75 per share purchase price.

**(17) Subsequent Events**



The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the financial statements to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure.

---

**Table of Contents**

**ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS.**

**Overview**

We are a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics for oncology and rare diseases. We use two distinct proprietary drug discovery technology platforms to design and develop drug candidates: our Toll-like receptor, or TLR, targeting technology and our third-generation antisense, or 3GA, technology. We developed these platforms based on our scientific expertise and pioneering work with synthetic oligonucleotides as therapeutic agents. Using our TLR targeting technology, we design synthetic oligonucleotide-based drug candidates to modulate the activity of specific TLRs. In addition, using our 3GA technology, we are developing drug candidates to turn off the messenger RNA, or mRNA, associated with disease causing genes. We believe that our 3GA technology may potentially reduce the immunotoxicity and increase the potency of earlier generation antisense and RNA interference, or RNAi, technologies.

We are focused on the development of drug candidates for oncology and rare diseases characterized by small, well-defined patient populations with serious unmet medical needs. We plan to explore potential collaborative alliances to support late-stage development and commercialization of some of our drug candidates.

Our TLR antagonist clinical-stage drug candidates are IMO-8400 and IMO-9200, which are both antagonists of TLR7, TLR8 and TLR9. We also have created compounds that are agonists of TLR3, TLR7, TLR8 or TLR9. IMO-2125, a TLR9 agonist, is our TLR agonist clinical-stage drug candidate.

*IMO-8400 Development Program in Genetically Defined Forms of B-cell Lymphoma*

We are developing IMO-8400 for the treatment of certain B-cell lymphomas in which the MYD88 L265P oncogenic mutation is present. Oncogenic mutations are changes in the DNA of tumor cells that promote the survival and proliferation of tumor cells. MYD88 is an adaptor protein in the TLR signaling pathway that mediates TLR signaling.

The U.S. Food and Drug Administration, or the FDA, has granted orphan drug designation for IMO-8400 for the treatment of Waldenström's macroglobulinemia and for the treatment of diffuse large B-cell lymphoma, or DLBCL. Orphan drug designation is granted by the FDA Office of Orphan Products Development to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States. This designation provides certain incentives, including eligibility for federal grants, research and development tax credits, a waiver of Prescription Drug User Fee Act filing fees and a seven-year marketing exclusivity period, once the product is approved and as long as orphan drug designation is maintained.

*Phase 1/2 Clinical Trial of IMO-8400 in Waldenström's Macroglobulinemia.*

In 2014, we initiated patient treatment in our ongoing open-label, dose-escalation Phase 1/2 clinical trial of IMO-8400 in patients with Waldenström's macroglobulinemia who have relapsed after or were refractory to prior therapy. Objectives of the trial include evaluation of safety and tolerability of escalating IMO-8400 dose levels and assessment of IMO-8400 clinical activity using disease-specific international guidelines for classifying clinical response. In this trial, we evaluated doses of 0.6, 1.2 mg/kg per week and 1.2 mg/kg twice weekly administered as subcutaneous injections for 24 weeks. This study is being conducted at 13 sites in the United States. We presented initial data from the ongoing trial at the 2015 American Society of Hematology Meeting which showed that IMO-8400 was demonstrating signals of efficacy as well as a favorable safety profile having not yet met the maximum tolerated dose (MTD). We enrolled additional patients at the highest dose level of 1.2 mg/kg/ twice weekly to further characterize

safety and clinical activity and plan to provide an update on those data during our Q1 2016 investor conference call. Concurrently, we amended the trial protocol to enable further dose escalation, with the initial planned dose escalations to include 2.4 and 3.6 mg/kg administered once weekly.

*Phase 1/2 Clinical Trial of IMO-8400 in Diffuse Large B-cell Lymphoma*

We are conducting an open-label, dose-escalation Phase 1/2 clinical trial of IMO-8400 in patients with DLBCL who have relapsed after or were refractory to prior therapy. We are using a prototype companion diagnostic we developed under our collaboration agreement with Abbott Molecular, Inc., or Abbott Molecular, to identify patients with the MYD88 L265P oncogenic mutation. Objectives of the trial include evaluation of safety and tolerability of escalating IMO-8400 dose levels and assessment of IMO-8400 clinical activity using disease-specific international guidelines for classifying clinical response. In this trial, we initially planned to evaluate escalating doses of 0.6, 1.2 mg/kg per week and 1.2 mg/kg twice weekly, administered as subcutaneous injections for 24 weeks. We are conducting the trial at 13 clinical sites in the United States. To date, we have enrolled five patients in the trial and fully enrolled the 0.6 mg/kg dose cohort. We have amended the trial protocol to advance dose escalation to the advanced dosing levels currently being tested in the Waldenström's macroglobulinemia trial (2.4 and 3.6 mg/kg once weekly).

We believe that B-cell lymphomas in patients with the MYD88 L265P oncogenic mutation are rare diseases with serious unmet medical needs, based on prevalence of the indications and our understanding of the current treatment paradigms and are evaluating associated regulatory and clinical development strategies. We cannot predict whether or when any of our drug candidates will prove effective or safe in humans, if we will be able to participate in FDA expedited review and approval programs, including breakthrough and fast track designation, or if our drug candidates will receive regulatory approval.

## **Table of Contents**

### *Application of TLR Agonists in Immuno-Oncology*

Our pipeline of drug candidates includes IMO-2125, a TLR9 agonist that may have potential applications as an immune therapy for the treatment of cancer.

In June 2015, we entered into a strategic clinical research alliance with MD Anderson Cancer Center, or MD Anderson, to advance clinical development of TLR9 agonists in combination with checkpoint inhibitors. In December 2015, a Phase 1/2 clinical trial was initiated to assess the safety and efficacy of IMO-2125, administered intra-tumorally in combination with ipilimumab, a CTLA4 antibody marketed as Yervoy<sup>®</sup> by Bristol-Myers Squibb Company, in patients with metastatic melanoma. In this clinical trial, escalating doses of IMO-2125 ranging from 4 mg/kg through 32 mg/kg are being administered intra-tumorally into one of two selected tumor lesions, with a standard dosing regimen of ipilimumab. The primary objectives of the Phase 1 portion of the trial are to determine the maximum tolerated dose and characterize the dose-limiting toxicities of IMO-2125 when administered intra-tumorally in combination with ipilimumab. The primary objective of the Phase 2 portion of the trial is to determine the efficacy of the combination utilizing the immune-related response criteria in addition to traditional RECIST criteria. Serial biopsies will be taken of selected injected and non-injected tumor lesions to assess immune changes and response assessments. We anticipate that the trial will enroll approximately 45 patients. The trial protocol is being amended to enable an additional cohort to study the combination of IMO-2125 and an anti-PD1 antibody in the same patient population.

We are also working on the design and planning for a second study of IMO-2125 as well as developing our strategy to optimize IMO-2125's position in the immune oncology therapeutic category.

### *Program in Rare Diseases*

We are developing IMO-8400 for non-cancer rare diseases. We believe that we demonstrated proof of concept for our approach of using TLR antagonists to inhibit the over-activation of specific TLRs in a randomized, double-blind, placebo-controlled Phase 2 clinical trial of IMO-8400 that we conducted in patients with moderate to severe plaque psoriasis, a well-characterized autoimmune disease. In this trial, we evaluated IMO-8400 at four subcutaneous dose levels of 0.075, 0.15, 0.3, and 0.6 mg/kg, versus placebo, administered once weekly for 12 weeks in 46 patients. The trial met its primary objective as IMO-8400 was well tolerated at all dose levels with no treatment-related discontinuations, treatment-related serious adverse events, or dose reductions. The trial also met its secondary objective of demonstrating clinical activity in psoriasis patients, as assessed by the Psoriasis Area Severity Index. In March 2015, we presented the complete data from the Phase 2 trial of IMO-8400 in patients with moderate to severe plaque psoriasis at the annual meeting of the American Academy of Dermatology. With our focus on rare diseases, we do not currently plan to conduct further clinical development of IMO-8400 for the treatment of psoriasis.

### *IMO-8400 Development Program for Dermatomyositis.*

We have selected dermatomyositis as the first non-cancer rare disease for application of IMO-8400. We selected this indication for development based on the reported role of TLRs in the pathogenesis of the disease state, clinical feasibility, including ease of patient identification and availability of endpoints for regulatory approval, and commercial potential.

In December 2015, we initiated a randomized, double-blind, placebo controlled Phase 2 clinical trial of IMO-8400 in adult patients with dermatomyositis with documented skin and muscle involvement to primarily assess safety, tolerability and treatment effect. Eligible patients are being randomized to receive once weekly subcutaneous injections of placebo or various dose levels of IMO-8400 for a period of 24 weeks. We expect to enroll approximately

36 patients in the trial which we expect to conduct at approximately 20 clinical sites. Several exploratory assessments in the trial also include: associations between treatment effect of IMO-8400 on indices of disease activity, patient-reported outcomes and pharmacodynamics measures; immunogenicity of IMO-8400, time-sensitive plasma concentrations of IMO-8400; and characterization of disease-specific autoantibodies in the enrolled population.

*Third-generation Antisense (3GA) Technology to Target RNA*

We are developing our 3GA technology to turn off the mRNA associated with disease causing genes. We have designed 3GA oligonucleotides to specifically address challenges associated with earlier generation antisense and RNAi technologies.

Our focus is on creating 3GA candidates targeted to specific genes to treat cancer and rare diseases. Our key considerations in identifying disease indications in our 3GA program include: strong evidence that the disease is caused by a specific protein; clear criteria to identify a target patient population; biomarkers for early assessment of clinical proof of concept; a targeted therapeutic mechanism for action; unmet medical need to allow for a rapid development path to approval and commercial opportunity. Based on

## Table of Contents

these criteria, we are developing 3GA compounds against two gene targets, NLRP3 (NOD-like receptor family, pyrin domain containing protein 3) and DUX4 (Double Homeobox 4) which we have begun to advance into clinical development activities. Potential disease indications include, but are not limited to interstitial cystitis, uveitis and facioscapulohumeral muscular dystrophy (FSHD), respectively. Concurrently, we are also engaging in academic collaborations to further evaluate the application of 3GA technology in additional gene targets and exploring development opportunities with third parties for applications outside of our current focus areas.

### *Collaboration with GlaxoSmithKline Intellectual Property Development Limited*

On November 20, 2015, we entered into a collaboration and license agreement with GlaxoSmithKline Intellectual Property Development Limited, or GSK, to license, research, develop and commercialize pharmaceutical compounds from our 3GA technology for the treatment of selected targets in renal disease, the GSK Agreement. The initial collaboration term is currently anticipated to last between two and four years. In connection with the GSK Agreement, GSK identified an initial target for us to attempt to identify a potential population of development candidates to address such target under a mutually agreed upon research plan, currently estimated to take 27 months to complete. From the population of identified development candidates, GSK may designate one development candidate in its sole discretion to move forward into clinical development. Once GSK designates a development candidate, GSK would be solely responsible for the development and commercialization activities for that designated development candidate.

At any time during the first two years of the GSK Agreement, GSK has the option to select up to two additional targets, for further research under mutually agreed upon research plans. GSK may then designate one development candidate for each additional target, at which time GSK would have sole responsibility to develop and commercialize each such designated development candidate.

In accordance with the GSK Agreement, a joint steering committee, or JSC, was formed with equal representation from us and GSK. The responsibilities of the JSC, include, but are not limited to monitoring the progress of the collaboration, reviewing research plans and dealing with disputes that may arise between the parties. If a dispute cannot be resolved by the JSC, GSK has final decision making authority.

Under the terms of the GSK Agreement, we received a \$2,500,000 upfront, non-refundable, non-creditable cash payment upon the execution of the GSK Agreement. We are eligible to receive up to approximately \$100,000,000 in license, research, clinical development and commercialization milestone payments. Approximately \$9,000,000 of these milestone payments are payable by GSK upon the identification of additional targets, the completion of current and future research plans and the designation of development candidates. Approximately \$89,000,000 is payable by GSK upon the achievement of clinical milestones and commercial milestones. In addition, we are eligible to receive royalty payments on sales upon commercialization at varying rates of up to five percent on annual net sales, as defined in the GSK Agreement.

### *Other Programs*

*IMO-9200 for Autoimmune Disease.* We have developed a second novel synthetic oligonucleotide antagonist of TLR7, TLR8, and TLR9, IMO-9200, as a drug candidate for potential use in selected autoimmune disease indications. In May 2015, we completed and reported top-line data from a Phase 1 clinical trial of subcutaneously administered IMO-9200 in healthy subjects. We have also completed and presented data during the 2015 Digestive Disease Week Conference from preclinical studies of orally administered IMO-9200 in mouse models of inflammatory bowel disease, or IBD, including Crohn's Disease and ulcerative colitis. In these preclinical studies, the results demonstrated the potential for orally dosed IMO-9200 as a treatment for IBD. As we are focused on drug development specifically in oncology and rare diseases, we are currently reviewing our various strategic options related to the future

development of IMO-9200.

*Accumulated Deficit*

As of March 31, 2016, we had an accumulated deficit of \$512,904,000. We expect to incur substantial operating losses in future periods. We do not expect to generate significant product revenue, sales-based milestones or royalties from our development programs until we successfully complete development and obtain marketing approval for drug candidates, either alone or in collaborations with third parties, which we expect will take a number of years. In order to commercialize our drug candidates, we need to complete clinical development and comply with comprehensive regulatory requirements.

**CRITICAL ACCOUNTING POLICIES AND ESTIMATES**

This management's discussion and analysis of financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States. The preparation of these financial statements requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities

## **Table of Contents**

and the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. On an ongoing basis, management evaluates its estimates and judgments, including those related to stock-based compensation. Management bases its estimates and judgments on historical experience and on various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

We regard an accounting estimate or assumption underlying our financial statements as a critical accounting estimate where:

the nature of the estimate or assumption is material due to the level of subjectivity and judgment necessary to account for highly uncertain matters or the susceptibility of such matters to change; and

the impact of the estimates and assumptions on financial condition or operating performance is material. Our significant accounting policies are described in Note 2 of the notes to our financial statements in our Annual Report on Form 10-K for the year ended December 31, 2015. Not all of these significant policies, however, fit the definition of critical accounting policies and estimates. We believe that our accounting policies relating to revenue recognition, stock-based compensation and research and development prepayments, accruals and related expenses, as described under the caption "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations - Critical Accounting Policies and Estimates" in our Annual Report on Form 10-K for the year ended December 31, 2015, fit the description of critical accounting estimates and judgments. There were no changes in these policies during the three months ended March 31, 2016.

## **RESULTS OF OPERATIONS**

### ***Three Months Ended March 31, 2016 and 2015***

#### *Alliance Revenue*

Alliance revenue increased by approximately \$260,000 from \$34,000 in three months ended March 31, 2015 to \$294,000 in the three months ended March 31, 2016 as a result of revenue recognized under our collaboration agreement with GSK. In November 2015, in connection with the execution of the GSK Agreement, we received a \$2,500,000 upfront payment that we recognized as deferred revenue. We are recognizing this as revenue in our statements of operations and comprehensive loss on a straight line basis over the anticipated 27-month performance period. Accordingly, we recognized approximately \$277,000 of alliance revenue related to the GSK Agreement during the three months ended March 31, 2016. We also recognized revenue from the reimbursement by licensees of costs associated with patent maintenance as alliance revenue in the three months ended March 31, 2016 and 2015.

#### *Research and Development Expenses*

Research and development expenses increased by \$576,000, or 7%, from \$8,720,000 for the three months ended March 31, 2015, to \$9,296,000 for the three months ended March 31, 2016. In the following table, research and development expenses are set forth in the following five categories which are discussed beneath the table:



	Three Months Ended March 31, (in thousands)		Percentage Increase (Decrease)
	2016	2015	
IMO-8400 external development expense	\$ 2,138	\$ 2,423	(12)%
IMO-2125 external development expense	1,174		
IMO-9200 external development expense	206	1,666	(88)%
Other drug development expense	3,152	2,572	23%
Basic discovery expense	2,626	2,059	28%
	\$ 9,296	\$ 8,720	7%

*IMO-8400 External Development Expenses.* These expenses include external expenses that we have incurred in connection with IMO-8400 since October 2012, when we commenced clinical development of IMO-8400. These external expenses include payments to independent contractors and vendors for drug development activities conducted after the initiation of IMO-8400 clinical development but exclude internal costs such as payroll and overhead expenses. Since October 2012, we have incurred approximately \$25,264,000 in IMO-8400 external development expenses through March 31, 2016, including costs associated with our Phase 1

---

**Table of Contents**

clinical trial in healthy subjects, our Phase 2 clinical trial in patients with psoriasis, preparation for and conduct of our ongoing Phase 1/2 clinical trial in patients with Waldenström's macroglobulinemia, our ongoing Phase 1/2 clinical trial in patients with DLBCL harboring the MYD88 L265P oncogenic mutation and our ongoing Phase 2 clinical trial in patients with dermatomyositis, the manufacture of additional drug substance for use in our clinical trials, and expenses associated with our collaboration with Abbott Molecular for the development of a companion diagnostic for identification of patients with B-cell lymphoma harboring the MYD88 L265P oncogenic mutation.

The decrease in our IMO-8400 external development expenses in the three months ended March 31, 2016, as compared to the three months ended March 31, 2015, was primarily due to lower costs incurred in the three months ended March 31, 2016 in connection with the manufacture of additional drug substance for use in our clinical trials. Decreases in the cost of developing a companion diagnostic for identification of patients with B-cell lymphoma harboring the MYD88 L265P oncogenic mutation and the cost of conducting our ongoing Phase 1/2 clinical trial in patients with DLBCL harboring the MYD88 L265P oncogenic mutation also contributed to the decrease in our IMO-8400 external development expenses in the three months ended March 31, 2016. These decreases were partially offset by increases in costs associated with our ongoing Phase 2 clinical trial in patients with dermatomyositis and our ongoing Phase 1/2 clinical trial in patients with Waldenström's macroglobulinemia.

We expect our IMO-8400 external development expenses to increase during 2016, as compared to 2015, as we plan to continue our Phase 1/2 clinical trial in patients with Waldenström's macroglobulinemia, our Phase 1/2 clinical trial in patients with DLBCL harboring the MYD88 L265P oncogenic mutation, and our Phase 2 clinical trial in patients with dermatomyositis, and continue manufacturing activities and nonclinical safety studies.

*IMO-2125 External Development Expenses.* These expenses include external expenses that we have incurred in connection with the development of IMO-2125 as part of our immuno-oncology program. These external expenses include payments to independent contractors and vendors for drug development activities conducted after the initiation of IMO-2125 clinical development in immuno-oncology, but exclude internal costs such as payroll and overhead expenses. We commenced clinical development of IMO-2125 as part of our immuno-oncology program in July 2015 and from July 2015 through March 31, 2016 we incurred approximately \$2,357,000 in IMO-2125 external development expenses, including costs associated with the preparation for and conduct of our ongoing Phase 1/2 clinical trial being conducted under our research alliance with MD Anderson to assess the safety and efficacy of IMO-2125 in combination with ipilimumab, marketed as Yervoy® by Bristol-Myers Squibb Company, in patients with metastatic melanoma, the manufacture of additional drug substance for use in our clinical trials and additional nonclinical studies. The \$2,357,000 in IMO-2125 external development expenses excludes costs incurred prior to July 2015 with respect to IMO-2125, including costs incurred for the development of IMO-2125 for the treatment of patients with chronic hepatitis C virus which we discontinued in the third quarter of 2011.

We expect our IMO-2125 external development expenses to increase during 2016, as compared to 2015, as we plan to continue our Phase 1/2 clinical trial being conducted under our research alliance with MD Anderson to assess the safety and efficacy of IMO-2125 in combination with ipilimumab in patients with metastatic melanoma, amend this protocol to enable an additional cohort to study the combination of IMO-2125 and an anti-PD1 antibody in the same patient population, work on the design and planning for a second study of IMO-2125 and develop our strategy to optimize IMO-2125's position in the immune oncology therapeutic category, and continue manufacturing activities and nonclinical studies.

*IMO-9200 External Development Expenses.* These expenses include external expenses that we have incurred in connection with IMO-9200 since October 2014, when we commenced clinical development of IMO-9200. These external expenses include payments to independent contractors and vendors for drug development activities conducted after the initiation of IMO-9200 clinical development but exclude internal costs such as payroll and overhead

expenses. We have incurred approximately \$4,367,000 in IMO-9200 external development expenses from October 2014 through March 31, 2016, including costs associated with our Phase 1 clinical trial in healthy subjects, the manufacture of additional drug substance for use in our clinical and nonclinical trials and additional nonclinical studies. We classified the IMO-9200 external development expenses incurred prior to October 2014 in other drug development expenses.

The decrease in IMO-9200 external development expenses in the three months ended March 31, 2016, as compared to the three months ended March 31, 2015, reflects decreases in clinical and nonclinical trial costs, drug manufacturing and nonclinical study costs incurred during the three months ended March 31, 2016, as compared to the corresponding 2015 period. We expect our IMO-9200 external development expenses to decrease during 2016, as compared to 2015, as we review our strategic options related to the future development of IMO-9200.

---

**Table of Contents**

*Other Drug Development Expenses.* These expenses include external expenses associated with preclinical development of identified compounds in anticipation of advancing these compounds into clinical development. In addition, these expenses include internal costs, such as payroll and overhead expenses, associated with preclinical development and products in clinical development. The external expenses associated with preclinical compounds include payments to contract vendors for manufacturing and the related stability studies, preclinical studies, including animal toxicology and pharmacology studies, and professional fees. Other drug development expenses also include costs associated with compounds that were previously being developed but are not currently being developed. The increase in other drug development expenses in the three months ended March 31, 2016, as compared to the three months ended March 31, 2015, was primarily due additional headcount associated with our expanded drug development programs. The increase in other drug development expenses was partially offset by lower consulting costs in the three months ended March 31, 2016.

*Basic Discovery Expenses.* These expenses include our internal and external expenses relating to our discovery efforts with respect to our TLR-targeted programs, including agonists and antagonists of TLR3, TLR7, TLR8 and TLR9, and our 3GA program. These expenses reflect payments for laboratory supplies, external research, and professional fees, as well as payroll and overhead expenses. The increase in basic discovery expenses in the three months ended March 31, 2016, as compared to the three months ended March 31, 2015, was primarily due to increases in stock-based compensation, the costs of laboratory supplies, facilities expenses, external research and consulting costs, and the accrual of incentive compensation during the three months ended March 31, 2016.

We do not know if we will be successful in developing any drug candidate from our research and development programs. At this time, and without knowing the results from our ongoing clinical trials of IMO-8400, our ongoing clinical trial of IMO-2125, and our ongoing clinical development program in the first gene target selected for development in our 3GA program, we cannot reasonably estimate or know the nature, timing, and costs of the efforts that will be necessary to complete the remainder of the development of, or the period, if any, in which material net cash inflows may commence from, any drug candidate from our research and development programs. Moreover, the clinical development of any drug candidate from our research and development programs is subject to numerous risks and uncertainties associated with the duration and cost of clinical trials, which vary significantly over the life of a project as a result of unanticipated events arising during clinical development.

*General and Administrative Expenses*

General and administrative expenses increased by \$79,000, or 2%, from \$3,837,000 in the three months ended March 31, 2015, to \$3,916,000 in the three months ended March 31, 2016. General and administrative expenses consist primarily of salary expense, stock-based compensation expense, consulting fees and professional legal fees associated with our patent applications and maintenance, our corporate regulatory filing requirements, our corporate legal matters, and our business development initiatives.

The increase in general and administrative expenses during the three months ended March 31, 2016 reflects increases in salaries, stock-based compensation and legal fees associated with our patent filing and maintenance, offset by decreases in corporate legal fees and investor relations expenses as compared to the three months ended March 31, 2015.

## **Table of Contents**

### *Interest Income*

Interest income increased by \$79,000, from \$41,000 in the three months ended March 31, 2015 to \$120,000 in the three months ended March 31, 2016 primarily due to an increase in interest income resulting from higher investment balances in the three months ended March 31, 2016 resulting from the investment of funds obtained from our follow-on underwritten public offering in February 2015 and warrant and option exercises since March, 2015.

### *Interest Expense*

Interest expense decreased during the three months ended March 31, 2016, as compared to the three months ended March 31, 2015, primarily due to a decrease in the outstanding principal amount of our note under our loan and security agreement with Oxford Finance LLC.

### *Net Loss*

As a result of the factors discussed above, our net loss was \$12,823,000 for the three months ended March 31, 2016, compared to \$12,481,000 for the three months ended March 31, 2015. Since January 1, 2001, we have primarily been involved in the development of our TLR pipeline. From January 1, 2001 through March 31, 2016, we incurred losses of \$252,711,000. We also incurred net losses of \$260,193,000 prior to December 31, 2000 during which time we were primarily involved in the development of antisense technology. Since our inception, we had an accumulated deficit of \$512,904,000 through March 31, 2016. We expect to continue to incur substantial operating losses in the future.

## **LIQUIDITY AND CAPITAL RESOURCES**

### *Sources of Liquidity*

We require cash to fund our operating expenses and to make capital expenditures. Historically, we have funded our cash requirements primarily through the following:

sale of common stock, preferred stock and warrants and warrant exercises;

debt financing, including capital leases;

license fees, research funding and milestone payments under collaborative and license agreements; and

interest income.

We have an effective shelf registration statement on Form S-3 that permits us to offer and sell up to an additional \$113,750,000 of securities in one or more offerings.

### *Cash Flows*

#### *Three Months Ended March 31, 2016*

As of March 31, 2016, we had approximately \$74,122,000 in cash, cash equivalents and investments, a net decrease of approximately \$13,035,000 from December 31, 2015. Net cash used in operating activities totaled \$12,855,000 during the three months ended March 31, 2016, reflecting our \$12,823,000 net loss, as adjusted for non-cash income and expenses, including stock-based compensation, depreciation and amortization expense and accretion of investment premiums. Net cash used in operating activities also reflects changes in our prepaid expenses, accounts payable, accrued expenses and other liabilities and the recognition of deferred revenue.

The \$8,177,000 net cash provided by investing activities during the three months ended March 31, 2016 reflects the purchase of \$2,946,000 of available-for-sale securities, which are investments that we do not have the positive intent to hold to maturity at the time of purchase, proceeds from the maturity of \$9,239,000 of available-for-sale securities, proceeds from the sale of \$1,974,000 of available-for-sale securities, and payments for the purchase of \$90,000 in property and equipment.

---

**Table of Contents**

The \$29,000 net cash used by financing activities during the three months ended March 31, 2016 reflects \$36,000 in net proceeds from employee stock purchases under our 1995 Employee Stock Purchase Plan, or ESPP, and \$63,000 in payments on our note payable.

*Three Months Ended March 31, 2015*

As of March 31, 2015, we had approximately \$116,877,000 in cash, cash equivalents and investments, a net increase of approximately \$68,306,000 from December 31, 2014. Net cash used in operating activities totaled \$12,709,000 during the three months ended March 31, 2015, reflecting our \$12,481,000 net loss, as adjusted for non-cash income and expenses, including stock-based compensation, depreciation and amortization expense and accretion of investment premiums. Net cash used in operating activities also reflects changes in our prepaid expenses and accounts payable, accrued expenses and other liabilities.

The \$3,822,000 net cash provided by investing activities during the three months ended March 31, 2015 reflects the purchase of \$3,674,000 of available-for-sale securities, the maturity of \$6,602,000 of available-for-sale securities, the sale of \$999,000 of available-for-sale securities, and payments for the purchase of \$105,000 in property and equipment.

The \$81,189,000 net cash provided by financing activities during the three months ended March 31, 2015 primarily reflects \$81,070,000 in net proceeds from our follow-on underwritten public offering of our securities in February 2015 and \$121,000 in net proceeds from employee stock purchases under our ESPP, and the exercise of common stock options.

*Funding Requirements*

We have incurred operating losses in all fiscal years since our inception except 2002, 2008 and 2009, and we had an accumulated deficit of \$512,904,000 at March 31, 2016. We expect to incur substantial operating losses in future periods. These losses, among other things, have had and will continue to have an adverse effect on our stockholders equity, total assets and working capital. We have received no revenues from the sale of drugs. As of April 15, 2016, substantially all of our revenues have been from collaboration and license agreements. We have devoted substantially all of our efforts to research and development, including clinical trials, and we have not completed development of any drugs. Because of the numerous risks and uncertainties associated with developing drugs, we are unable to predict the extent of any future losses, whether or when any of our products will become commercially available or when we will become profitable, if at all.

We do not expect to generate significant additional funds internally until we successfully complete development and obtain marketing approval for products, either alone or in collaboration with third parties, which we expect will take a number of years. In addition, we have no committed external sources of funds.

We had cash, cash equivalents and investments of approximately \$74,122,000 at March 31, 2016. We believe that, based on our current operating plan, our existing cash, cash equivalents and investments will enable us to fund our operations into the third quarter of 2017. Specifically, we believe that our available funds will be sufficient to enable us to:

complete our ongoing Phase 1/2 clinical trial of IMO-8400 in patients with Waldenström's  
macroglobulinemia and our ongoing Phase 1/2 clinical trial of IMO-8400 in patients with DLBCL harboring

the MYD88 L265P oncogenic mutation;

continue our ongoing Phase 1/2 clinical trial of IMO-2125, administered intra-tumorally, in combination with ipilimumab in patients with metastatic melanoma and begin a second clinical trial with IMO-2125;

continue our ongoing Phase 2 clinical trial of IMO-8400 in patients with dermatomyositis;

review our strategic options related to the future development of IMO-9200; and

continue our ongoing clinical development program in the first gene target selected for further development in our 3GA program.

We expect that we will require substantial additional funds to conduct any additional research and development of our TLR drug candidates or 3GA technology, including preclinical testing and clinical trials of our drug candidates, and to fund our operations. We are seeking and expect to continue to seek additional funding through collaborations, the sale or license of assets or financings of equity or debt securities. We believe that the key factors that will affect our ability to obtain funding are:

the results of our clinical and preclinical development activities in our genetically defined forms of B-cell lymphoma and rare disease programs, our immuno-oncology program, and our 3GA program and our ability to advance our drug candidates and 3GA technology on the timelines anticipated;

the cost, timing, and outcome of regulatory reviews;



**Table of Contents**

competitive and potentially competitive products and technologies and investors' receptivity to our drug candidates and the technology underlying them in light of competitive products and technologies;

the receptivity of the capital markets to financings by biotechnology companies generally and companies with drug candidates and technologies such as ours specifically; and

our ability to enter into additional collaborations with biotechnology and pharmaceutical companies and the success of such collaborations.

In addition, increases in expenses or delays in clinical development may adversely impact our cash position and require additional funds or further cost reductions.

Financing may not be available to us when we need it or may not be available to us on favorable or acceptable terms or at all. We could be required to seek funds through collaborative alliances or through other means that may require us to relinquish rights to some of our technologies, drug candidates or drugs that we would otherwise pursue on our own. In addition, if we raise additional funds by issuing equity securities, our then existing stockholders will experience dilution. The terms of any financing may adversely affect the holdings or the rights of existing stockholders. An equity financing that involves existing stockholders may cause a concentration of ownership. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, and are likely to include rights that are senior to the holders of our common stock. Any additional debt or equity financing may contain terms which are not favorable to us or to our stockholders, such as liquidation and other preferences, or liens or other restrictions on our assets. As discussed in Note 10 to the financial statements appearing in our Annual Report on Form 10-K for the year ended December 31, 2015, additional equity financings may also result in cumulative changes in ownership over a three-year period in excess of 50% which would limit the amount of net operating loss and tax credit carryforwards that we may utilize in any one year.

If we are unable to obtain adequate funding on a timely basis or at all, we will be required to terminate, modify or delay preclinical or clinical trials of one or more of our drug candidates, significantly curtail or terminate discovery or development programs for new drug candidates or relinquish rights to portions of our technology, drug candidates and/or products.

*Contractual Obligations*

During the three months ended March 31, 2016, there were no material changes outside the ordinary course of our business to our contractual obligations as disclosed in our Annual Report on Form 10-K for the year ended December 31, 2015.

As of March 31, 2016, we had no off-balance sheet arrangements.

*New Accounting Pronouncements*

New accounting pronouncements are discussed in Note 2 in the notes to the financial statements in this Quarterly Report on Form 10-Q.

**ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK.**

As of March 31, 2016, all material assets and liabilities are in U.S. dollars, which is our functional currency.

We maintain investments in accordance with our investment policy. The primary objectives of our investment activities are to preserve principal, maintain proper liquidity to meet operating needs and maximize yields. Although our investments are subject to credit risk, our investment policy specifies credit quality standards for our investments and limits the amount of credit exposure from any single issue, issuer or type of investment. We regularly review our investment holdings in light of the then current economic environment. We do not own auction rate securities or derivative financial investment instruments in our investment portfolio. At March 31, 2016, all of our invested funds were invested in two money market funds and commercial paper, classified in cash and cash equivalents on the accompanying balance sheet, and corporate bonds and municipal bonds classified in short-term investments and long-term investments on the accompanying balance sheet.

Based on a hypothetical ten percent adverse movement in interest rates, the potential losses in future earnings, fair value of risk sensitive financial instruments, and cash flows are immaterial, although the actual effects may differ materially from the hypothetical analysis.

#### **ITEM 4. CONTROLS AND PROCEDURES.**

(a) *Evaluation of Disclosure Controls and Procedures.* Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-

**Table of Contents**

15(e) and 15d-15(e) under the Exchange Act) as of March 31, 2016. In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applied its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on this evaluation, our principal executive officer and principal financial officer concluded that as of March 31, 2016, our disclosure controls and procedures were (1) designed to ensure that material information relating to us is made known to our principal executive officer and principal financial officer by others, particularly during the period in which this report was prepared, and (2) effective, in that they provide reasonable assurance that information required to be disclosed by us in the reports we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms.

(b) *Changes in Internal Controls*. No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the fiscal quarter ended March 31, 2016 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Table of Contents

**PART II OTHER INFORMATION**

**ITEM 1A. RISK FACTORS.**

**RISK FACTORS**

*Investing in our securities involves a high degree of risk. You should carefully consider the risks and uncertainties described below in addition to the other information included or incorporated by reference in this Quarterly Report on Form 10-Q before purchasing our common stock. Our business, financial condition and results of operations could be materially and adversely affected by any of these and currently unknown risks or uncertainties. In that case, the market price of our common stock could decline, and you may lose all or part of your investment in our securities.*

**Risks Relating to Our Financial Results and Need for Financing**

*We will need additional financing, which may be difficult to obtain. Our failure to obtain necessary financing or doing so on unattractive terms could result in the termination of our operations and the sale and license of our assets or otherwise adversely affect our research and development programs and other operations.*

We had cash, cash equivalents and investments of approximately \$74.1 million at March 31, 2016. We believe that, based on our current operating plan, our existing cash, cash equivalents and investments will enable us to fund our operations into the third quarter of 2017. Specifically, we believe that our available funds will be sufficient to enable us to:

complete our ongoing Phase 1/2 clinical trial of IMO-8400 in patients with Waldenström's macroglobulinemia and our ongoing Phase 1/2 clinical trial of IMO-8400 in patients with DLBCL harboring the MYD88 L265P oncogenic mutation;

continue our ongoing Phase 1/2 clinical trial of IMO-2125, administered intra-tumorally, in combination with ipilimumab in patients with metastatic melanoma and begin a second clinical trial with IMO-2125;

continue our ongoing Phase 2 clinical trial of IMO-8400 in patients with dermatomyositis;

review our strategic options related to the future development of IMO-9200; and

continue our ongoing clinical development program in the first gene target selected for further development in our 3GA program.

We expect that we will require substantial additional funds to complete any additional research and development of our TLR drug candidates or 3GA technology, including preclinical testing and clinical trials of our drug candidates, and to fund our operations. We are seeking and expect to continue to seek additional funding through collaborations, the sale or license of assets or financings of equity or debt securities. We believe that the key factors that will affect our ability to obtain funding are:

the results of our clinical and preclinical development activities in our genetically defined forms of B-cell lymphoma and rare disease programs, our immuno-oncology program, and our 3GA program, and our ability to advance our drug candidates and 3GA technology on the timelines anticipated;

the cost, timing, and outcome of regulatory reviews;

competitive and potentially competitive products and technologies and investors' receptivity to our drug candidates and the technology underlying them in light of competitive products and technologies;

the receptivity of the capital markets to financings by biotechnology companies generally and companies with drug candidates and technologies such as ours specifically; and

our ability to enter into additional collaborations with biotechnology and pharmaceutical companies and the success of such collaborations.

In addition, increases in expenses or delays in clinical development may adversely impact our cash position and require additional funds or further cost reductions.

Financing may not be available to us when we need it or may not be available to us on favorable or acceptable terms or at all. We could be required to seek funds through collaborative alliances or through other means that may require us to relinquish rights to some of our technologies, drug candidates or drugs that we would otherwise pursue on our own. In addition, if we raise additional funds by issuing equity securities, our then existing stockholders will experience dilution. The terms of any financing may adversely affect the holdings or the rights of existing stockholders. An equity financing that involves existing stockholders may cause a concentration of ownership. Debt financing, if available, may involve agreements that include covenants limiting or restricting our

## **Table of Contents**

ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, and are likely to include rights that are senior to the holders of our common stock. Any additional debt or equity financing may contain terms which are not favorable to us or to our stockholders, such as liquidation and other preferences, or liens or other restrictions on our assets. As discussed in Note 10 to the financial statements appearing in our Annual Report on Form 10-K for the year ended December 31, 2015 that was filed with the SEC, additional equity financings may also result in cumulative changes in ownership over a three-year period in excess of 50% which would limit the amount of net operating loss and tax credit carryforwards that we may utilize in any one year.

If we are unable to obtain adequate funding on a timely basis or at all, we will be required to terminate, modify or delay preclinical or clinical trials of one or more of our drug candidates, significantly curtail or terminate discovery or development programs for new drug candidates or relinquish rights to portions of our technology, drug candidates and/or products.

***We have incurred substantial losses and expect to continue to incur losses. We will not be successful unless we reverse this trend.***

We have incurred losses in every year since our inception, except for 2002, 2008, and 2009 when our recognition of revenues under license and collaboration agreements resulted in our reporting net income for those years. As of March 31, 2016, we had an accumulated deficit of \$512.9 million. Since January 1, 2001, we have primarily been involved in the development of our TLR pipeline. From January 1, 2001 to March 31, 2016, we incurred losses of \$252.7 million. We incurred losses of \$260.2 million prior to December 31, 2000, during which time we were primarily involved in the development of earlier generation antisense technology. These losses, among other things, have had and will continue to have an adverse effect on our stockholders' equity, total assets, and working capital.

We have never had any products of our own available for commercial sale and have received no revenues from the sale of drugs. As of March 31, 2016, substantially all of our revenues have been from collaborative and license agreements. We have devoted substantially all of our efforts to research and development, including clinical trials, and have not completed development of any drug candidates. Because of the numerous risks and uncertainties associated with developing drugs, we are unable to predict the extent of any future losses, whether or when any of our drug candidates will become commercially available, or when we will become profitable, if at all. We expect to incur substantial operating losses in future periods.

### **Risks Relating to Our Business, Strategy and Industry**

***We are depending heavily on the development of TLR-targeted drug candidates for the treatment of certain genetically defined forms of B-cell lymphoma and rare diseases and in our immuno-oncology program and on the development of our 3GA technology. If we terminate the development of any of our programs or any of our drug candidates in such programs, are unable to successfully develop and commercialize any of our drug candidates, or experience significant delays in doing so, our business may be materially harmed.***

We have invested a significant portion of our time and financial resources in the development of TLR-targeted clinical-stage drug candidates as part of our rare disease program. In the future, we intend to invest a significant portion of our time and financial resources in the development of our TLR-targeted candidates for the treatment of certain genetically defined forms of B-cell lymphoma and rare diseases and in our immuno-oncology program. We also plan to invest substantial time and resources to further advance the development of drug candidates under our 3GA program. For instance:

we are conducting a Phase 1/2 clinical trial of IMO-8400 in patients with Waldenström's macroglobulinemia and a Phase 1/2 clinical trial of IMO-8400 in patients with DLBCL harboring the MYD88 L265P oncogenic mutation;

we are conducting a Phase 1/2 clinical trial of IMO-2125, administered intra-tumorally, in combination with ipilimumab in patients with metastatic melanoma;

we are conducting a Phase 2 clinical trial of IMO-8400 in patients with dermatomyositis;

we conducted a Phase 1 clinical trial of IMO-9200 in healthy subjects and are reviewing our strategic options related to the future development of IMO-9200; and

we are conducting a clinical development program in the first gene target selected for further development in our 3GA program.

We anticipate that our ability to generate product revenues will depend heavily on the successful development and commercialization of our TLR drug candidates in our genetically defined forms of B-cell lymphoma, rare disease and immuno-oncology programs, and the successful identification, development and commercialization of drug candidates in our 3GA program.

Our ability to generate product revenues under our collaborations with Merck & Co. and GSK, and under any other collaboration that we enter into with respect to our other programs, will depend on the development and commercialization of the drug candidates being developed.

**Table of Contents**

Our efforts, and the efforts of Merck & Co. and GSK, to develop and commercialize these compounds are at an early stage and are subject to many challenges. We have experienced setbacks with respect to our programs for IMO-2125 and IMO-2055. For example, in April 2011 we chose to delay initiation of our planned 12-week Phase 2 randomized clinical trial of IMO-2125 plus ribavirin in treatment-naïve, genotype 1 hepatitis C virus, or HCV, patients based on observations of lymphoproliferative malignancies in an ongoing 26-week chronic nonclinical toxicology study of IMO-2125 in rodents. We subsequently completed a 39-week chronic nonclinical toxicology study of IMO-2125 in non-human primates in which there were no similar observations.

We are conducting multiple clinical trials of IMO-8400 in different indications. If patients in any of these trials experience adverse safety events, we may be required to delay, discontinue or modify all of our clinical trials of IMO-8400.

We have entered into and expect to continue to seek to enter into collaborative alliances with pharmaceutical companies to advance our TLR antagonist candidates in broader autoimmune disease indications and with respect to additional applications of our 3GA technology program. Our previous setbacks with respect to our programs for IMO-2125 and IMO-2055 could negatively impact our ability to license any of such compounds to a third party.

Our ability to successfully develop and commercialize these drug candidates, or other potential candidates, will depend on our ability to overcome these recent challenges and on several factors, including the following:

the drug candidates demonstrating activity in clinical trials;

the drug candidates demonstrating an acceptable safety profile in nonclinical toxicology studies and during clinical trials;

timely enrollment in clinical trials of IMO-8400 and other drug candidates, which may be slower than anticipated, potentially resulting in significant delays;

satisfying conditions imposed on us and/or our collaborators by the FDA or equivalent foreign regulatory authorities regarding the scope or design of clinical trials;

the ability to demonstrate to the satisfaction of the FDA, or equivalent foreign regulatory authorities, the safety and efficacy of the drug candidates through current and future clinical trials;

timely receipt of necessary marketing approvals from the FDA and equivalent foreign regulatory authorities;

the ability to combine our drug candidates and the drug candidates being developed by Merck & Co. and any other collaborators safely and successfully with other therapeutic agents;



achieving and maintaining compliance with all regulatory requirements applicable to the products;

establishment of commercial manufacturing arrangements with third-party manufacturers;

the ability to secure orphan drug exclusivity for our drug candidates either alone or in combination with other products;

the successful commercial launch of the drug candidates, assuming FDA approval is obtained, whether alone or in combination with other products;

acceptance of the products as safe and effective by patients, the medical community, and third-party payors;

competition from other companies and their therapies;

changes in treatment regimens;

favorable market conditions in which to raise additional capital;

the strength of our intellectual property portfolio in the United States and abroad; and

a continued acceptable safety and efficacy profile of the drug candidates following marketing approval.

***We are developing drug candidates for use in the treatment of certain genetically defined forms of B-cell lymphoma. Our approach for the treatment of these genetically defined forms of B-cell lymphoma is novel and may not result in any approved and marketable products.***

We are in the early stages of developing our program in genetically defined forms of B-cell lymphoma, an area in which we have little experience. In connection with this program, we are focusing our efforts on the research and development of TLR antagonist drug candidates for use in the treatment of certain genetically defined forms of B-cell lymphoma. The scientific evidence to support the feasibility of developing drug candidates for this use is both preliminary and limited. We have conducted preclinical studies in human lymphoma cell lines that carry the MYD88 L265P oncogenic mutation to evaluate our TLR antagonists as a potential approach to the treatment of certain genetically defined forms of B-cell lymphoma. Although the preliminary results of our preclinical studies have been promising, it is unknown whether these results are indicative of results that may be obtained in our clinical trials. Therefore, we do not know if our approach of inhibiting TLRs to treat patients with genetically defined forms of B-cell lymphoma will

## **Table of Contents**

be successful or if we will ever succeed in obtaining regulatory approval to market any product for this purpose. In addition, in the event that our development efforts for such a drug candidate progress towards commercialization, we likely will need to develop companion diagnostics for such drug candidate. We have no experience in developing companion diagnostics and will be dependent on the efforts of third-party collaborators to successfully develop and commercialize these companion diagnostics on our behalf. In May 2014, we entered into an agreement with Abbott Molecular to develop a companion diagnostic for identification of patients with B-cell lymphoma harboring the MYD88 L265P oncogenic mutation. We cannot assume that the program under this agreement will be successful.

***We are in the early stages of developing our TLR9 agonists in combination with checkpoint inhibitors, which is a novel technology, and our efforts may not be successful or result in any approved and marketable products.***

In June 2015, we entered into a strategic clinical research alliance with MD Anderson to advance clinical development of TLR9 agonists in combination with checkpoint inhibitors. We initiated the first trial from the research alliance, a Phase 1/2 clinical trial to assess the safety and efficacy of IMO-2125, administered intra-tumorally in combination with ipilimumab, a CTLA4 antibody, in patients with metastatic melanoma in the fourth quarter of 2015. While we have evaluated the safety profile of IMO-2125 in previous trials and, as a marketed product, the safety profile of ipilimumab is known, the safety profile of the combination of IMO-2125 and ipilimumab has not been evaluated in previous trials and may result in participating subjects experiencing serious adverse events or undesirable side effects or exposure to unacceptable health risks requiring us to suspend or terminate any clinical trials that we may conduct of IMO-2125 in combination with ipilimumab, or any other checkpoint inhibitor.

***We are in the early stages of developing our 3GA program, which is a novel technology, and our efforts may not be successful or result in any approved and marketable products.***

We are in the early stages of developing our 3GA technology program, and the scientific evidence to support the feasibility of developing drugs based on this technology is preliminary. Further, neither we nor any other company has received regulatory approval to market therapeutics utilizing 3GA drug candidates.

The future success of our 3GA technology program depends on our success in identifying and developing marketable products based on such technology. Although the results of our preclinical studies to date have been supportive of the viability of this technology, it is unknown whether these results are indicative of results that may be obtained in any future clinical trials that we may conduct. We are currently undertaking an analysis of priority oncology and rare disease indications for development of drug candidates generated from our 3GA technology. We are developing 3GA compounds against two gene targets, NLRP3 and DUX4, which we have begun to advance into clinical development activities. However, many steps must be successfully achieved prior to the declaration of a 3GA drug candidate and the initiation of clinical development. Given the level of uncertainty of our ability to successfully achieve these many steps and the uncertainty of the drug discovery and clinical development processes in general, there can be no assurance that we will succeed in developing any marketable products as a result of our efforts with respect to our 3GA technology program.

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

We may not be able to initiate or continue clinical trials for our drug candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside the United States. For example, because there are a limited number of patients with Waldenström's macroglobulinemia or patients with DLBCL harboring the MYD88 L265P oncogenic mutation, and a limited number of patients with dermatomyositis, or other rare diseases having indications for which we may determine to develop our

TLR antagonists, our ability to enroll eligible patients in any clinical trials for these indications may be limited or may result in slower enrollment than we anticipate. In addition, the relapsed or refractory DLBCL patients that we are seeking to enroll in our Phase 1/2 clinical trial of IMO-8400 typically have late stage disease, which confers a very poor prognosis. As a result, some patients, who screen positive for the MYD88 L265P oncogenic mutation based on tumor tissue testing, may experience rapid deterioration that precludes them from meeting study eligibility criteria and they are unable to enroll into the trial. If enrolled, the disease in these patients may be too advanced for them to derive any clinically meaningful benefit from treatment or for their participation in the study to contribute meaningful data to the clinical trial. In addition, some of our competitors have ongoing clinical trials for drug candidates that treat the same indications as our drug candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' drug candidates.

Patient enrollment is affected by other factors including the:

severity of the disease under investigation;

eligibility criteria for the trial in question;

**Table of Contents**

perceived risks and benefits of the TLR antagonist drug candidates under study;

efforts to facilitate timely enrollment in clinical trials;

availability of competing clinical trials or other therapies;

patient referral practices of physicians;

ability to monitor patients adequately during and after treatment; and

proximity and availability of clinical trial sites for prospective patients.

Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our drug candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

***If our clinical trials are unsuccessful, or if they are delayed or terminated, we may not be able to develop and commercialize our drug candidates.***

In order to obtain regulatory approvals for the commercial sale of our drug candidates, we are required to complete extensive clinical trials in humans to demonstrate the safety and efficacy of our drug candidates. Clinical trials are lengthy, complex, and expensive processes with uncertain results. We may not be able to complete any clinical trial of a potential product within any specified time period. Moreover, clinical trials may not show our potential products to be both safe and efficacious. The FDA or other equivalent foreign regulatory agencies may not allow us to complete these trials or commence and complete any other clinical trials.

The results from preclinical testing of a drug candidate that is under development may not be predictive of results that will be obtained in human clinical trials. In addition, the results of early human clinical trials may not be predictive of results that will be obtained in larger scale, advanced stage clinical trials. Furthermore, interim results of a clinical trial do not necessarily predict final results, and failure of any of our clinical trials can occur at any stage of testing. Companies in the biotechnology and pharmaceutical industries, including companies with greater experience in preclinical testing and clinical trials than we have, have suffered significant setbacks in clinical trials, even after demonstrating promising results in earlier trials. Moreover, effects seen in nonclinical studies, even if not observed in clinical trials, may result in limitations or restrictions on clinical trials. Numerous unforeseen events may occur during, or as a result of, preclinical testing, nonclinical testing or the clinical trial process that could delay or inhibit the ability to receive regulatory approval or to commercialize drug products.

Other companies developing drugs targeted to TLRs have experienced setbacks in clinical trials. These setbacks may result in enhanced scrutiny by regulators or institutional review boards, or IRBs, of clinical trials of our drug candidates, including our TLR-targeted drug candidates, which could result in regulators or IRBs prohibiting the commencement of clinical trials, requiring additional nonclinical studies as a precondition to commencing clinical trials or imposing restrictions on the design or scope of clinical trials that could slow enrollment of trials, increase the costs of trials or limit the significance of the results of trials. Such setbacks could also adversely impact the desire of

investigators to enroll patients in, and the desire of patients to enroll in, clinical trials of our drug candidates.

Other events that could delay or inhibit conduct of our clinical trials include:

regulators or IRBs may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;

nonclinical or clinical data may not be readily interpreted, which may lead to delays and/or misinterpretation;

our nonclinical tests, including toxicology studies, or clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional nonclinical testing or clinical trials or we may abandon projects that we expect may not be promising;

the rate of enrollment or retention of patients in our clinical trials may be lower than we expect;

we might have to suspend or terminate our clinical trials if the participating subjects experience serious adverse events or undesirable side effects or are exposed to unacceptable health risks;

regulators or IRBs may hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements, issues identified through inspections of manufacturing or clinical trial operations or clinical trial sites, or if, in their opinion, the participating subjects are being exposed to unacceptable health risks;

regulators may hold or suspend our clinical trials while collecting supplemental information on, or clarification of, our clinical trials or other clinical trials, including trials conducted in other countries or trials conducted by other companies;

we, along with our collaborators and subcontractors, may not employ, in any capacity, persons who have been debarred under the FDA's Application Integrity Policy, or similar policy under foreign regulatory authorities. Employment of such debarred persons, even if inadvertent, may result in delays in the FDA's or foreign equivalent's review or approval of our drug candidates, or the rejection of data developed with the involvement of such person(s);

**Table of Contents**

we or our contract manufacturers may be unable to manufacture sufficient quantities of our drug candidates for use in clinical trials;

the cost of our clinical trials may be greater than we currently anticipate making continuation and/or completion improbable; and

our drug candidates may not cause the desired effects or may cause undesirable side effects or our drug candidates may have other unexpected characteristics.

We do not know whether clinical trials will begin as planned, will need to be restructured or will be completed on schedule, if at all. Significant clinical trial delays also could allow our competitors to bring products to market before we do and impair our ability to commercialize our drug candidates.

***Delays in commencing clinical trials of potential products could increase our costs, delay any potential revenues, and reduce the probability that a potential product will receive regulatory approval.***

Our drug candidates and our collaborators' drug candidates will require preclinical and other nonclinical testing and extensive clinical trials prior to submission of any regulatory application for commercial sales. In conducting clinical trials, we cannot be certain that any planned clinical trial will begin on time, if at all. Delays in commencing clinical trials of potential products could increase our drug candidate development costs, delay any potential revenues, reduce the potential length of patent exclusivity and reduce the probability that a potential product will receive regulatory approval.

Commencing clinical trials may be delayed for a number of reasons, including delays in:

manufacturing sufficient quantities of drug candidate that satisfy the required quality standards for use in clinical trials;

demonstrating sufficient safety to obtain regulatory approval for conducting a clinical trial;

reaching an agreement with any collaborators on all aspects of the clinical trial;

reaching agreement with contract research organizations, if any, and clinical trial sites on all aspects of the clinical trial;

resolving any objections from the FDA or any regulatory authority on an IND or proposed clinical trial design;

obtaining additional financing;

obtaining IRB approval for conducting a clinical trial at a prospective site; and

enrolling patients in order to commence the clinical trial.

***The technologies on which we rely are unproven and may not result in any approved and marketable products.***

Our technologies or therapeutic approaches are relatively new and unproven. We have focused our efforts on the research and development of RNA- and DNA-based compounds, or oligonucleotides, targeted to TLRs and on 3GA drug candidates. Neither we nor any other company have obtained regulatory approval to market such compounds as therapeutic drugs, and no such products currently are being marketed. The results of preclinical studies with TLR-targeted compounds may not be indicative of results that may be obtained in clinical trials, and results we have obtained in the clinical trials we have conducted to date may not be predictive of results in subsequent large-scale clinical trials. Further, the chemical and pharmacological properties of RNA- and DNA-based compounds targeted to TLRs or of 3GA drug candidates may not be fully recognized in preclinical studies and small-scale clinical trials, and such compounds may interact with human biological systems in unforeseen, ineffective or harmful ways that we have not yet identified.

Moreover, only one oligonucleotide drug, Kynamro<sup>®</sup>, has been approved by the FDA for marketing in the United States since 1998. As such, oligonucleotides as a chemical class of drug candidates have limited precedence for successful late-stage development and regulatory approval. As we progress our oligonucleotide drug candidates into Phase 2 clinical trials involving patients with severe disease and as we conduct long-term nonclinical toxicology studies, we expect to encounter an increased risk of generating clinical adverse events and nonclinical toxicology study results that will require careful interpretation. In animal toxicology studies, we have observed adverse treatment-related effects on serum complement as well as evidence of adverse kidney, vascular, and heart pathology in longer term dosing of animals with our oligonucleotide compounds, which we believe are consistent with data previously generated with other third party oligonucleotides. Given the limited experience in assessing the relevance of oligonucleotide-related adverse animal toxicology findings to humans, the clinical and regulatory context for interpreting the significance of such events and results is not well established.

## **Table of Contents**

As a result of these factors, we may never succeed in obtaining regulatory approval to market any product. Furthermore, the commercial success of any of our drug candidates for which we may obtain marketing approval from the FDA or other regulatory authorities will depend upon their acceptance by patients, the medical community, and third-party payors as clinically useful, safe, and cost-effective. In addition, if products being developed by our competitors have negative clinical trial results or otherwise are viewed negatively, the perception of our technologies and market acceptance of our drug candidates could be impacted negatively.

Our setbacks with respect to our TLR-targeted compounds, together with the setbacks experienced by other companies developing TLR-targeted compounds, may result in a negative perception of our technology and our TLR-targeted compounds, impact our ability to obtain marketing approval of these drug candidates and adversely affect acceptance of our technology and our TLR-targeted compounds by patients, the medical community and third-party payors.

Our efforts to educate the medical community on our potentially unique approaches may require greater resources than would be typically required for products based on conventional technologies or therapeutic approaches. The safety, efficacy, convenience, and cost-effectiveness of our drug candidates as compared to competitive products will also affect market acceptance.

***We face substantial competition, which may result in others discovering, developing or commercializing drugs before or more successfully than us.***

We are developing our TLR-targeted drug candidates for use in the treatment of certain genetically defined forms of B-cell lymphoma and rare diseases and in our immuno-oncology program. One of our drug candidates, IMO-8400, is in clinical development for the treatment of certain genetically defined forms of B-cell lymphoma, including Waldenström's macroglobulinemia and DLBCL harboring the MYD88 L265P oncogenic mutation. We initiated a Phase 2 clinical trial of IMO-8400 in patients with dermatomyositis in the fourth quarter of 2015. Through our clinical alliance partner MD Anderson, we also initiated a Phase 1/2 clinical trial of IMO-2125, administered intra-tumorally, in combination with ipilimumab, a CTLA4 antibody, in patients with metastatic melanoma in the fourth quarter of 2015. We also entered into a collaborative alliance agreement with GSK and expect to continue to seek to enter into collaborative alliances with pharmaceutical companies with respect to applications of our 3GA technology program. For all of these disease areas, there are many other companies, public and private, that are actively engaged in discovery, development, and commercializing products and technologies that may compete with our drug candidates and programs, including TLR-targeted compounds as well as non-TLR-targeted therapeutics.

We are developing IMO-8400 for the treatment of certain genetically defined forms of B-cell lymphoma. There are currently no drugs specifically approved for the treatment of Waldenström's macroglobulinemia or DLBCL harboring the MYD88 L265P oncogenic mutation. Ibrutinib, which is marketed as Imbruvica® by Pharmacyclics, Inc., was approved in January 2015 for the treatment of Waldenström's macroglobulinemia in the United States. Currently, patients with any form of non-Hodgkin lymphoma are most often treated with the monoclonal antibody rituximab and/or with one or more chemotherapeutic agents. Rituximab is co-marketed in the United States by Biogen Idec Inc., Genentech Inc. and Hoffmann-La Roche, and Chugai Pharmaceutical Co., Ltd. in territories outside the United States. We believe there are additional compounds in development for the treatment of genetically defined forms of B-cell lymphoma, including an inhibitor of interleukin-1 receptor-associated kinase 4, which is being developed by Nimbus Discovery, Inc.

We are aware that other companies including Dynavax, Checkmate Pharmaceuticals, Inc., or Checkmate, InDex Pharmaceuticals AB, Mologen AG, BioLineRx Ltd., Innate Immunotherapeutics Ltd., VentiRx Pharmaceuticals Inc., Telomedix S.A., Gilead Sciences Inc., GlaxoSmithKline plc, AstraZeneca plc and Hoffmann-La Roche may be



developing TLR agonists for various indications, some of which are in the field of oncology.

Immuno-oncology, which utilizes a patient's own immune system to combat cancer, is currently an active area of research for biotechnology and pharmaceutical companies. Interest in immuno-oncology is driven by recent efficacy data in cancers with historically bleak outcomes and the potential to achieve a cure or functional cure for some patients. As such, our efforts in this field will be competitive with a wide variety of different approaches. Any one of these competitive approaches may result in the development of novel technologies that are more effective, safer or less costly than any that we are developing. In addition, Dynavax is reportedly conducting a Phase 1/2 clinical trial of an investigational TLR9 agonist in combination with checkpoint inhibitors and Checkmate is reportedly conducting a Phase 1b clinical trial of an investigational TLR9 agonist in combination with a checkpoint inhibitor.

Many of the drug development programs in dermatomyositis are focusing on expanding the use of drugs approved in different indications through investigator sponsored studies such as the ongoing studies of the monoclonal antibodies, belimumab and tocilizumab. We are not aware of other new chemical or molecular entities being developed for the treatment of dermatomyositis.

## Table of Contents

We are also developing 3GA drug candidates that we have created using our proprietary technology, to inhibit the production of disease-associated proteins by targeting RNA. We also face competition from other companies working to develop novel drugs using technologies that may compete with our 3GA technology. We are aware of multiple companies that are developing technologies that use oligonucleotide-based compounds to inhibit the production of disease associated proteins. These technologies include, but are not limited to, antisense technology as well as RNAi. In the field of antisense technologies, we compete with multiple companies, including Ionis and its partners. Ionis is currently marketing an antisense drug, Kynamro, and reportedly has several antisense drug candidates in clinical trials. In the field of RNAi, our primary competition is with Alnylam and its partners. Alnylam is reportedly developing multiple RNAi-based technologies and has several drug candidates in clinical trials. Any of the competing companies may develop gene-silencing technologies more rapidly and more effectively than us, and antisense technology and RNAi may become the preferred technology for drugs that target RNA in order to inhibit the production of disease-associated proteins.

Some of these potentially competitive products have been in development or commercialized for years, in some cases by large, well established pharmaceutical companies. Many of the marketed products have been accepted by the medical community, patients, and third-party payors. Our ability to compete may be affected by the previous adoption of such products by the medical community, patients, and third-party payors. Additionally, in some instances, insurers and other third-party payors seek to encourage the use of generic products, which makes branded products, such as is planned for our drug candidates upon commercialization, potentially less attractive, from a cost perspective, to buyers.

We recognize that other companies, including large pharmaceutical companies, may be developing or have plans to develop products and technologies that may compete with ours. Many of our competitors have substantially greater financial, technical, and human resources than we have. In addition, many of our competitors have significantly greater experience than we have in undertaking preclinical studies and human clinical trials of new pharmaceutical products, obtaining FDA and other regulatory approvals of products for use in health care and manufacturing, and marketing and selling approved products. Our competitors may discover, develop or commercialize products or other novel technologies that are more effective, safer or less costly than any that we are developing. Our competitors may also obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours.

We anticipate that the competition with our drug candidates and technologies will be based on a number of factors including product efficacy, safety, availability, and price. The timing of market introduction of our drug candidates and competitive products will also affect competition among products. We expect the relative speed with which we can develop products, complete the clinical trials and approval processes, and supply commercial quantities of the products to the market to be important competitive factors. Our competitive position will also depend upon our ability to attract and retain qualified personnel, to obtain patent protection or otherwise develop proprietary products or processes, protect our intellectual property, and to secure sufficient capital resources for the period between technological conception and commercial sales.

***Competition for technical and management personnel is intense in our industry, and we may not be able to sustain our operations or grow if we are unable to attract and retain key personnel.***

Our success is highly dependent on the retention of principal members of our technical and management staff, including Mr. Vincent Milano and Dr. Sudhir Agrawal. Mr. Milano serves as our President and Chief Executive Officer, and Dr. Agrawal serves as our President of Research.

We are a party to employment agreements with Mr. Milano and Dr. Agrawal. Mr. Milano's employment agreement is terminable upon 15 days prior written notice at the election of either party and immediately in the event of a termination for cause (as defined therein). Dr. Agrawal's employment agreement expires on October 19, 2018, but

automatically extends annually for additional one-year periods. This agreement may be terminated by us or Dr. Agrawal for any reason or no reason at any time upon notice to the other party. We do not carry key man life insurance for Mr. Milano or Dr. Agrawal.

Furthermore, our future growth will require hiring a number of qualified technical and management personnel. Accordingly, recruiting and retaining such personnel in the future will be critical to our success. There is intense competition from other companies and research and academic institutions for qualified personnel in the areas of our activities. If we are not able to continue to attract and retain, on acceptable terms, the qualified personnel necessary for the continued development of our business, we may not be able to sustain our operations or growth.

**Table of Contents**

**Regulatory Risks**

*We are subject to comprehensive regulatory requirements, which are costly and time consuming to comply with; if we fail to comply with these requirements, we could be subject to adverse consequences and penalties.*

The testing, manufacturing, labeling, advertising, promotion, export, and marketing of our drug candidates are subject to extensive regulation by governmental authorities in Europe, the United States, and elsewhere throughout the world.

In general, submission of materials requesting permission to conduct clinical trials may not result in authorization by the FDA or any equivalent foreign regulatory agency to commence clinical trials. Further, permission to continue ongoing trials may be withdrawn by the FDA or other regulatory agencies at any time after initiation, based on new information available after the initial authorization to commence clinical trials or for other reasons. In addition, submission of an application for marketing approval to the relevant regulatory agency following completion of clinical trials may not result in the regulatory agency approving the application if applicable regulatory criteria are not satisfied, and may result in the regulatory agency requiring additional testing or information.

Even if we obtain regulatory approval for any of our drug candidates, we will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, current Good Manufacturing Practices, or cGMP, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians, advertising and promotion, and recordkeeping. Even if marketing approval of a drug candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a risk evaluation and mitigation strategy. If any of our drug candidates receives marketing approval, the accompanying label may limit the approved use of our drug in this way, which could limit sales of the product. For example, new cancer drugs frequently are indicated only for patient populations that have not responded to an existing therapy or have relapsed.

Both before and after approval is obtained, failure to comply with regulatory requirements, or discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, may result in:

the regulatory agency's delay in approving, or refusal to approve, an application for marketing of a product or a supplement to an approved application;

total or partial suspension of any ongoing clinical trials;

restrictions on our drug candidates or the marketing or manufacturing of our drug candidates;

warning letters or untitled letters;

voluntary or mandatory product recalls;

finer, restitution or disgorgement of profits or revenues;

suspension or withdrawal of regulatory approvals and our products from the market;

product seizure or detention;

refusal to permit the import or export of our drug candidates;

injunctions or the imposition of civil penalties; and

criminal penalties.

## **Table of Contents**

The regulatory requirements and policies may change and additional government regulations may be enacted for which we may also be required to comply. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or in other countries. If we or any current or future collaborator are not able to maintain regulatory compliance, we or such collaborator, as applicable, will not be permitted to market our future products and our business will suffer.

### ***We may not be able to obtain marketing approval for products resulting from our development efforts.***

All of the drug candidates that we are developing, or may develop in the future, will require additional research and development, extensive preclinical studies, nonclinical testing, clinical trials, and regulatory approval prior to any commercial sales. This process is lengthy, often taking a number of years, is uncertain, and is expensive. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the drug candidate's safety and purity. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities.

Since our inception, we have conducted clinical trials of a number of compounds and are planning to initiate clinical trials for a number of additional disease indications. Specifically, we are currently:

conducting a Phase 1/2 clinical trial of IMO-8400 in patients with Waldenström's macroglobulinemia and a Phase 1/2 clinical trial of IMO-8400 in patients with DLBCL harboring the MYD88 L265P oncogenic mutation;

conducting a Phase 1/2 clinical trial of IMO-2125, administered intra-tumorally, in combination with ipilimumab, a CTLA4 antibody, in patients with metastatic melanoma;

conducting a Phase 2 clinical trial of IMO-8400 in patients with dermatomyositis;

reviewing our strategic options related to the future development of IMO-9200; and

developing 3GA compounds against two gene targets, NLRP3 and DUX4, which we have begun to advance into clinical development activities.

The FDA and other regulatory authorities may not approve any of our potential products for any indication.

We may need to address a number of technological challenges in order to complete development of our drug candidates. Moreover, these products may not be effective in treating any disease or may prove to have undesirable or unintended side effects, unintended alteration of the immune system over time, toxicities or other characteristics that may preclude our obtaining regulatory approval or prevent or limit commercial use. In addition, changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require

additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a drug candidate. If we do not obtain necessary regulatory approvals, or there are delays in doing so, our business will be adversely affected.

*We may not be able to obtain orphan drug exclusivity for applications of our TLR antagonist drug candidates.*

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States.

Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the European Medicines Agency, or EMA, or the FDA from approving another marketing application for the same drug for the same indication for that exclusivity period. The applicable period is seven years in the United States and ten years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable such that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

The FDA has granted us orphan drug designation for IMO-8400 for the treatment of Waldenström's macroglobulinemia and the treatment of DLBCL. However, there can be no assurance that we will obtain orphan drug exclusivity for Waldenström's macroglobulinemia, DLBCL or any other disease indications for which we develop IMO-8400 or our other drug candidates. Even if

## **Table of Contents**

we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

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

We intend to seek fast track designation for some applications of our drug candidates. If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for FDA fast track designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular drug candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive fast track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program.

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

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

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

***If we are required by the FDA to obtain approval of a companion diagnostic in connection with and as a condition to approval of a drug candidate, and we do not obtain or we experience delays in obtaining FDA approval of a diagnostic device, we will not be able to commercialize the drug candidate and our ability to generate revenue will be materially impaired.***

We plan to develop companion diagnostics for our TLR antagonist drug candidates in our genetically defined forms of B-cell lymphoma program. We expect that, at least in some cases, the FDA and similar regulatory authorities outside the United States may require the development and regulatory approval of a companion diagnostic as a condition to approving our TLR antagonist drug candidates specifically for the treatment of patients with a genetically defined



form of B-cell lymphoma. According to FDA guidance, if the FDA determines that a companion diagnostic device is essential to the safe and effective use of a novel therapeutic product or indication, the FDA generally will not approve the therapeutic product or new therapeutic product indication if the companion diagnostic is not also approved or cleared for that indication. Under the Federal Food, Drug, Cosmetic Act, companion diagnostics are regulated as medical devices and the FDA has generally required companion diagnostics intended to select the patients who will respond to cancer treatment to obtain Premarket Approval, or a PMA. The PMA process, including the gathering of clinical and preclinical data and the submission to and review by the FDA, involves a rigorous premarket review during which the applicant must prepare and provide the FDA with reasonable assurance of the device's safety and effectiveness and information about the device and its components regarding, among other things, device design, manufacturing and labeling. PMA approval is not guaranteed and may take considerable time, and the FDA may ultimately respond to a PMA submission with a not approvable determination based on deficiencies in the application and require additional clinical trial or other data that may be expensive and time-consuming to generate and that can substantially delay approval.

We do not have experience or capabilities in developing or commercializing diagnostics and plan to rely on third parties or collaborators to perform these functions. In May 2014, we entered into an agreement with Abbott Molecular for the development and potential commercialization of a companion diagnostic for use with IMO-8400 with respect to our identification of patients with B-cell lymphoma harboring the MYD88 L265P oncogenic mutation in our genetically defined forms of B-cell lymphoma program. We may enter into similar agreements for our other drug candidates and possible expansion indications for IMO-8400. Companion diagnostics are subject to regulation by the FDA and similar regulatory authorities outside the United States as medical devices and require separate regulatory approval prior to commercialization.

**Table of Contents**

If we, any third parties that we engage to assist us or any of our collaborators are unable to successfully develop companion diagnostics for our TLR antagonist drug candidates, or experience delays in doing so:

the development of our TLR antagonist drug candidates may be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials;

our TLR antagonist drug candidates may not receive marketing approval if their safe and effective use depends on a companion diagnostic; and

we may not realize the full commercial potential of any TLR antagonist drug candidates that receive marketing approval if, among other reasons, we are unable to appropriately identify patients with the specific oncogenic mutation targeted by our TLR antagonist drug candidates.

If any of these events were to occur, our business would be harmed, possibly materially.

***We have only limited experience in regulatory affairs and our drug candidates are based on new technologies; these factors may affect our ability or the time we require to obtain necessary regulatory approvals.***

We have only limited experience in filing the applications necessary to obtain regulatory approvals. Moreover, the products that result from our research and development programs will likely be based on new technologies and new therapeutic approaches that have not been extensively tested in humans. The regulatory requirements governing these types of products may be more rigorous than for conventional drugs. As a result, we may experience a longer regulatory process in connection with obtaining regulatory approvals of any product that we develop.

***Failure to obtain regulatory approval in jurisdictions outside the United States will prevent us from marketing our products abroad.***

We intend to market our products, if approved, in markets outside the United States, which will require separate regulatory approvals and compliance with numerous and varying regulatory requirements. The approval procedures vary among such markets and may involve requirements for additional testing, and the time required to obtain approval may differ from that required to obtain FDA approval. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. The foreign regulatory approval process may include all of the risks associated with obtaining FDA approval. We may not obtain foreign regulatory approvals on a timely basis, if at all.

***Our relationships with healthcare providers, physicians and third party payors are subject, directly or indirectly, to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which, in the event of a violation, could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.***

Our arrangements with healthcare providers and physicians may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we for which we are seeking to obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

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

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

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

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

## **Table of Contents**

State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

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

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

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

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

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

an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription products and biologic agents;

an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;

a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for products that are inhaled, infused, instilled, implanted or injected;

expansion of healthcare fraud and abuse laws, including the civil False Claims Act and the federal Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;

a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand products to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient products to be covered under Medicare Part D;

extension of manufacturers' Medicaid rebate liability to individuals enrolled in Medicaid managed care organizations;

expansion of eligibility criteria for Medicaid programs;

expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;

new requirements to report certain financial arrangements with physicians and teaching hospitals;

a new requirement to annually report product samples that manufacturers and distributors provide to physicians;

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

## **Table of Contents**

a new Independent Payment Advisory Board, which has authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription products; and

established the Center for Medicare and Medicaid Innovation within the Centers for Medicare & Medicaid Services to test innovative payment and service delivery models.

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

### **Risks Relating to Collaborators**

*Our existing collaborations and any collaborations we enter into in the future may not be successful.*

Historically, an important element of our business strategy has included entering into collaborative alliances with corporate collaborators, primarily large pharmaceutical companies, for the development, commercialization, marketing, and distribution of some of our drug candidates. In December 2006, we entered into an exclusive license and research collaboration with Merck & Co. to research, develop, and commercialize vaccine products containing our TLR7, TLR8 and TLR9 agonists in the fields of cancer, infectious diseases, and Alzheimer's disease. In November 2015, we entered into a collaboration and license agreement with GSK for the development of our 3GA technology for certain renal indications. Additionally, in May 2014, we entered into a development and commercialization agreement with Abbott Molecular for the development of an in vitro companion diagnostic for use in our clinical development programs to treat certain genetically defined forms of B-cell lymphoma with IMO-8400.

Any collaboration that we enter into may not be successful. The success of our collaborative alliances, if any, will depend heavily on the efforts and activities of our collaborators. Our existing collaborations and any potential future collaborations have risks, including the following:

our collaborators may control the development of the drug candidates being developed with our technologies and compounds including the timing of development;

our collaborators may control the development of the companion diagnostic to be developed for use in conjunction with our drug candidates including the timing of development;

our collaborators may control the public release of information regarding the developments, and we may not be able to make announcements or data presentations on a schedule favorable to us;

disputes may arise in the future with respect to the ownership of rights to technology developed with our collaborators;

disagreements with our collaborators could delay or terminate the research, development or commercialization of products, or result in litigation or arbitration;

we may have difficulty enforcing the contracts if any of our collaborators fail to perform;

our collaborators may terminate their collaborations with us, which could make it difficult for us to attract new collaborators or adversely affect the perception of us in the business or financial communities;

our collaboration agreements are likely to be for fixed terms and subject to termination by our collaborators in the event of a material breach or lack of scientific progress by us;

our collaborators may have the first right to maintain or defend our intellectual property rights and, although we would likely have the right to assume the maintenance and defense of our intellectual property rights if our collaborators do not, our ability to do so may be compromised by our collaborators' acts or omissions;

our collaborators may challenge our intellectual property rights or utilize our intellectual property rights in such a way as to invite litigation that could jeopardize or invalidate our intellectual property rights or expose us to potential liability;

our collaborators may not comply with all applicable regulatory requirements, or may fail to report safety data in accordance with all applicable regulatory requirements;

our collaborators may change the focus of their development and commercialization efforts. Pharmaceutical and biotechnology companies historically have re-evaluated their priorities following mergers and consolidations, which have been common in recent years in these industries. For example, we have a strategic collaboration with Merck & Co., which merged with Schering-Plough Corporation, which has been involved with certain TLR-targeted research and development programs. Although the merger has not affected our collaboration with Merck & Co. to date, management of the combined

**Table of Contents**

company could determine to reduce the efforts and resources that the combined company will apply to its strategic collaboration with us or terminate the strategic collaboration. The ability of our drug candidates to reach their potential could be limited if our collaborators decrease or fail to increase spending relating to such drug candidates;

our collaborators may under fund or not commit sufficient resources to the testing, marketing, distribution or development of our drug candidates; and

our collaborators may develop alternative products either on their own or in collaboration with others, or encounter conflicts of interest or changes in business strategy or other business issues, which could adversely affect their willingness or ability to fulfill their obligations to us.

Given these risks, it is possible that any collaborative alliance into which we enter may not be successful.

Collaborations with pharmaceutical companies and other third parties often are terminated or allowed to expire by the other party. The termination or expiration of our agreement with Merck & Co., GSK or Abbott Molecular or any other collaboration agreement that we enter into in the future may adversely affect us financially and could harm our business reputation.

***If we are unable to establish additional collaborative alliances, our business may be materially harmed.***

Collaborators provide the necessary resources and drug development experience to advance our compounds in their programs. We are seeking and expect to continue to seek to enter into collaborative alliances with pharmaceutical companies to advance our TLR antagonist candidates in broader autoimmune disease indications. We are also continuing to seek to enter into collaborative alliances with pharmaceutical companies with respect to applications of our 3GA technology program.



## **Table of Contents**

Upfront payments and milestone payments received from collaborations help to provide us with the financial resources for our internal research and development programs. Our internal programs are focused on developing TLR-targeted drug candidates for the potential treatment of certain genetically defined forms of B-cell lymphoma and autoimmune diseases and on 3GA drug candidates. We believe that additional resources will be required to advance compounds in all of these areas. If we do not reach agreements with additional collaborators in the future, we may not be able to obtain the expertise and resources necessary to achieve our business objectives, our ability to advance our compounds will be jeopardized and we may fail to meet our business objectives.

We may have difficulty establishing additional collaborative alliances, particularly with respect to our TLR-targeted drug candidates and technology and our 3GA technology. For example, potential partners may note that our TLR collaborations with Novartis and with Merck KGaA have been terminated. Potential partners may also be reluctant to establish collaborations with respect to IMO-2125 and IMO-2055, given our setbacks with respect to these drug candidates. Additionally, in the event we seek collaborations for our 3GA program, any potential collaborators may not be willing to enter into a collaboration with us due to the early stage of this technology. We also face, and expect to continue to face, significant competition in seeking appropriate collaborators.

Even if a potential partner were willing to enter into a collaborative alliance with respect to our TLR-targeted compounds or technology or our 3GA technology, the terms of such a collaborative alliance may not be on terms that are favorable to us. Moreover, collaborations are complex and time consuming to negotiate, document, and implement. We may not be successful in our efforts to establish and implement collaborations on a timely basis.

### **Risks Relating to Intellectual Property**

*If we are unable to obtain and maintain patent protection for our discoveries, the value of our technology and products will be adversely affected.*

Our patent positions, and those of other drug discovery companies, are generally uncertain and involve complex legal, scientific, and factual questions. Our ability to develop and commercialize drugs depends in significant part on our ability to:

obtain and maintain valid and enforceable patents;

obtain licenses to the proprietary rights of others on commercially reasonable terms;

operate without infringing upon the proprietary rights of others;

prevent others from infringing on our proprietary rights; and

protect our trade secrets.

We do not know whether any of our patent applications or those patent applications that we license will result in the issuance of any patents. Our issued patents and those that may be issued in the future, or those licensed to us, may be challenged, invalidated, held unenforceable, narrowed in the course of a post-issuance proceeding or circumvented,

and the rights granted thereunder may not provide us proprietary protection or competitive advantages against competitors with similar technology. Moreover, intellectual property laws may change and negatively impact our ability to obtain issued patents covering our technologies or to enforce any patents that issue. Because of the extensive time required for development, testing, and regulatory review of a potential product, it is possible that, before any of our products can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thus reducing any advantage provided by the patent.

Because patent applications in the United States and many foreign jurisdictions are typically not published until 18 months after filing, or in some cases not at all, and because publications of discoveries in the scientific literature often lag behind actual discoveries, neither we nor our licensors can be certain that we or they were the first to make the inventions claimed in issued patents or pending patent applications, or that we or they were the first to file for protection of the inventions set forth in these patent applications.

As of April 15, 2016, we owned more than 45 U.S. patents and patent applications and more than 80 patents and patent applications throughout the rest of the world for our TLR-targeted immune modulation technologies. These patents and patent applications include claims covering the chemical compositions of matter and methods of use of our IMO compounds, such as IMO-8400, IMO-9200, IMO-2125 and IMO-2055, as well as other compounds. As of April 15, 2016, all of our intellectual property covering immune modulatory compositions and methods of their use is based on discoveries made solely by us. These patents expire at various dates ranging from 2017 to 2034. With respect to IMO-8400, we have an issued U.S. patent that covers the chemical composition of matter of IMO-8400 and certain methods of its use that has a statutory expiration date in 2031. With respect to IMO-9200, we have one U.S. patent application and one issued U.S. patent that cover the chemical composition for IMO-9200 and methods of its use. The issued patent has a statutory expiration date in 2034. With respect to IMO-2125, we have an issued U.S. patent that covers the chemical composition of matter of IMO-2125 and methods of its use that has a statutory expiration date in 2026. With respect to IMO-2055, we have issued patents that cover the chemical composition of matter of IMO-2055 and certain methods of its use, including in combination with marketed cancer products, with the composition claims expiring in 2023.

## **Table of Contents**

As of April 15, 2016, we owned two issued U.S. patents, 37 issued foreign patents, six pending U.S. patent applications and 12 foreign patent applications related to our 3GA compounds and methods of their use. The issued patents covering our 3GA technologies have statutory expiration dates in 2030 and 2031.

In addition to our TLR-targeted and 3GA patent portfolios, we are the owner of or hold licenses to patents and patent applications related to antisense technology. As of April 15, 2016, our antisense patent portfolio included more than five issued U.S. patents and more than 30 issued patents throughout the rest of the world. These antisense patents and patent applications include novel compositions of matter, the use of these compositions for various genes, sequences and therapeutic targets, and oral and other routes of administration. Some of the patents and patent applications in our antisense portfolio were in-licensed. These in-licensed patents expire at various dates through 2021.

***Third parties may own or control patents or patent applications and require us to seek licenses, which could increase our development and commercialization costs, or prevent us from developing or marketing products.***

Although we have many issued patents and pending patent applications in the United States and other countries, we may not have rights under certain third-party patents or patent applications related to our compounds under development. Third parties may own or control these patents and patent applications in the United States and abroad. In particular, we are aware of certain third-party U.S. patents that contain claims related to TLR modulation as well as antisense technology. Although we do not believe any of our TLR or antisense compounds under development infringe any valid claim of these patents, we cannot be assured that the holder of such patents would not seek to assert such patents against us or, if the holder did, that the courts would not interpret the claims of such patents more broadly than we believe appropriate and determine that we are in infringement of such patents. In addition, there may be other patents and patent applications related to our products of which we are not aware. Therefore, in some cases, in order to develop, manufacture, sell or import some of our products, we or our collaborators may choose to seek, or be required to seek, licenses under third-party patents issued in the United States and abroad or under third-party patents that might issue from U.S. and foreign patent applications. In such an event, we would be required to pay license fees or royalties or both to the licensor. If licenses are not available to us on acceptable terms, we or our collaborators may not be able to develop, manufacture, sell or import these products.

***We may become involved in expensive patent litigation or other proceedings, which could result in our incurring substantial costs and expenses or substantial liability for damages, require us to stop our development and commercialization efforts or result in our patents being invalidated, interpreted narrowly or limited.***

There has been substantial litigation and other proceedings regarding patent and other intellectual property rights in the biotechnology industry. We may become a party to various types of patent litigation or other proceedings regarding intellectual property rights from time to time even under circumstances where we are not practicing and do not intend to practice any of the intellectual property involved in the proceedings.

Other patent office proceedings include oppositions, reexaminations, supplemental examinations and *inter partes* reviews involving our patents or the patents of third parties. We may initiate such proceedings or have such proceedings brought against us. An adverse determination in any such proceeding, or in litigation, could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future drug candidates. An adverse determination in a proceeding involving a patent in our portfolio could result in the loss of protection or a narrowing in the scope of protection provided by that patent.

The cost to us of any patent litigation or other proceeding, even if resolved in our favor, could be substantial. Some of our competitors may be able to sustain the cost of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. If any patent litigation or other proceeding is resolved against us, we or our collaborators may be enjoined from developing, manufacturing, selling or importing our drugs without a license from the other party and we may be held liable for significant damages. We may not be able to obtain any required license on commercially acceptable terms or at all. In a patent office proceeding, such as an opposition, reexamination or *inter partes* review, our patents may be narrowed or invalidated.

Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace. Patent litigation and other proceedings may also absorb significant management time.

**Table of Contents**

**Risks Relating to Product Manufacturing, Marketing and Sales, and Reliance on Third Parties**

*Because we have limited manufacturing experience, and no manufacturing facilities or infrastructure, we are dependent on third-party manufacturers to manufacture drug candidates for us. If we cannot rely on third-party manufacturers, we will be required to incur significant costs and devote significant efforts to establish our own manufacturing facilities and capabilities.*

We have limited manufacturing experience and no manufacturing facilities, infrastructure or clinical or commercial scale manufacturing capabilities. In order to continue to develop our drug candidates, apply for regulatory approvals, and ultimately commercialize products, we need to develop, contract for or otherwise arrange for the necessary manufacturing capabilities.

We currently rely upon third parties to produce material for nonclinical and clinical testing purposes and expect to continue to do so in the future. We also expect to rely upon third parties to produce materials that may be required for the commercial production of our drug candidates, if approved. Our current and anticipated future dependence upon others for the manufacture of our drug candidates may adversely affect our future profit margins and our ability to develop drug candidates and commercialize any drug candidates on a timely and competitive basis. We currently do not have any long term supply contracts.

There are a limited number of manufacturers that operate under the FDA's cGMP regulations capable of manufacturing our drug candidates. As a result, we may have difficulty finding manufacturers for our drug candidates with adequate capacity for our needs. If we are unable to arrange for third-party manufacturing of our drug candidates on a timely basis, or to do so on commercially reasonable terms, we may not be able to complete development of our drug candidates or market them.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured drug candidates ourselves, including:

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

the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control;

the possibility of termination or nonrenewal of the agreement by the third party, based on its own business priorities or otherwise, at a time that is costly or inconvenient for us;

the potential that third-party manufacturers will develop know-how owned by such third party in connection with the production of our drug candidates that becomes necessary for the manufacture of our drug candidates; and

reliance upon third-party manufacturers to assist us in preventing inadvertent disclosure or theft of our proprietary knowledge.

Any contract manufacturers with which we enter into manufacturing arrangements will be subject to ongoing periodic, unannounced inspections by the FDA, or foreign equivalent, and corresponding state and foreign agencies or their designees to ensure compliance with cGMP requirements and other governmental regulations and corresponding foreign standards. Any failure by our third-party manufacturers to comply with such requirements, regulations or standards could lead to a delay in the conduct of our clinical trials, or a delay in, or failure to obtain, regulatory approval of any of our drug candidates. Such failure could also result in sanctions being imposed, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, product seizures or recalls, imposition of operating restrictions, total or partial suspension of production or distribution, or criminal prosecution.

Additionally, contract manufacturers may not be able to manufacture our drug candidates at a cost or in quantities necessary to make them commercially viable. As of April 15, 2016, our third-party manufacturers have met our manufacturing requirements, but we cannot be assured that they will continue to do so. Furthermore, changes in the manufacturing process or procedure, including a change in the location where the drug substance or drug product is manufactured or a change of a third-party manufacturer, may require prior FDA review and approval in accordance with the FDA's cGMP and New Drug Application/biologics license application regulations. Contract manufacturers may also be subject to comparable foreign requirements. This review may be costly and time-consuming and could delay or prevent the launch of a drug candidate. The FDA or similar foreign regulatory agencies at any time may also implement new standards, or change their interpretation and enforcement of existing standards for manufacture, packaging or testing of products. If we or our contract manufacturers are unable to comply, we or they may be subject to regulatory action, civil actions or penalties.

***We have no experience selling, marketing or distributing products and no internal capability to do so.***

If we receive regulatory approval to commence commercial sales of any of our drug candidates, we will face competition with respect to commercial sales, marketing, and distribution. These are areas in which we have no experience. To market any of our drug candidates directly, we would need to develop a marketing and sales force with technical expertise and with supporting distribution capability. In particular, we would need to recruit experienced marketing and sales personnel. Alternatively, we could engage a pharmaceutical or other healthcare company with an existing distribution system and direct sales force to assist us. However, to the extent we entered into such arrangements, we would be dependent on the efforts of third parties. If we are unable to establish sales and distribution capabilities, whether internally or in reliance on third parties, our business would suffer materially.

---

**Table of Contents**

***If third parties on whom we rely for clinical trials do not perform as contractually required or as we expect, we may not be able to obtain regulatory approval for or commercialize our drug candidates and our business may suffer.***

We do not have the ability to independently conduct the clinical trials required to obtain regulatory approval for our drug candidates. We depend on independent clinical investigators, contract research organizations, and other third-party service providers in the conduct of the clinical trials of our drug candidates and expect to continue to do so. We have contracted with contract research organizations to manage our ongoing Phase 1/2 clinical trial of IMO-8400 in patients with Waldenström's macroglobulinemia, our ongoing Phase 1/2 clinical trial of IMO-8400 in patients with DLBCL harboring the MYD88 L265P oncogenic mutation, our ongoing Phase 2 clinical trial of IMO-8400 in patients with dermatomyositis, and our Phase 1/2 clinical trial of IMO-2125, administered intra-tumorally, in combination with ipilimumab, in patients with metastatic melanoma and expect to contract with such organizations for future clinical trials. We rely heavily on these parties for successful execution of our clinical trials, but do not control many aspects of their activities. We are responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA and foreign regulatory agencies require us to comply with certain standards, commonly referred to as good clinical practices, and applicable regulatory requirements, for conducting, recording, and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity, and confidentiality of clinical trial participants are protected. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. Third parties may not complete activities on schedule, or at all, or may not conduct our clinical trials in accordance with regulatory requirements or our stated protocols. If these third parties fail to carry out their obligations, we may need to enter into new arrangements with alternative third parties. This could be difficult, costly or impossible, and our preclinical studies or clinical trials may need to be extended, delayed, terminated or repeated, and we may not be able to obtain regulatory approval in a timely fashion, or at all, for the applicable drug candidate, or to commercialize such drug candidate being tested in such studies or trials. If we seek to conduct any of these activities ourselves in the future, we will need to recruit appropriately trained personnel and add to our research, clinical, quality and corporate infrastructure.

***Failure of our third-party collaborators to successfully commercialize companion diagnostics developed for use with any TLR antagonist drug candidates that we develop with respect to our genetically defined forms of B-cell lymphoma program could harm our ability to commercialize these TLR antagonist drug candidates.***

Some of the TLR antagonist drug candidates that we develop with respect to our genetically defined forms of B-cell lymphoma program will necessitate the use of companion diagnostics. We do not plan to develop companion diagnostics internally and, as a result, we will be dependent on the efforts of our third-party collaborators to successfully commercialize these companion diagnostics. Our collaborators:

may not perform their obligations as expected;

may encounter production difficulties that could constrain the supply of the companion diagnostics;

may have difficulties gaining acceptance of the use of the companion diagnostics in the clinical community;

may not pursue commercialization of any companion diagnostics that achieve regulatory approval;

may elect not to continue or renew commercialization programs based on changes in the collaborators strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;

may not commit sufficient resources to the marketing and distribution of such companion diagnostics; and

may terminate their relationship with us.

If companion diagnostics for use with our genetically defined forms of B-cell lymphoma TLR antagonist drug candidates fail to gain market acceptance, our ability to derive revenues from sales of these TLR antagonist drug candidates, if approved, could be harmed. If our collaborators fail to commercialize these companion diagnostics, we may not be able to enter into arrangements with another diagnostic company to obtain supplies of an alternative diagnostic test for use in connection with genetically defined forms of B-cell lymphoma TLR antagonist drug candidates or do so on commercially reasonable terms, which could adversely affect and delay the development or commercialization of these TLR antagonist drug candidates.

***The commercial success of any drug candidates that we may develop will depend upon the degree of market acceptance by physicians, patients, third-party payors, and others in the medical community.***

Any products that we ultimately bring to the market, if they receive marketing approval, may not gain market acceptance by physicians, patients, third-party payors or others in the medical community. For example, current cancer treatments like chemotherapy and radiation therapy are well established in the medical community, and doctors may continue to rely on these treatments. If our products do not achieve an adequate level of acceptance, we may not generate product revenue and we may not become profitable. The degree of market acceptance of our products, if approved for commercial sale, will depend on a number of factors, including:



**Table of Contents**

the prevalence and severity of any side effects, including any limitations or warnings contained in the product's approved labeling;

the efficacy and potential advantages over alternative treatments;

the ability to offer our drug candidates for sale at competitive prices;

relative convenience and ease of administration;

the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;

the strength of marketing and distribution support and the timing of market introduction of competitive products; and

publicity concerning our products or competing products and treatments.

Even if a potential product displays a favorable efficacy and safety profile, market acceptance of the product will not be known until after it is launched. Our efforts to educate patients, the medical community, and third-party payors on the benefits of our drug candidates may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by conventional technologies marketed by our competitors.

***If we are unable to obtain adequate reimbursement from third-party payors for any products that we may develop or acceptable prices for those products, our revenues and prospects for profitability will suffer.***

Most patients rely on Medicare, Medicaid, private health insurers, and other third-party payors to pay for their medical needs, including any drugs we may market. If third-party payors do not provide adequate coverage or reimbursement for any products that we may develop, our revenues and prospects for profitability will suffer. Congress enacted a limited prescription drug benefit for Medicare recipients in the Medicare Prescription Drug, Improvement, and Modernization Act of 2003. While the program established by this statute may increase demand for our products if we were to participate in this program, our prices will be negotiated with drug procurement organizations for Medicare beneficiaries and are likely to be lower than we might otherwise obtain. Non-Medicare third-party drug procurement organizations may also base the price they are willing to pay on the rate paid by drug procurement organizations for Medicare beneficiaries or may otherwise negotiate the price they are willing to pay.

A primary trend in the United States healthcare industry is toward cost containment. In addition, in some foreign countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take six months or longer after the receipt of regulatory marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost effectiveness of our drug candidates or products to other available therapies. The conduct of such a clinical trial could be expensive and result in delays in commercialization of our drug candidates. These further clinical trials would require additional time,

resources, and expenses. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our prospects for generating revenue, if any, could be adversely affected and our business may suffer.

In March 2010, the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act became law. These health care reform laws are intended to broaden access to health insurance; reduce or constrain the growth of health care spending, especially Medicare spending; enhance remedies against fraud and abuse; add new transparency requirements for health care and health insurance industries; impose new taxes and fees on certain sectors of the health industry; and impose additional health policy reforms. Among the new fees is an annual assessment on makers of branded pharmaceuticals and biologics, under which a company's assessment is based primarily on its share of branded drug sales to federal health care programs. Such fees could affect our future profitability. Although it is too early to determine the effect of the health care legislation on our future profitability and financial condition, the law appears likely to continue the pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

Third-party payors are challenging the prices charged for medical products and services, and many third-party payors limit reimbursement for newly-approved health care products. These third-party payors may base their coverage and reimbursement on the coverage and reimbursement rate paid by carriers for Medicare beneficiaries. Furthermore, many such payors are investigating or implementing methods for reducing health care costs, such as the establishment of capitated or prospective payment systems. Cost containment pressures have led to an increased emphasis on the use of cost-effective products by health care providers. In particular, third-party payors may limit the indications for which they will reimburse patients who use any products that we may develop. Cost control initiatives could limit the price we might establish for products that we or our current or future collaborators may develop or sell, which would result in lower product revenues or royalties payable to us.

**Table of Contents**

*We face a risk of product liability claims and may not be able to obtain insurance.*

Our business exposes us to the risk of product liability claims that is inherent in the manufacturing, testing, and marketing of human therapeutic drugs. We face an inherent risk of product liability exposure related to the testing of our drug candidates in human clinical trials and will face an even greater risk if we commercially sell any products. Regardless of merit or eventual outcome, liability claims and product recalls may result in:

decreased demand for our drug candidates and products;

damage to our reputation;

regulatory investigations that could require costly recalls or product modifications;

withdrawal of clinical trial participants;

costs to defend related litigation;

substantial monetary awards to clinical trial participants or patients, including awards that substantially exceed our product liability insurance, which we would then have to pay using other sources, if available, and would damage our ability to obtain liability insurance at reasonable costs, or at all, in the future;

loss of revenue;

the diversion of management's attention away from managing our business; and

the inability to commercialize any products that we may develop.

Although we have product liability and clinical trial liability insurance that we believe is adequate, this insurance is subject to deductibles and coverage limitations. We may not be able to obtain or maintain adequate protection against potential liabilities. If we are unable to obtain insurance at acceptable cost or otherwise protect against potential product liability claims, we will be exposed to significant liabilities, which may materially and adversely affect our business and financial position. These liabilities could prevent or interfere with our commercialization efforts.

**Risks Relating to Ownership of Our Common Stock**

*Our corporate governance structure, including provisions in our certificate of incorporation and by-laws and Delaware law, may prevent a change in control or management that stockholders may consider desirable.*

Section 203 of the Delaware General Corporation Law and our certificate of incorporation and by-laws contain provisions that might enable our management to resist a takeover of our company or discourage a third party from attempting to take over our company. These provisions include:

a classified board of directors;

limitations on the removal of directors;

limitations on stockholder proposals at meetings of stockholders;

the inability of stockholders to act by written consent or to call special meetings; and

the ability of our board of directors to designate the terms of and issue new series of preferred stock without stockholder approval.

In addition, Section 203 of the Delaware General Corporation Law imposes restrictions on our ability to engage in business combinations and other specified transactions with significant stockholders. These provisions could have the effect of delaying, deferring or preventing a change in control of us or a change in our management that stockholders may consider favorable or beneficial. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors and take other corporate actions. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock.

***We have two significant securityholders. If these securityholders choose to act together, they could exert substantial influence over our business. In addition, in connection with any merger, consolidation or sale of all or substantially all of our assets, they would be entitled to receive consideration in excess of their reported beneficial ownership of our common stock.***

As of March 31, 2016, Baker Bros. Advisors LP, and certain of its affiliated funds, which we refer to collectively as Baker Brothers, held 6,990,189 shares of our common stock, warrants to purchase up to 20,316,327 shares of our common stock at an exercise price of \$0.47 per share and pre-funded warrants to purchase up to 22,151,052 shares of our common stock at an exercise price of \$0.01 per share. In addition, two members of our board of directors are affiliates of Baker Brothers. Under the terms of the

---

**Table of Contents**

warrants and pre-funded warrants issued to Baker Brothers, Baker Brothers is not permitted to exercise such warrants to the extent that such exercise would result in Baker Brothers (and its affiliates) beneficially owning more than 4.999% of the number of shares of our common stock outstanding immediately after giving effect to the issuance of shares of common stock issuable upon exercise of such warrants. Baker Brothers has the right to increase this beneficial ownership limitation in its discretion on 61 days prior written notice to us, provided that in no event is Baker Brothers permitted to exercise such warrants to the extent that such exercise would result in Baker Brothers (and its affiliates) beneficially owning more than 19.99% of the number of shares of our common stock outstanding or the combined voting power of our securities outstanding immediately after giving effect to the issuance of shares of common stock issuable upon exercise of such warrants. After giving effect to the 4.999% beneficial ownership limitation currently in effect with respect to the warrants and pre-funded warrants held by Baker Brothers, as of March 31, 2016, Baker Brothers beneficially owned 5.9% of our outstanding common stock. If the warrants and pre-funded warrants held by Baker Brothers could be exercised without this limitation, then as of March 31, 2016, Baker Brothers would have beneficially owned 30.3% of our common stock. The information in this paragraph is based on a Schedule 13G filed with the SEC on February 16, 2016 and on information provided to us by Baker Brothers. On February 9, 2015, we entered into a registration rights agreement with Baker Brothers, pursuant to which we agreed to file a registration statement to register for resale the shares of our common stock, including shares issuable upon the exercise of warrants, held by Baker Brothers. We filed this registration statement in the first quarter of 2016.

As of March 31, 2016, entities affiliated with Pillar Invest Corporation, which we refer to collectively as the Pillar Investment Entities, held 18,455,578 shares of our common stock and warrants to purchase up to 11,962,731 shares of our common stock at exercise prices ranging from \$0.47 per share to \$1.46 per share. In addition, one member of our board of directors is an affiliate of the Pillar Investment Entities. The Pillar Investment Entities are subject to contractual limitations that limit their ability to exercise any securities held by them that are exercisable into shares of our common stock to the extent that such exercise would result in the Pillar Investment Entities and their affiliates beneficially owning more than 19.99% of the number of shares of our common stock outstanding or the combined voting power of our securities outstanding immediately after giving effect to the issuance of shares of common stock issuable upon exercise of such securities. After giving effect to the 19.99% beneficial ownership limitation currently in effect with respect to the securities held by the Pillar Investment Entities, as of March 31, 2016, the Pillar Investment Entities beneficially owned 19.99% of our outstanding common stock. If the warrants held by the Pillar Investment Entities could be exercised without these limitations, then as of March 31, 2016, the Pillar Investment Entities would have beneficially owned 22.9% of our common stock. The information in this paragraph is based on information provided to us by the Pillar Investment Entities.

Although there are contractual limitations on the beneficial ownership of Baker Brothers and the Pillar Investment Entities, which we refer to collectively as our significant securityholders, if our significant securityholders were to exercise their warrants for common stock and were to choose to act together, they could be able to exert substantial influence over our business. This concentration of voting power could delay, defer or prevent a change of control, entrench our management and the board of directors or delay or prevent a merger, consolidation, takeover or other business combination involving us on terms that other stockholders may desire. In addition, conflicts of interest could arise in the future between us, on the one hand, and either or both of our significant securityholders on the other hand, concerning potential competitive business activities, business opportunities, the issuance of additional securities and other matters. Furthermore in the event of a sale of our company, whether by merger, sale of all or substantially all of our assets or otherwise, our significant securityholders would be entitled to receive, with respect to each share of common stock issuable upon exercise of the warrants then held by them and without regard to the beneficial ownership limitations imposed on the conversion or exercise of such securities, the same amount and kind of securities, cash or property as they would have been entitled to receive if such securities had been converted into or exercised for shares of our common stock immediately prior to such sale of our company. Because the significant

securityholders would receive this sale consideration with respect to warrants not included in their reported beneficial ownership of our common stock, in the event of a sale of our company, they would be entitled to receive a significantly larger portion of the total proceeds distributable to the holders of our securities than is represented by their reported beneficial ownership of our common stock.

***Our stock price has been and may in the future be extremely volatile. In addition, because our common stock has historically been traded at low volume levels, our investors' ability to trade our common stock may be limited. As a result, investors may lose all or a significant portion of their investment.***

Our stock price has been volatile. During the period from January 1, 2015 to April 15, 2016, the closing sales price of our common stock ranged from a high of \$5.37 per share to a low of \$1.64 per share. The stock market has also experienced periods of significant price and volume fluctuations and the market prices of biotechnology companies in particular have been highly volatile, often for reasons that have been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

our cash resources;

timing and results of nonclinical studies and clinical trials of our drug candidates or those of our competitors;

**Table of Contents**

the regulatory status of our drug candidates;

failure of any of our drug candidates, if approved, to achieve commercial success;

the success of competitive products or technologies;

regulatory developments in the United States and foreign countries;

our success in entering into collaborative agreements;

developments or disputes concerning patents or other proprietary rights;

the departure of key personnel;

our ability to maintain the listing of our common stock on The Nasdaq Capital Market or an alternative national securities exchange;

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

the terms of any financing consummated by us;

changes in the structure of healthcare payment systems;

market conditions in the pharmaceutical and biotechnology sectors and issuance of new or changed securities analysts' reports or recommendations; and

general economic, industry, and market conditions.

In addition, our common stock has historically been traded at low volume levels and may continue to trade at low volume levels. As a result, any large purchase or sale of our common stock could have a significant impact on the price of our common stock and it may be difficult for investors to sell our common stock in the market without depressing the market price for the common stock or at all.

As a result of the foregoing, investors may not be able to resell their shares at or above the price they paid for such shares. Investors in our common stock must be willing to bear the risk of fluctuations in the price of our common stock and the risk that the value of their investment in our stock could decline.

***Because we do not intend to pay dividends on our common stock, investor returns will be limited to any increase in the value of our stock.***

We have never declared or paid any cash dividends on our common stock. In addition, under the terms of our loan and security agreement with Oxford Finance LLC, we are required to obtain the prior written consent of Oxford Finance LLC in order to declare or pay a cash dividend on our common stock in an amount in excess of \$500,000 in any fiscal year. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business and do not anticipate declaring or paying any cash dividends on our common stock for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock, if any.

**ITEM 6. EXHIBITS.**

The list of Exhibits filed as part of this Quarterly Report on Form 10-Q is set forth on the Exhibit Index immediately preceding such Exhibits and is incorporated herein by this reference.



**Table of Contents**

**SIGNATURES**

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

**IDERA PHARMACEUTICALS, INC.**

Date: May 9, 2016

/s/ Vincent J. Milano  
Vincent J. Milano  
President and Chief Executive Officer

(Principal Executive Officer)

Date: May 9, 2016

/s/ Louis J. Arcudi, III  
Louis J. Arcudi, III  
Chief Financial Officer

(Principal Financial and Accounting Officer)

**Exhibit Index**

**Exhibit No.**

10.1	Employment Letter, dated June 5, 2015, by and between Idera Pharmaceuticals, Inc. and Mark J. Casey.
31.1	Certification of Chief Executive Officer pursuant to Exchange Act Rules 13a-14 and 15d-14, as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002.
31.2	Certification of Chief Financial Officer pursuant to Exchange Act Rules 13a-14 and 15d-14, as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002.
32.1	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.
32.2	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.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

