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

FORM 10-Q

(Mark One)

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

FOR THE QUARTERLY PERIOD ENDED MARCH 31, 2015

OR

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

FOR THE TRANSITION PERIOD FROM _____ TO _____

Commission File Number 001-36500

CymaBay Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction of
incorporation or organization)

7999 Gateway Blvd, Suite 130
Newark, CA
(Address of principal executive offices)

94-3103561
(I.R.S. Employer
Identification No.)

94560
(Zip Code)

(510) 293-8800

(Registrant's telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No

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

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

Large accelerated filer

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

As of April 30, 2015, there were 15,259,003 shares of the registrant's Common Stock outstanding.



[Table of Contents](#)

**CYMABAY THERAPEUTICS, INC.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTERLY PERIOD ENDED MARCH 31, 2015**

TABLE OF CONTENTS

	<u>Page</u>
PART I	
FINANCIAL INFORMATION	
Item 1.	
Financial Statements	3
Condensed Balance Sheets — March 31, 2015 (unaudited) and December 31, 2014	3
Condensed Statements of Operations and Comprehensive Loss for the three months ended March 31, 2015 and 2014 (unaudited)	4
Condensed Statements of Cash Flows for the three months ended March 31, 2015 and 2014 (unaudited)	5
Notes to Condensed Financial Statements (unaudited)	6
Item 2.	
Management’s Discussion and Analysis of Financial Condition and Results of Operations	16
Item 3.	
Quantitative and Qualitative Disclosures About Market Risk	21
Item 4.	
Controls and Procedures	21
PART II	
OTHER INFORMATION	
Item 1A.	
Risk Factors	21
Item 2.	
Unregistered Sales of Equity Securities and Use of Proceeds	44
Item 6.	
Exhibits	45
Signatures	46

[Table of Contents](#)

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

CymaBay Therapeutics, Inc.
Condensed Balance Sheets
(In thousands, except share and per share amounts)

	<u>March 31,</u> <u>2015</u>	<u>December 31,</u> <u>2014</u>
	(unaudited)	(Note 2)
Assets		
Current assets:		
Cash and cash equivalents	\$ 10,841	\$ 11,586
Marketable securities	21,950	23,209
Contract receivables	—	211
Accrued interest receivable	199	136
Prepaid expenses	1,363	1,991
Other current assets	139	96
Total current assets	34,492	37,229
Property and equipment, net	80	86
Other assets	158	159
Total assets	<u>\$ 34,730</u>	<u>\$ 37,474</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 1,527	\$ 2,085
Accrued liabilities	2,998	3,388
Warrant liability	7,704	13,596
Facility loan	1,403	1,355
Accrued interest payable	32	35
Total current liabilities	13,664	20,459
Facility loan, less current portion	2,784	3,152
Other liabilities	14	13
Total liabilities	16,462	23,624
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.0001 par value: 10,000,000 shares authorized; no shares issued and outstanding	—	—
Common stock, \$0.0001 par value: 100,000,000 shares authorized; 15,249,300 and 14,696,108 shares issued and outstanding as of March 31, 2015 and December 31, 2014, respectively	1	1
Additional paid-in capital	401,361	394,622
Accumulated other comprehensive loss	(7)	(14)
Accumulated deficit	(383,087)	(380,759)
Total stockholders' equity	18,268	13,850
Total liabilities and stockholders' equity	<u>\$ 34,730</u>	<u>\$ 37,474</u>

See accompanying notes.

[Table of Contents](#)

CymaBay Therapeutics, Inc.
Condensed Statements of Operations and Comprehensive Loss
(In thousands, except share and per share information)
(unaudited)

	Three Months Ended March 31,	
	2015	2014
Operating expenses:		
Research and development	\$ 4,187	\$ 2,615
General and administrative	2,589	2,500
Total operating expenses	6,776	5,115
Loss from operations	(6,776)	(5,115)
Other income (expense):		
Interest income	27	12
Interest expense	(154)	(184)
Other income (expense), net	4,575	(4,775)
Net loss	\$ (2,328)	\$ (10,062)
Net loss	(2,328)	(10,062)
Other comprehensive income:		
Unrealized gain on marketable securities	7	1
Other comprehensive income	7	1
Comprehensive loss	\$ (2,321)	\$ (10,061)
Basic net loss per common share	\$ (0.15)	\$ (1.02)
Diluted net loss per common share	\$ (0.44)	\$ (1.02)
Weighted average common shares outstanding used to calculate basic net loss per common share	15,099,567	9,873,687
Weighted average common shares outstanding used to calculate diluted net loss per common share	15,743,167	9,873,687

See accompanying notes.

[Table of Contents](#)

CymaBay Therapeutics, Inc.
Condensed Statements of Cash Flows
(In thousands)
(unaudited)

	Three Months Ended	
	March 31,	
	2015	2014
Operating activities		
Net loss	\$(2,328)	\$(10,062)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	6	3
Non-employee stock-based compensation expense	28	2
Employee and director stock-based compensation expense	705	714
Amortization of premium on marketable securities	167	71
Non-cash interest associated with debt discount accretion	36	49
Change in fair value of warrant liability	(4,575)	4,784
Changes in assets and liabilities:		
Contract receivables	211	(58)
Accrued interest receivable	(63)	(22)
Prepaid expenses	628	(1,512)
Other assets	(42)	(211)
Accounts payable	(558)	372
Accrued liabilities	(390)	830
Accrued interest payable	15	26
Other liabilities	1	3
Net cash used in operating activities	(6,159)	(5,011)
Investing activities		
Purchases of property and equipment	—	(32)
Purchases of marketable securities	(6,617)	—
Proceeds from sales and maturities of marketable securities	7,716	582
Net cash provided by investing activities	1,099	550
Financing activities		
Proceeds from issuance of common stock, net of issuance costs	4,263	2,402
Proceeds from issuance of common stock upon warrant exercises	426	—
Repayment of loan principal	(374)	—
Net cash provided by financing activities	4,315	2,402
Net decrease in cash and cash equivalents	(745)	(2,059)
Cash and cash equivalents at beginning of period	11,586	24,401
Cash and cash equivalents at end of period	<u>\$10,841</u>	<u>\$ 22,342</u>
Supplemental disclosure of cash flow information		
Cash paid for interest	\$ 101	\$ 109
Fixed assets in accrued expenses	—	28
Issuance of common stock warrants	—	443
Issuance of common stock upon warrant exercises	1,317	55
Noncash issuance costs incurred in common stock financing	—	453

See accompanying notes.

[Table of Contents](#)

CymaBay Therapeutics, Inc.
Notes to Condensed Financial Statements
(unaudited)

1. Organization and Description of Business

CymaBay Therapeutics, Inc. (the “Company” or “CymaBay”) is a biopharmaceutical company focused on developing therapies to treat metabolic diseases with high unmet medical need, including serious rare and orphan disorders. The Company’s lead product candidate, arhalofenate, is being developed for the treatment of gout. The Company’s second product candidate, MBX-8025, is being considered for the treatment of certain orphan diseases. The Company was incorporated in Delaware in October 1988 as Transtech Corporation. The Company’s headquarters and operations are located in Newark, California and it operates in one segment.

Liquidity

The Company has incurred net operating losses and negative cash flows from operations since its inception. During the three months ended March 31, 2015, the Company incurred a loss from operations of \$2.3 million and used \$6.2 million of cash in operations. At March 31, 2015, the Company had an accumulated deficit of \$383.1 million. CymaBay expects to incur increased research and development expenses as it continues to study its product candidates in clinical trials. To date, none of the Company’s product candidates have been approved for marketing and sale, and the Company has not recorded any product sales. As a result, management expects operating losses to continue in future years. The Company’s ability to achieve profitability is dependent primarily on its ability to successfully develop, acquire or in-license additional product candidates, continue clinical trials for product candidates currently in clinical development, obtain regulatory approvals, and support commercialization activities for partnered product candidates. Products developed by the Company will require approval of the U.S. Food and Drug Administration (FDA) or a foreign regulatory authority prior to commercial sale. The regulatory approval process is expensive, time-consuming, and uncertain, and any denial or delay of approval could have a material adverse effect on the Company. Even if approved, the Company’s products may not achieve market acceptance and will face competition from both generic and branded pharmaceutical products.

As of March 31, 2015, the Company’s cash, cash equivalents and marketable securities totaled \$32.8 million. These funds, together with \$5 million available for drawdown under a facility loan, will satisfy the Company’s liquidity requirements through at least the end of the first quarter of 2016. The Company expects to incur substantial expenditures in the future for the development and potential commercialization of its product candidates. Because of this, the Company expects its future liquidity and capital resource needs will be impacted by numerous factors, including but not limited to, the timing of initiation of planned clinical trials, including phase 2 trials to study the therapeutic benefits of MBX-8025 on patients with certain orphan diseases as well as a phase 3 clinical trial to study the therapeutic benefits of arhalofenate on patients with gout. The Company will therefore continue to require additional financing to develop its products and fund future operating losses and will seek funds through equity financings, debt, collaborative or other arrangements with corporate sources, or through other sources of financing. It is unclear if or when any such financing transactions will occur, on satisfactory terms or at all. The Company’s failure to raise capital as and when needed could have a negative impact on its financial condition and its ability to pursue its business strategies. If adequate funds are not available, the Company may be required to reduce development activities or to close its business.

[Table of Contents](#)

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying interim condensed financial statements are unaudited. These unaudited interim financial statements have been prepared in accordance U.S. GAAP (“GAAP”) and following the requirements of the United States Securities and Exchange Commission (“SEC”) for interim reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by GAAP can be condensed or omitted. In management’s opinion, the unaudited interim financial statements have been prepared on the same basis as the audited financial statements and include all adjustments, which include only normal recurring adjustments, necessary for the fair presentation of the Company’s financial position and its results of operations and comprehensive loss and its cash flows for the periods presented. These statements do not include all disclosures required by GAAP and should be read in conjunction with the Company’s financial statements and accompanying notes for the fiscal year ended December 31, 2014, which is contained in the Company’s Annual Report on Form 10-K as filed with the SEC on March 23, 2015. The results for the three months ended March 31, 2015, are not necessarily indicative of results to be expected for the year or for any other period.

Use of Estimates

The financial statements have been prepared in accordance with GAAP, which requires management to make estimates and assumptions that affect the amounts and disclosures reported in the financial statements and accompanying notes. Management bases its estimates on historical experience and on assumptions believed to be reasonable under the circumstances. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes, and management must select an amount that falls within that range of reasonable estimates. Actual results could differ materially from those estimates. The Company believes significant judgment is involved in estimating stock-based compensation, accrued clinical liabilities, and equity and liability instrument valuations.

Fair Value of Financial Instruments

The Company’s financial instruments during the periods reported consist of cash and cash equivalents, contract receivables, short-term marketable securities, accounts payable, accrued expenses, and warrant liabilities. Fair value estimates of these instruments are made at a specific point in time, based on relevant market information. These estimates may be subjective in nature and involve uncertainties and matters of significant judgment and therefore cannot be determined with precision.

Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants at the measurement date. Assets and liabilities that are measured at fair value are reported using a three-level fair value hierarchy that prioritizes the inputs used to measure fair value. This hierarchy maximizes the use of observable inputs and maximizes the use of unobservable inputs and is as follows:

Level 1—Quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

Level 2—Inputs other than quoted prices in active markets that are observable for the asset or liability, either directly or indirectly.

Level 3—Inputs that are unobservable for the asset or liability.

The carrying amounts of financial instruments such as cash and cash equivalents, contract receivables, accounts payable and accrued expenses approximate the related fair values due to the short-term maturities of these instruments. The carrying value of the facility loan is reflective of the fair value based on market interest rates.

[Table of Contents](#)

Description	As of March 31, 2015			
	(In thousands)			
	Level 1	Level 2	Level 3	Fair Value
Money market funds	\$9,660	\$ —	\$ —	\$ 9,660
Corporate debt and asset backed securities	—	21,950	—	21,950
Total assets measured at fair value	\$9,660	\$21,950	\$ —	\$ 31,610
Warrant liability	\$ —	\$ —	\$7,704	\$ 7,704
Total liabilities measured at fair value	\$ —	\$ —	\$7,704	\$ 7,704

Description	As of December 31, 2014			
	(In thousands)			
	Level 1	Level 2	Level 3	Fair Value
Money market funds	\$9,941	\$ —	\$ —	\$ 9,941
Corporate debt and asset backed securities	—	23,209	—	23,209
Total assets measured at fair value	\$9,941	\$23,209	\$ —	\$ 33,150
Warrant liability	\$ —	\$ —	\$13,596	\$ 13,596
Total liabilities measured at fair value	\$ —	\$ —	\$13,596	\$ 13,596

Marketable securities consist of available-for-sale securities that are reported at fair value, with the related unrealized gains and losses included in accumulated other comprehensive income (loss). The Company values cash equivalents and marketable securities using quoted market prices or alternative pricing sources and models utilizing observable market inputs and, as such, classifies cash equivalents and marketable securities within Level 1 or Level 2.

The Company holds a Level 3 liability associated with common stock warrants that were issued in connection with the Company's 2013 financing. The warrants are considered a liability and are valued using an option-pricing model, the inputs for which include the exercise price of the warrants, market price of the underlying common shares, expected term, volatility based on a group of the Company's peers and the risk-free rate corresponding to the expected term of the warrants. Changes to any of the inputs to the option-pricing models used by the Company can have a significant impact to the estimated fair value of the warrants.

The following table sets forth an activity summary which includes the changes in the fair value of the Company's Level 3 financial instruments (in thousands):

	Warrant Liability
Balance as of December 31, 2014	\$13,596
Issuance of financial instrument	—
Change in fair value	(4,575)
Settlement of financial instrument	(1,317)
Balance as of March 31, 2015	\$ 7,704

The gains and losses from remeasurement of Level 3 financial liabilities are recorded through other income (expense), net on the accompanying condensed statements of operations and comprehensive loss.

Cash, Cash Equivalents, and Marketable Securities

The Company considers all highly liquid investments with a remaining maturity of 90 days or less at the time of purchase to be cash equivalents. Cash and cash equivalents consist of deposits with commercial banks in checking, interest-bearing, and demand money market accounts. The Company invests excess cash in marketable securities with high credit ratings. These securities consist primarily of corporate debt and asset-backed securities and are classified as "available-for-sale." Management may liquidate any of these investments in order to meet the Company's liquidity needs in the next year. Accordingly, any investments with contractual maturities greater than one year from the balance sheet date are classified as short-term in the balance sheet.

Realized gains and losses from the sale of marketable securities, if any, are calculated using the specific identification method. Realized gains and losses and declines in value judged to be other-than-temporary are included in interest income or expense in the statements of operations. Unrealized holding gains and losses are reported in accumulated other comprehensive income (loss), in the balance sheet. To date, the Company has not recorded any impairment charges on its marketable securities related to other-than-temporary declines in market value. In determining whether a decline in market value is other-than-temporary, various factors are considered, including the cause, duration of time and severity of the impairment, any adverse changes in the investees' financial condition, and the Company's intent and ability to hold the security for a period of time sufficient to allow for an anticipated recovery in market value.

[Table of Contents](#)

Restricted Cash

The Company is required to maintain compensating cash balances with financial institutions that provide the Company with its corporate credit cards. As of March 31, 2015 and December 31, 2014, cash restricted under these arrangements was \$100,000. This amount is presented in other assets on the accompanying condensed balance sheets.

Concentration of Credit Risk

Cash, cash equivalents, and marketable securities consist of financial instruments that potentially subject the Company to a concentration of credit risk to the extent of the fair value recorded in the balance sheet. The Company invests cash that is not required for immediate operating needs primarily in highly liquid instruments that bear minimal risk. The Company has established guidelines relating to the quality, diversification, and maturities of securities to enable the Company to manage its credit risk.

Property and Equipment

Property and equipment is stated at cost, less accumulated depreciation and amortization. Depreciation and amortization is calculated using the straight-line method, and the cost is amortized over the estimated useful lives of the respective assets, generally three to seven years. Leasehold improvements are amortized over the shorter of the useful lives or the non-cancelable term of the related lease. Maintenance and repair costs are charged as expense in the statements of operations and comprehensive income (loss) as incurred.

Impairment of Long-Lived Assets

The Company reviews long-lived assets for impairment whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. An impairment loss is recognized if the estimated undiscounted future cash flow expected to result from the use and eventual disposition of an asset is less than the carrying amount. While the Company's current and historical operating losses and cash flows are indicators of impairment, the Company believes the future cash flows to be received support the carrying value of its long-lived assets. Accordingly, the Company has not recognized any impairment losses as of March 31, 2015 and December 31, 2014.

Deferred Rent

The Company records its costs under facility operating lease agreements as rent expense. Rent expense is recognized on a straight-line basis over the non-cancelable term of the operating lease. The difference between the actual amounts paid and amounts recorded as rent expense is recorded to deferred rent in the balance sheet.

Common Stock Warrant Liability

Warrants issued to common stock holders and lenders by the Company in conjunction with the 2013 financing were classified as liabilities in the accompanying condensed balance sheets, as the terms for redemption of the underlying security were outside the Company's control. The warrants were recorded at fair value using either the Black-Scholes option pricing model, probability weighted expected return model or a binomial model, depending on the characteristics of the warrants. The fair value of these warrants is re-measured at each financial reporting period and immediately before exercise, with any changes in fair value being recognized as a component of other income (expense), net in the accompanying condensed statements of operations and comprehensive loss.

Research and Development Expenses

Research and development expenses consist of costs incurred in identifying, developing, and testing product candidates. These expenses consist primarily of costs for research and development personnel (including related stock-based compensation); contract research organizations and other third parties that assist in managing, monitoring, and analyzing clinical trials; investigator and site fees; laboratory services; consultants; contract manufacturing services; non-clinical studies, including materials; and allocated expenses, such as depreciation of assets, and facilities and information technology that support research and development activities. Research and development costs are expensed as incurred, including expenses that may or may not be reimbursed under research and development funding arrangements.

The expenses related to clinical trials are based upon estimates of the services received and efforts expended pursuant to contracts with multiple research institutions and clinical research organizations (CROs) that conduct and manage clinical trials on behalf of the Company. Expenses related to clinical trials are accrued based upon the level of activity incurred under each contract as indicated by such factors as progress made against specified milestones or targets in each period, patient enrollment levels, and other trial activities as reported by CROs. Accordingly, the Company's clinical trial accrual is dependent upon the timely and accurate reporting of expenses by clinical research organizations and other third-party vendors. Payments made to third parties under these clinical trial arrangements in advance of the receipt of the related services are recorded as prepaid assets, depending on the terms of the agreement, until the services are rendered.

[Table of Contents](#)

Stock-Based Compensation

Employee and director stock-based compensation is measured at the grant date, based on the fair-value based measurements of the stock awards, and the portion that is ultimately expected to vest is recognized as an expense over the related vesting periods, net of estimated forfeitures. The Company calculates the fair-value based measurements of options using the Black-Scholes valuation model and recognizes expense using the straight-line attribution method.

Equity awards granted to non-employees are accounted for using the Black-Scholes valuation model to determine the fair value of such instruments. The fair value of equity awards granted to non-employees are re-measured over the related vesting period and amortized to expense as earned.

Income Taxes

The Company utilizes the liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on differences between the financial reporting and the tax bases of assets and liabilities and are measured using enacted tax rates and laws that will be in effect when the differences are expected to reverse. A valuation allowance is recorded when it is more likely than not that all or part of a deferred tax asset will not be realized.

The accounting guidance for uncertainty in income taxes prescribes a recognition threshold and measurement attribute criteria for the financial recognition and measurement of tax positions taken or expected to be taken in a tax return. For those benefits to be recognized, a tax position must be more likely than not to be sustained upon examination based on the technical merits of the position. The Company records interest related to income taxes, if any, as interest, and any penalties would be recorded as other expense in the statements of comprehensive income (loss). There was no interest or penalties related to income taxes recorded during the three months ended March 31, 2015 and 2014.

Comprehensive Loss

Comprehensive loss includes net loss and net unrealized gains and losses on marketable securities, which are presented in a single continuous statement. Accumulated other comprehensive income (loss) is disclosed in the condensed balance sheets, and is stated net of related tax effects, if any.

Net Loss Per Common Share

Basic net loss per share of common stock is based on the weighted average number of shares of common stock outstanding equivalents during the period. Diluted net loss per share of common stock is calculated as the weighted average number of shares of common stock outstanding adjusted to include the assumed exercises of stock options and warrants, if dilutive.

The calculation of diluted loss per share also requires that, to the extent the average market price of the underlying shares for the reporting period exceeds the exercise price of the warrants and the presumed exercise of such securities are dilutive to earnings (loss) per share for the period, adjustments to net income or net loss used in the calculation are required to remove the change in fair value of the warrants for the period. Likewise, adjustments to the denominator are required to reflect the related dilutive shares.

The Company's computation of loss per share is as follows (in thousands, except share and per share amounts):

[Table of Contents](#)

	Three Months Ended March 31,	
	2015	2014
Numerator:		
Net loss allocated to common stock-basic	\$ (2,328)	\$ (10,062)
Adjustments for revaluation of warrants	(4,575)	—
Net loss allocated to common stock-diluted	<u>(6,903)</u>	<u>(10,062)</u>
Denominator:		
Weighted average number of common stock shares outstanding—basic	15,099,567	9,873,687
Dilutive Securities		
Common stock warrants	<u>643,600</u>	<u>—</u>
Weighted average number of common stock shares outstanding—diluted	<u>15,743,167</u>	<u>9,873,687</u>
Net loss per share-basic:	<u>\$ (0.15)</u>	<u>\$ (1.02)</u>
Net loss per share—diluted	<u>\$ (0.44)</u>	<u>\$ (1.02)</u>

The following table shows the total outstanding securities considered anti-dilutive and therefore excluded from the computation of diluted net loss per share.

	Three Months Ended March 31,	
	2015	2014
	(unaudited)	
Warrants for common stock	—	1,848
Common stock options	1,707	907
Incentive awards	<u>247</u>	<u>—</u>
	<u>1,954</u>	<u>2,755</u>

[Table of Contents](#)

3. Certain Balance Sheet Items

Property and equipment consists of the following (in thousands):

	March 31, 2015	December 31, 2014
	(unaudited)	
Office and computer equipment	\$ 176	\$ 176
Purchased software	46	46
Furniture and fixtures	33	33
Leasehold improvements	66	66
Total	321	321
Less accumulated depreciation and amortization	(241)	(235)
Property and equipment, net	<u>\$ 80</u>	<u>\$ 86</u>

Accrued liabilities consist of the following (in thousands):

	March 31, 2015	December 31, 2014
	(unaudited)	
Accrued compensation	\$ 580	\$ 1,504
Accrued pre-clinical and clinical trial expenses	1,967	1,732
Accrued professional fees	380	73
Other accruals	71	79
Total accrued liabilities	<u>\$ 2,998</u>	<u>\$ 3,388</u>

4. Common Stock Warrants

During the three months ended March 31, 2015, the Company issued an aggregate of 122,592 shares of common stock to stockholders upon the exercise of warrants exercisable for shares of the Company's common stock. The 122,592 shares of common stock were issued pursuant to both cash and net exercise provisions as provided in the warrants. Specifically, 74,136 shares of the Company's common stock were issued in exchange for \$0.4 million and 48,456 shares of the Company's common stock were issued in exchange for cancellation of 52,793 shares its common stock. For each warrant exercised, the Company determined the warrant's exercise date fair value and reclassified the fair value of such settled warrants from the warrant liability to additional paid-in capital, a component of stockholder's equity. The aggregate amount of these fair value reclassifications totaled \$1.3 million during the three months ended March 31, 2015.

In January 2014, in connection with the 2013 financing, the Company completed the sale of common stock for aggregate proceeds of \$3.0 million and as part of this transaction, the Company issued five-year warrants to purchase 120,800 shares of common stock at an exercise price of \$5.75 per share. Due to certain provisions, the Company is required to account for the warrants issued as a liability at fair value. In addition, the estimated liability related to the warrants is required to be revalued at each reporting period until the earlier of the exercise of the warrants, at which time the liability will be reclassified to stockholders' equity, or expiration of the warrants. At issuance date, the fair value of the total warrant liability was estimated to be \$0.4 million using a binomial lattice options-pricing model.

5. Collaboration and License Agreements

In June 2006, the Company entered into an exclusive worldwide, royalty-bearing license to MBX-8025 and certain other PPARd compounds (the "PPARd Products") with Janssen Pharmaceutical NV, with the right to grant sublicenses to third parties to make, use and sell such PPARd Products. Under the terms of the agreement, the Company has full control and responsibility over the research, development and registration of any PPARd Products and is required to use diligent efforts to conduct all such activities. Janssen has the sole responsibility for the preparation, filing, prosecution, maintenance of, and defense of the patents with respect to, the PPARd Products. Janssen has a right of first negotiation under the agreement to license a particular PPARd Product from the Company in the event that the Company elects to seek a third party corporate partner for the research, development, promotion, and/or commercialization of such PPARd Products. Under the terms of the agreement Janssen is entitled to receive up to an 8% royalty on net sales of PPARd Products. No payments were made and no royalties were received under this agreement during the three months ended March 31, 2015 and 2014.

In June 2010, the Company entered into two development and license agreements with Janssen Pharmaceuticals, Inc. (Janssen), a subsidiary of Johnson and Johnson, to further develop and discover undisclosed metabolic disease target agonists for the treatment of T2DM and other disorders and received a one-time nonrefundable technology access fee related to the agreements. The Company is also eligible to receive up to \$228 million in contingent payments if certain development and commercial events are achieved as well

Table of Contents

as royalties on worldwide net sales of products. No such payments have been made to date. Under the terms of the agreements, Janssen has full control and responsibility over the research, development and registration of any products developed and/or discovered from the metabolic disease targets and is required to use diligent efforts to conduct all such activities. The Company received a termination notice from Johnson and Johnson, effectively ending these development and licensing agreements in early April 2015.

In June 1998, the Company entered into a license agreement with DiaTex, Inc. (DiaTex) relating to products containing halofenate, its enantiomers, derivatives, and analogs (the licensed products). The license agreement provides that DiaTex and the Company are joint owners of all of the patents and patent applications covering the licensed products and methods of producing or using such compounds, as well as certain other know-how (the covered IP). As part of the license agreement, the Company received an exclusive worldwide license, including as to DiaTex, to use the covered IP to develop and commercialize the licensed products. The Company also retained the right to sub-license the covered IP. The license agreement contains a \$2,000 per month license fee as well as a requirement to make additional payments for development achievements and royalty payments on any sales of licensed products. Pursuant to the license agreement, all of the Company's patents and patent applications related to arhalofenate, its use, and production are jointly owned with DiaTex. DiaTex is entitled to up to \$0.8 million for the future development of arhalofenate, as well as royalty payments on any sales of products containing arhalofenate. No development payments were made in the three months ended March 31, 2015 and 2014 and no royalties have been paid to date.

6. Facility Loan

On September 30, 2013, the Company entered into a facility loan agreement with Silicon Valley Bank and Oxford Finance for a total loan amount of \$10.0 million of which the first tranche of \$5.0 million was drawn as part of the 2013 financing. The loan has a fixed interest rate of 8.75% payable as interest only for twelve months and a thirty-six month loan amortization period thereafter, with a final interest payment of \$0.3 million at the end of the loan period. The second tranche of \$5.0 million became available to the Company upon its February 24, 2015 announcement of the achievement of positive Phase 2b data (the second draw milestone) and shall remain available to the Company until June 30, 2015. Loans under the second tranche will bear interest at a rate fixed at the time of borrowing equal to the greater of (i) 8.75% per annum and (ii) the sum of the Wall Street Journal prime rate plus 4.25% per annum. The Company was also required to pay a facility fee of 1.00% on the term loan facility commitment. As of March 31, 2015, the Company had not drawn down any amount under the \$5 million second tranche.

At the time the first \$5 million tranche of the facility loan was drawn down, the Company issued warrants exercisable for a total of 121,739 shares of the Company's common stock to the lenders at an exercise price of \$5.00 per share. As a result of this issuance, a warrant liability of \$0.5 million was recorded in the accompanying condensed balance sheets and it must be revalued at each balance sheet date.

7. Commitments and Contingencies

The Company leased office and laboratory space in a single building in Hayward, California. The facility lease, as amended on July 15, 2010, had a term of four years and expired on April 30, 2014. On November 8, 2013, the Company entered into a new lease commencing January 16, 2014, and expiring on December 31, 2018, for 8,894 square feet of office space in Newark, California. Rent expense was \$0.1 million and \$0.2 million for the three months ended March 31, 2015 and 2014, respectively.

Future minimum lease payments are as follows (in thousands):

	Lease Payments
Year ending December 31:	
2015 (from April to December)	157
2016	216
2017	222
2018	228
Total future minimum payments	<u>\$ 823</u>

Indemnification

In the normal course of business, the Company enters into contracts and agreements that contain a variety of representations and warranties and provide for general indemnification, including indemnification associated with product liability or infringement of intellectual property rights. The Company's exposure under these agreements is unknown because it involves future claims that may be made against the Company that may be, but have not yet been, made. To date, the Company has not paid any claims or been required to defend any action related to these indemnification obligations, and no amounts have been accrued in the accompanying balance sheets related to these indemnification obligations.

[Table of Contents](#)

The Company has agreed to indemnify its executive officers and directors for losses and costs incurred in connection with certain events or occurrences, including advancing money to cover certain costs, subject to certain limitations. The maximum potential amount of future payments the Company could be required to make under this indemnification is unlimited; however, the Company maintains insurance policies that may limit its exposure and may enable it to recover a portion of any future amounts paid. Assuming the applicability of coverage, the willingness of the insurer to assume coverage, and subject to certain retention, loss limits, and other policy provisions, the Company believes the fair value of these indemnification obligations is not material. Accordingly, the Company has not recognized any liabilities relating to these obligations as of March 31, 2015 and December 31, 2014. No assurances can be given that the covering insurers will not attempt to dispute the validity, applicability, or amount of coverage without expensive litigation against these insurers, in which case the Company may incur substantial liabilities as a result of these indemnification obligations.

8. Stockholders' Equity

The Company is authorized to issue 100,000,000 shares of common stock with a par value of \$0.0001 per share as of March 31, 2015.

As of March 31, 2015 and December 31, 2014, the Company had reserved shares of authorized but unissued common stock as follows:

	<u>March 31,</u> <u>2015</u>	<u>December 31,</u> <u>2014</u>
	(unaudited)	
Common stock warrants	1,592,962	1,768,347
Equity incentive plans	<u>2,284,421</u>	<u>1,549,616</u>
Total reserved shares of common stock	<u>3,877,383</u>	<u>3,317,963</u>

9. Stock Plans and Stock-Based Compensation

Stock Plans

On January 1, 2015, the share reserve of the Company's 2013 Equity Incentive Plan, or 2013 Plan, automatically increased by 734,805 shares. From plan inception through March 31, 2015, the Company had granted options for an aggregate of 1,652,850 shares of the Company's common stock under the 2013 Plan.

[Table of Contents](#)

Stock-Based Compensation Expense

Employee and Director Expense

Employee and director stock-based compensation expense recorded was as follows (in thousands):

	Three Months Ended	
	March 31,	
	2015	2014
	(unaudited)	
Research and development	\$ 186	\$ 146
General and administrative	519	568
Total	<u>\$ 705</u>	<u>\$ 714</u>

On June 3, 2014, stockholders approved a proposal to increase the Company's stock option plan share reserve with sufficient shares to enable the Company to share settle certain incentive awards previously issued to the Company's employees and directors. Prior to the share reserve increase the Company could only cash settle the incentive awards and therefore these awards were required to be revalued at each reporting date and presented as liabilities on the condensed balance sheet. On June 3, 2014, the incentive awards became equity classified for accounting purposes and accordingly, the Company revalued the awards and reclassified \$0.1 million from accrued liabilities to additional paid in capital.

10. Related-Party Transactions

The Company paid a former member of its Board of Directors, who is also a member of its Scientific and Clinical Advisory Boards, a total of \$60,000 in the year ended December 31, 2014 and \$15,000 for the three months ended March 31, 2015, in monthly cash retainers. The Company also issued options to purchase shares of common stock and incentive awards to this individual in his capacity as a member of its Scientific Advisory Board.

[Table of Contents](#)

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

Operating results for the three months ended March 31, 2015, are not necessarily indicative of results that may occur in future interim periods or for the full fiscal year.

This Quarterly Report on Form 10-Q contains statements indicating expectations about future performance and other forward-looking statements within the meaning of Section 27A of the Securities Act, and Section 21E of the Securities Exchange Act of 1934, or the Exchange Act, that involve risks and uncertainties. We usually use words such as “may,” “will,” “should,” “could,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “predict,” “intend,” or the negative of these terms or similar expressions to identify these forward-looking statements. These statements appear throughout this Quarterly Report on Form 10-Q and are statements regarding our current expectation, belief or intent, primarily with respect to our operations and related industry developments. Examples of these statements include, but are not limited to, statements regarding the following: our business and scientific strategies; the progress of our and our collaborators’ product development programs, including clinical testing, and the timing of results thereof; our corporate collaborations and revenues that may be received from our collaborations and the timing of those potential payments; our expectations with respect to regulatory submissions and approvals; our drug discovery technologies; our research and development expenses; protection of our intellectual property; sufficiency of our cash and capital resources and the need for additional capital; and our operations and legal risks. You should not place undue reliance on these forward-looking statements. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including as a result of the risks and uncertainties discussed under the heading “Risk Factors” in Item 1A of Part II of this Quarterly Report on Form 10-Q. Any forward-looking statement speaks only as of the date on which it is made, and we undertake no obligation to update any forward-looking statement to reflect events or circumstances after the date on which the statement is made or to reflect the occurrence of unanticipated events. New factors emerge from time to time, and it is not possible for us to predict which factors will arise. In addition, we cannot assess the impact of each factor on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements.

Overview

CymaBay Therapeutics, Inc. is focused on developing therapies to treat metabolic diseases with high unmet medical need, including serious rare and orphan disorders. Arhalofenate, our lead product candidate, is being developed for the treatment of gout. Arhalofenate has successfully completed five Phase 2 clinical trials in patients with gout and consistently demonstrated the ability to reduce gout flares and reduce serum uric acid (sUA). Gout flares are recurring and painful episodes of joint inflammation that are triggered by the presence of monosodium urate crystals that form as a result of elevated sUA levels. We believe the potential for arhalofenate to prevent or reduce flares while also lowering sUA could differentiate it from currently available treatments for gout and classify it as the first potential drug in what we believe could be a new class of gout therapy referred to as Urate Lowering Anti-Flare Therapy (ULAFT). Arhalofenate has established a favorable safety profile in clinical trials involving over 1,000 patients exposed to date. We are currently planning to hold an end of phase 2 meeting with the FDA in the second half of 2015 to review the results of our completed studies and to discuss the design of a phase 3 program for arhalofenate. Our second product candidate, MBX-8025, demonstrated favorable effects on cholesterol, triglycerides and markers of liver health in a Phase 2 clinical trial in patients with mixed dyslipidemia. We are planning to pursue development of MBX-8025 in a number of orphan diseases in which these attributes could be beneficial, such as homozygous familial hypercholesterolemia (HoFH), primary biliary cirrhosis (PBC) and severe hypertriglyceridemia (SHTG). We also believe that MBX-8025 could have utility in the treatment of the more prevalent, but high unmet need, indication of nonalcoholic steatohepatitis (NASH). We have obtained orphan-drug designations for MBX-8025 in both HoFH and severe Frederickson type I or V hyperlipoproteinemia, and may pursue such designations in other indications as well.

In April 2015, we initiated a Phase 2 pilot study for MBX-8025 in patients with HoFH for which we expect to have data by the end of 2015. The primary objective of this study is to evaluate the effect of MBX-8025 on Low Density Lipoprotein Cholesterol (LDL-C). In addition, we are reviewing plans to potentially move MBX-8025 forward in a Phase 2 clinical study for patients with PBC. We anticipate the study would enroll approximately 100 patients into groups receiving either placebo or 50 or 200 mg of MBX-8025, once daily. The primary endpoint would be reductions in alkaline phosphatase, a biomarker that has been used as a surrogate marker of improvement (efficacy measure) for other drugs tested in PBC. Secondary assessments in the study would examine signs and symptoms of PBC, including fatigue and pruritus.

We are an emerging growth company. Under the JOBS Act emerging growth companies can delay adopting new or revised accounting standards until such time of those standards apply to private companies. We have adopted this exemption from new or revised accounting standards, and therefore, we may not be subject to the same new or revised accounting standards as other public companies that are not “emerging growth companies.”

[Table of Contents](#)**Critical Accounting Policies and Use of Estimates**

Our management's discussion and analysis of our 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 us to make estimates and judgments that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. We base our estimates on historical experience and on various other factors that we believe to be materially reasonable under the circumstances and review our estimates on an ongoing basis. We consider certain accounting policies including, but not limited to, research and development expenses and clinical accruals, stock-based compensation and valuation of warrant liabilities to be critical policies. Actual results may materially differ from these estimates under different assumptions or conditions. There have been no significant changes in our critical accounting estimates during the three months ended March 31, 2015, as compared with those previously disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2014, filed with the SEC on March 23, 2015.

Results of Operations**General**

To date, we have not generated any income from operations. As of March 31, 2015, we had an accumulated deficit of \$383.1 million, primarily as a result of expenditures for research and development and general and administrative expenses from inception to that date. While we may in the future generate revenue from a variety of sources, including product sales, royalties and license fees and milestone payments in connection with strategic partnerships, our product candidates are still under clinical development and may never be successfully developed or commercialized. Accordingly, we expect to continue to incur substantial losses from operations for the foreseeable future and there can be no assurance that we will ever generate significant revenue to achieve and sustain profitability.

	Three Months Ended		Variance
	March 31,		
	2015	2014	
<i>(\$ in thousands)</i>			
Operating expenses:			
Research and development	\$ 4,187	\$ 2,615	\$ 1,572
General and administrative	2,589	2,500	89
Loss from operations	(6,776)	(5,115)	(1,661)
Interest expense, net	(127)	(172)	45
Other income (expense), net	4,575	(4,775)	9,350
Net loss	<u>\$(2,328)</u>	<u>\$(10,062)</u>	<u>\$ 7,734</u>

[Table of Contents](#)

Research & Development Expenses

Conducting research and development is central to our business model. For the three months ended March 31, 2015 and 2014, research and development expenses were \$4.2 million and \$2.6 million, respectively. Research and development expenses are detailed in the table below:

(\$ in thousands)	Three Months Ended March 31,	
	2015	2014
	(unaudited)	
Arhalofenate—Phase 2b Randomized Study	\$ 914	\$ 1,486
Arhalofenate—Febuxostat Combo Study	77	—
Arhalofenate Gout – Drug manufacturing	657	122
Arhalofenate Gout – Three Phase 2 Randomized Studies	—	(90)
MBX-8025	1,205	1
Other Projects	10	16
Total Project Costs	2,863	1,535
Internal Research and Development Costs	1,324	1,080
Total Research and Development	\$ 4,187	\$ 2,615

Our external research and development costs consist primarily of:

- expenses incurred under agreements with contract research organizations, investigative sites and consultants that conduct our clinical trials and a substantial portion of our preclinical activities;
- the cost of acquiring and manufacturing clinical trial and other materials; and
- other costs associated with development activities, including additional studies

Internal research and development costs consist primarily of salaries and related fringe benefits costs for our employees (such as workers compensation and health insurance premiums), stock-based compensation charges, travel costs, lab supplies and overhead expenses. Internal costs generally benefit multiple projects and are not separately tracked per project.

Total project costs increased by \$1.3 million during the three months ended March 31, 2015 as compared to the three months ended March 31, 2014, primarily due to costs incurred for toxicology studies and other development activities associated with MBX-8025 which commenced in the second half of 2014. Development costs of arhalofenate, our lead product candidate, were consistent during the three months ended March 31, 2015 and 2014 as lower clinical trial costs were offset by increased drug manufacturing costs related to registration batch production and other manufacturing process development activities. Internal research and development cost increased by \$0.2 million for the three months ended March 31, 2015 as compared to the three months ended March 31, 2014 due to increased employee compensation expenses incurred in 2015 primarily to support the expansion of our clinical development activities.

We expect to continue to incur substantial expenses related to our development activities for the foreseeable future as we continue product development for arhalofenate and MBX-8025. Since product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later stage clinical trials, we expect that our research and development expenses will increase in the future. In addition, if our product development efforts are successful, we expect to incur substantial costs to prepare for potential Phase 3 clinical trials and activities.

General and Administrative Expenses

General and administrative expenses consist principally of personnel-related costs, professional fees for legal, consulting, audit services, and other general operating expenses not otherwise included in research and development.

Table of Contents

General and administrative expenses were generally consistent for the three months ended March 31, 2015 as compared to the three months ended March 31, 2014. For the next several quarters, we anticipate general and administrative expenses will remain relatively consistent with current levels, given that we have completed a substantial portion of the effort required to expand our infrastructure and we have secured the professional services necessary to support us as a public reporting company under the Exchange Act.

Other Income (Expense), Net

Other income (expense), net for the three months ended March 31, 2015 reflected a gain of \$4.6 million and for the three months ended March 31, 2014 reflected a loss of \$4.8 million, in each case due to the remeasurement of our warrant liabilities at fair value. We use a binomial lattice option pricing model to value our warrants at each reporting date and the warrant valuations changed primarily due to variations in the price of our common stock which is an input to our valuation model. Specifically, during the three months ended March 31, 2015, the gain recognized was due primarily to a decrease in the value of our common stock from \$9.83 at December 31, 2014 to \$6.92 at March 31, 2015. During the three months ended March 31, 2014, the loss recognized was due primarily to an increase in the value of our common stock from \$5.00 at December 31, 2013, to \$8.00 at March 31, 2014.

Liquidity and Capital Resources

To date, we have funded our operations through the sale of equity securities, licensing fees, issuance of debt and collaborations with third parties. At March 31, 2015, we had cash, cash equivalents and marketable securities of \$32.8 million, primarily as a result of the aggregate proceeds received in a series of equity and debt transactions initiated in 2013 and completed in the third quarter of 2014, as well as ongoing sales under our at-the-market facility which we entered into in November 2014.

Specifically, on September 30, 2013, all of the shares of our outstanding redeemable convertible preferred stock converted to common stock, we sold shares of our common stock and warrants to purchase shares of our common stock in a private placement for aggregate gross proceeds of \$26.8 million, and raised an additional \$5.0 million in venture debt financing pursuant to a \$10.0 million loan agreement which we entered into simultaneously with the private placement, resulting in aggregate net proceeds to us of \$28.8 million after deducting placement agent fees and offering expenses. At the same time we issued shares of our common stock in cancellation of approximately \$16.9 million of debt owed to the holder of that debt.

On October 31, 2013, we sold additional shares of our common stock and warrants to purchase shares of our common stock, which sales are also part of the private placement, for net proceeds to us of \$2.2 million after deducting placement agent fees and offering expenses.

Further, on November 22, 2013, we entered into an agreement with investors to purchase shares of our common stock and warrants to purchase shares of our common stock for net proceeds of \$2.7 million, which sales occurred on January 29, 2014. We refer to the private placement, the venture debt financing and the issuance of our common stock in cancellation of the \$16.9 million of debt as the 2013 financing.

On July 25, 2014, we completed a public offering of 4.6 million shares of our common stock at \$5.50 per share which we refer to as our 2014 public offering. Net proceeds to us in connection with the 2014 public offering, were approximately \$23.0 million after deducting underwriting discounts, commissions and offering expenses.

On November 7, 2014, we filed a \$100 million registration statement on Form S-3 with the SEC and also entered into an at-the-market facility to sell up to \$25 million of common stock under the registration statement. In January and February 2015, we sold additional shares of our common stock under this facility for net proceeds to us of \$4.3 million.

Term Loan Facility

The venture debt financing which was part of the 2013 financing was provided to us pursuant to a term loan facility with Silicon Valley Bank and Oxford Finance LLC, collectively referred to as the lenders, for an aggregate amount of \$10 million, the first \$5 million tranche of which was made available to us as of September 30, 2013. The remaining \$5 million, referred to as the second tranche, became available to us upon our February 24, 2015, announcement of the achievement of positive Phase 2b study data (the second draw milestone) and shall remain available to us until June 30, 2015, or the occurrence and continuation of an event of default (as described in the term loan facility). Each tranche matures 48 months following the funding date of such tranche. The proceeds of the term loan facility may be used for general corporate purposes.

The first tranche loans under the term loan facility bear interest at a rate equal 8.75% per annum. Loans under the second tranche will bear interest at a rate fixed at the time of borrowing equal to the greater of (i) 8.75% per annum and (ii) the sum of the Wall Street Journal prime rate plus 4.25% per annum. We were also required to pay a facility fee of 1.00% on the term loan facility commitment.

Table of Contents

We are permitted to make voluntary prepayments of the term loans with a prepayment fee equal to 3% of the term loans prepaid. On each tranche, we are required to make 12 monthly interest only payments after the funding date followed by a repayment schedule equal to 36 equal monthly payments of the outstanding principal of the outstanding term loans of each tranche. After the 36-month amortization period of each tranche, the remaining balance of such tranche and a final payment equal to 6.50% of the original principal amount of the applicable tranche are payable on the maturity date of such tranche. We are required to make mandatory prepayments of the outstanding term loans upon the acceleration by the lenders of such loans following the occurrence of an event of default, along with a payment of the final payment, the prepayment fee and any all other obligations (each as defined or described under the term loan facility) that are due and payable at the time of the prepayment.

Our obligations under the term loan facility are secured, subject to customary permitted liens and other agreed upon exceptions, (1) by a first priority pledge of all of the equity interests of each of our direct and indirect subsidiaries, and (2) a perfected first priority interest in all of our tangible and intangible assets, including all of our intellectual property.

The term loan facility contains customary representations and warranties and customary affirmative and negative covenants applicable to us and our subsidiaries, including, among other things, restrictions on dispositions, changes in business, management, ownership or business locations, mergers or acquisitions, indebtedness, encumbrances, distributions, investments, transactions with affiliates and subordinated debt. Until the occurrence of the second draw milestone in February 2015, the term loan facility contained financial covenants that required us to maintain certain cash liquidity. Upon achievement of this milestone this financial covenant was eliminated.

The term loan facility also includes customary events of default, including but not limited to the nonpayment of principal or interest, violations of covenants, material adverse change, attachment, levy, restraint on business, cross-defaults on our or any our subsidiary's material indebtedness, bankruptcy, material judgments and misrepresentations. Upon an event of default, the lenders may, among other things, accelerate the loans and foreclose on the collateral. As of March 31, 2015, we were in compliance with the terms of the term loan covenants and there were no identified events of default.

Cash Flows

The following table sets forth a summary of the net cash flow activity for each of the periods indicated below (in thousands):

	Three Months Ended March 31,	
	2015	2014
Net cash used in operating activities	\$ (6,159)	\$ (5,011)
Net cash provided by investing activities	1,099	550
Net cash provided by financing activities	4,315	2,402
Net decrease in cash and cash equivalents	\$ (745)	\$ (2,059)

Operating Activities: Net cash used in operating activities for the three months ended March 31, 2015 was \$6.2 million primarily due to a net loss of \$2.3 million resulting from ongoing drug development activities, a \$4.6 million noncash gain recorded to revalue our warrant liability, \$0.7 million of stock-based compensation, and other changes in working capital.

Investing Activities: Net cash provided by investing activities was \$1.1 million for the three months ended March 31, 2015, primarily due to net maturities of marketable securities.

Financing Activities: Cash provided by financing activities was \$4.3 million for the three months ended March 31, 2015, primarily as a result of \$4.3 million in net proceeds received from sales of our common stock in January and February 2015 pursuant to a \$25 million at-the-market facility.

Capital Requirements

As of March 31, 2015, our cash, cash equivalents and marketable securities totaled \$32.8 million. These funds, together with \$5 million available for drawdown under our facility loan, will satisfy our liquidity requirements through at least the end of the first quarter of 2016. We expect to incur substantial expenditures in the future for the development and potential commercialization of our product candidates. Because of this, we expect our future liquidity and capital resource needs will be impacted by numerous factors, including but not limited to, the timing of initiation of planned clinical trials, including phase 2 trials to study the therapeutic benefits of MBX-8025 on patients with certain orphan diseases as well as a phase 3 clinical trial to study the therapeutic benefits of arhaolfenate on patients with gout. We will therefore continue to require additional financing to develop our products and fund future operating losses and will seek funds through equity financings, debt, collaborative or other arrangements with corporate sources, or through other sources of financing. It is unclear if or when any such financing transactions will occur, on satisfactory terms or at all. Our failure to raise capital as and when needed could have a negative impact on our financial condition and our ability to pursue our business strategies. If adequate funds are not available to us, we may be required to reduce our development activities or to close our business.

[Table of Contents](#)

Contractual Obligations and Commitments

There have been no significant changes to our aggregate contractual obligations as compared to the disclosures in our Annual Report on Form 10-K for the year ended December 31, 2014 as filed with the SEC on March 23, 2015.

Off-Balance Sheet Arrangements

We do not currently have, nor have we ever had, any relationships with unconsolidated entities or financial partnerships, such as entities often referred to as structured finance or special purpose entities, established for the purpose of facilitating off-balance sheet arrangements or other contractually narrow or limited purposes. In addition, we do not engage in trading activities involving non-exchange traded contracts.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

This item is not applicable to us as a smaller reporting company.

Item 4. Controls and Procedures

- (a) *Evaluation of Disclosure Controls and Procedures.* Based on the evaluation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act), our chief executive officer and chief financial officer have concluded that, as of the end of the period covered by this report, our disclosure controls and procedures were effective.
- (b) *Limitations on the Effectiveness of Controls.* A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the controls are met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues, if any, within a company have been detected. Accordingly, our disclosure controls and procedures are designed to provide reasonable, not absolute, assurance that the objectives of our disclosure control system are met and, as set forth above, our chief executive officer and chief financial officer have concluded, based on their evaluation as of the end of the period covered by this report, that our disclosure controls and procedures were sufficiently effective to provide reasonable assurance that the objectives of our disclosure control system were met.
- (c) *Changes in Internal Controls.* There were no changes in our internal control over financial reporting that occurred during the quarter ended March 31, 2015, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1A. Risk Factors

In evaluating our business, you should carefully consider the following risks, as well as the other information contained in this Quarterly Report on Form 10-Q. These risk factors could cause our actual results to differ materially from those contained in forward-looking statements we have made in this Quarterly Report on Form 10-Q and those we may make from time to time. If any of the following risks actually occurs, our business, financial condition and operating results could be harmed. The risks and uncertainties described below are not the only ones facing us. Additional risks and uncertainties not presently known to us, or that we currently see as immaterial, may also harm our business. The risks facing our business have not changed substantively from those discussed in our Annual Report on Form 10-K for the year ended December 31, 2014, except for those risk factors below designated by an asterisk ().*

Risks Related to Our Financial Condition and Capital Requirements

We will need additional capital in the future to sufficiently fund our operations and research.

Table of Contents

We have consumed substantial amounts of capital to date as we continue our research and development activities. As of March 31, 2015, we had cash, cash equivalents and marketable securities of approximately \$32.8 million. These funds were obtained through recent equity and debt financings. We believe that our existing cash will allow us to continue operation through at least the end of the first quarter of 2016. We currently believe that we will need to raise additional capital to continue our operations thereafter. Our monthly spending levels vary based on new and ongoing development and corporate activities.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a time-consuming, expensive and uncertain process that takes years to complete. We expect our research and development expenses to substantially increase in connection with our ongoing activities, particularly as we advance development of our lead clinical product candidate, arhalofenate, for the prevention of gout flares and the treatment of hyperuricemia in patients with gout.

In the event we do not successfully raise sufficient funds in financing our product development activities, particularly related to the ongoing development of arhalofenate and planned development of MBX-8025, it will be necessary to curtail our product development activities commensurate with the magnitude of the shortfall or our product development activities may cease altogether. To the extent that the costs of the ongoing development of arhalofenate exceed our current estimates and we are unable to raise sufficient additional capital to cover such additional costs, we will need to reduce operating expenses, enter into a collaboration or other similar arrangement with respect to development and/or commercialization rights to arhalofenate, outlicense intellectual property rights to arhalofenate, sell assets or effect a combination of the above. No assurance can be given that we will be able to effect any of such transactions on acceptable terms, if at all. Failure to progress the development of arhalofenate and MBX-8025 will have a negative effect on our business, future prospects and ability to obtain further financing on acceptable terms (if at all).

Beyond the plan of operations outlined above, our future funding requirements and sources will depend on many factors, including but not limited to the following:

- the rate of progress and cost of our clinical studies, including in particular the Phase 3 studies of arhalofenate and planned proof-of-concept studies of MBX-8025;
- the need for additional or expanded clinical studies;
- the rate of progress and cost of our Chemistry, Manufacturing and Control registration and validation program;
- the timing, economic and other terms of any licensing, collaboration or other similar arrangement into which we may enter;
- the costs and timing of seeking and obtaining FDA and other regulatory approvals;
- the extent of our other development activities;
- the costs of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights; and
- the effect of competing products and market developments.

If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we will be prevented from pursuing development and commercialization efforts, which will have a material adverse effect on our business, operating results and prospects and on our ability to develop our product candidates.

We have incurred significant losses since our inception. We anticipate that we will continue to incur significant losses for the foreseeable future, and we may never achieve or maintain profitability.

We are a biopharmaceutical company focused primarily on developing our lead product candidate, arhalofenate. We have incurred significant net losses in each year since our inception, including a net loss of approximately \$31.9 million for the year ended December 31, 2014, and a net loss of \$2.3 million for the three months ended March 31, 2015. As of March 31, 2015, we had an accumulated deficit of \$383.1 million.

To date, we have financed our operations primarily through the sale of equity securities, licensing fees, issuance of debt and collaborations with third parties. We have devoted most of our financial resources to research and development, including our preclinical development activities and clinical trials. We have not completed development of any product candidates. We expect to continue to incur significant and increasing losses and negative cash flows for the foreseeable future. The size of our losses will depend, in part, on the rate of future expenditures and our ability to generate revenues. In particular, we expect to incur substantial and increased expenses as we:

Table of Contents

- continue the development of our lead product candidate, arhalofenate, for the prevention of flares and treatment of hyperuricemia in patients with gout;
- seek to obtain regulatory approvals for arhalofenate;
- prepare for the potential commercialization of arhalofenate;
- scale up manufacturing capabilities to commercialize arhalofenate for any indications for which we receive regulatory approval;
- begin outsourcing of the commercial manufacturing of arhalofenate for any indications for which we receive regulatory approval;
- establish an infrastructure for the sales, marketing and distribution of arhalofenate for any indications for which we receive regulatory approval;
- expand our research and development activities and advance our clinical programs, including MBX-8025;
- maintain, expand and protect our intellectual property portfolio;
- continue our research and development efforts and seek to discover additional product candidates; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts and operations as a public company.

We do not anticipate that we will generate revenue from the sale of our products for the foreseeable future. Our ability to become profitable depends upon our ability to generate significant continuing revenues.

In the absence of additional sources of capital, which may not be available to us on acceptable terms, or at all, the development of arhalofenate or future product candidates may be reduced in scope, delayed or terminated. If our product candidates fail in clinical studies or do not gain regulatory approval, or if our future products, if any, do not achieve market acceptance, we may never become profitable.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations.

Our ability to generate future revenues from product sales is uncertain and depends upon our ability to successfully develop, obtain regulatory approval for, and commercialize our product candidates. *

Our ability to generate revenue and achieve profitability depends on our ability, alone or with collaborators, to successfully complete the development of, obtain the necessary regulatory approvals for and commercialize our product candidates. We do not anticipate generating revenues from sales of our product candidates for the foreseeable future, if ever. Our ability to generate future revenues from product sales depends heavily on our success in:

- obtaining favorable results for and advancing the development of arhalofenate, including raising sufficient capital or partnering with a third party to successfully initiate our Phase 3 clinical development;
- obtaining United States (U.S.) and foreign regulatory approvals for arhalofenate;
- launching and commercializing arhalofenate, either on our own or with a partner, including building a sales force and collaborating with third parties;
- achieving broad market acceptance of arhalofenate in the medical community and by third-party payors and patients;
- obtaining favorable results for and advancing the development of MBX-8025; and
- generating a pipeline of product candidates.

Conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data required to obtain regulatory approval and achieve product sales. Our anticipated development costs would likely increase if we do not obtain favorable results or if development of our product candidates is delayed. In particular, we would likely incur higher costs than we currently anticipate if development of our product candidates is delayed because we are required by the U.S. FDA to perform studies or trials in addition to those that we currently anticipate. Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to predict the timing or amount of any increase in our anticipated development costs.

Table of Contents

In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for several years, if at all. Even if one or more of our product candidates is approved for commercial sale, we anticipate incurring significant costs in connection with commercialization. As a result, we cannot assure you that we will be able to generate revenues from sales of any approved product candidates, or that we will achieve or maintain profitability even if we do generate sales.

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

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other marketing and distribution arrangements. We do not have any committed external source of funds.

In order to raise additional funds to support our operations, we may sell additional equity or debt securities, enter into collaborations, strategic alliances, or licensing arrangements or other marketing or distribution arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, ownership interests of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of stockholders. 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, and declaring dividends, and will impose limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business.

If we raise additional funds through collaborations, strategic alliances, or licensing arrangements or other marketing or distribution arrangements with third parties, we may have to relinquish valuable rights to our intellectual property, technologies, future revenue streams, research programs or product candidates, or grant licenses on terms that may not be favorable to us. If we are unable to expand our operations or otherwise capitalize on our business opportunities, our business, financial condition and results of operations could be materially adversely affected and we may not be able to meet our debt service obligations. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts, or grant others rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Table of Contents

We are an emerging growth company and we cannot be certain if the reduced disclosure requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an emerging growth company. Under the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We plan to avail ourselves of this exemption from new or revised accounting standards and, therefore, we may not be subject to the same new or revised accounting standards as other public companies that are not “emerging growth companies.”

For as long as we continue to be an emerging growth company, we also intend to take advantage of certain other exemptions from various reporting requirements that are applicable to other public companies including, but not limited to, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, exemptions from the requirements of holding a nonbinding advisory stockholder vote on executive compensation and any golden parachute payments not previously approved, exemption from the requirement of auditor attestation in the assessment of our internal control over financial reporting and exemption from any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements (auditor discussion and analysis). If we do, the information that we provide stockholders may be different than what is available with respect to other public companies. We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If investors find our common stock less attractive as a result of our status as an emerging growth company, there may be less liquidity for our common stock and our stock price may be more volatile.

We will remain an emerging growth company until the earliest of (i) the end of the fiscal year in which the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the end of the second fiscal quarter, (ii) the end of the fiscal year in which we have total annual gross revenues of \$1 billion or more during such fiscal year, (iii) the date on which we issue more than \$1 billion in non-convertible debt in a three-year period or (iv) the end of the fiscal year following the fifth anniversary of the date of the first sale of our common stock pursuant to an effective registration statement filed under the Securities Act.

Risks Related to Clinical Development and Regulatory Approval

We depend on the success of our lead product candidate, arhalofenate, which is still under clinical development, and MBX-8025, which we currently plan to develop, and may not obtain regulatory approval or successfully commercialize either of these product candidates.

We have not marketed, distributed or sold any products. The success of our business depends upon our ability to develop and commercialize our lead product candidate, arhalofenate, which has completed eight Phase 1 and nine Phase 2 clinical trials, including five Phase 2 studies in gout and our second product candidate, MBX-8025, which has completed five Phase 1 and one Phase 2 clinical trials. We are currently planning to meet with the FDA at an end of phase 2 meeting prior to finalizing our plans for and initiating a Phase 3 program for arhalofenate in gout. There is no guarantee that our clinical trials will be completed or, if completed, will be successful. We also plan to initiate one or more proof-of-concept studies for MBX-8025 in the first half of 2015. The success of arhalofenate and MBX-8025 will depend on several factors, including the following:

- successful enrollment and completion of clinical trials;
- recognition by the FDA and other regulatory authorities outside of the U.S. of orphan disease designation;
- receipt of marketing approvals from the FDA and regulatory authorities outside the U.S. for our product candidate;
- establishing commercial manufacturing capabilities by making arrangements with third-party manufacturers;
- launching commercial sales of the product, whether alone or in collaboration with others;
- acceptance of the product by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- a continued acceptable safety profile of the product following approval; and
- obtaining, maintaining, enforcing and defending intellectual property rights and claims.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize arhalofenate, which would materially harm our business.

Table of Contents

We have never obtained regulatory approval for a drug and we may be unable to obtain, or may be delayed in obtaining, regulatory approval for arhalofenate.

We have never obtained regulatory approval for a drug. In the U.S. it is possible that the FDA may refuse to accept our New Drug Application (NDA) for substantive review or may conclude after review of our data that our application is insufficient to obtain regulatory approval of arhalofenate. If the FDA does not accept or approve our NDA, it may require that we conduct additional clinical, nonclinical or manufacturing validation studies and submit that data before it will reconsider our application. Depending on the extent of these or any other FDA required studies, approval of any NDA or application that we submit may be delayed by several years, or may require us to expend more resources than we have available. It is also possible that additional studies, if performed and completed, may not be considered sufficient by the FDA to approve our NDA.

We currently do not know when we might commence our Phase 3 study of arhalofenate or achieve FDA approval of arhalofenate. We currently do not have the capital necessary to conduct or complete Phase 3 studies of arhalofenate and we may not be able to raise sufficient funds necessary or secure a partnership to conduct this study.

Any delay in obtaining, or an inability to obtain, regulatory approvals would prevent us from commercializing arhalofenate, generating revenues and achieving and sustaining profitability. If any of these outcomes occur, we may be forced to abandon our development efforts for arhalofenate, which would have a material adverse effect on our business and could potentially cause us to cease operations.

We depend on the successful completion of clinical trials for our product candidates, including arhalofenate. The positive clinical results obtained for our product candidates in prior clinical studies may not be repeated in future clinical studies.

Before obtaining regulatory approval for the sale of our product candidates, including arhalofenate and MBX-8025, we must conduct additional clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more of our clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval for their products.

We have completed five Phase 2 clinical studies of arhalofenate in gout. In addition, six clinical studies with MBX-8025 and five clinical studies with MBX-2982 have been completed. However, we have never conducted a Phase 3 clinical trial. The positive results we have seen to date in our Phase 2 clinical trials of arhalofenate for gout do not ensure that later clinical trials will demonstrate similar results. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy characteristics despite having progressed satisfactorily through preclinical studies and initial clinical testing. A number of companies in the pharmaceutical and biotechnology industries, including those with greater resources and experience, have suffered significant setbacks in Phase 3 clinical development, even after seeing promising results in earlier clinical trials.

We may experience a number of unforeseen events during clinical trials for our product candidates, including arhalofenate, that could delay or prevent the commencement and/or completion of our clinical trials, including the following:

- regulators or institutional review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- the clinical study protocol may require one or more amendments delaying study completion;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- the number of subjects required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be insufficient or slower than we anticipate or subjects may drop out of these clinical trials at a higher rate than we anticipate;
- clinical investigators or study subjects fail to comply with clinical study protocols;
- trial conduct and data analysis errors may occur, including, but not limited to, data entry and/or labeling errors;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we might have to suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the subjects are being exposed to unacceptable health risks;

Table of Contents

- regulators or institutional review boards may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
- the supply or quality of our clinical trial materials or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators to suspend or terminate the trials.

We expect our research and development expenses to increase in connection with our ongoing activities, particularly if we commence a Phase 3 clinical trial with arhalofenate and undertake additional clinical trials of our other product candidates MBX-8025 and MBX-2982. Before we commence a Phase 3 clinical trial for arhalofenate, we will need to raise substantial additional capital. We also will need to raise substantial additional capital in the future to complete the development and commercialization of MBX-8025, as well as MBX-2982, for which we currently have no planned clinical trials. Because successful development of our product candidates is uncertain, we are unable to estimate the actual funds required to complete research and development and commercialize our products under development.

Negative or inconclusive results of our future clinical trials of arhalofenate, or any other clinical trial we conduct, could cause the FDA to require that we repeat or conduct additional clinical studies. Despite the results reported in earlier clinical trials for arhalofenate, we do not know whether any clinical trials we may conduct will demonstrate adequate efficacy and safety to result in regulatory approval to market our product candidates, including arhalofenate. If later stage clinical trials do not produce favorable results, our ability to obtain regulatory approval for our product candidates, including arhalofenate, may be adversely impacted.

We have never conducted a clinical trial of MBX-8025 for the indications which we are considering for MBX-8025, including homozygous familial hypercholesterolemia (HoFH) and Primary Biliary Cirrhosis (PBC). If MBX-8025 does not demonstrate safety or efficacy in the treatment of any of these indications, or if the benefits of treatment with MBX-8025 do not outweigh the risks, our ability to successfully develop and commercialize MBX-8025 may be adversely affected.*

We have not previously conducted a clinical trial of MBX-8025 for any of the indications for which we currently are considering, including HoFH and PBC. As a result, although we believe that MBX-8025 may be beneficial to address the diseases for which we are considering redirecting its development, there is no guarantee that MBX-8025 will prove to be safe or efficacious in the treatment of these diseases, or that we will be able to obtain FDA approval for these indications. The results of these clinical studies and other nonclinical studies may determine whether the benefits perceived from the use of MBX-8025 would outweigh the risks perceived from treatment with MBX-8025.

Delays in clinical trials are common and have many causes, and any delay could result in increased costs to us and jeopardize or delay our ability to obtain regulatory approval and commence product sales.

Clinical testing is expensive, difficult to design and implement, can take many years to complete, and is uncertain as to outcome. We may experience delays in clinical trials at any stage of development and testing of our product candidates. Our planned clinical trials may not begin on time, have an effective design, enroll a sufficient number of subjects, or be completed on schedule, if at all.

Events which may result in delays or unsuccessful completion of clinical trials, including our future clinical trials for arhalofenate, include the following:

- inability to raise funding necessary to initiate or continue a trial;
- delays in obtaining regulatory approval to commence a trial;
- delays in reaching agreement with the FDA on final trial design;
- imposition of a clinical hold following an inspection of our clinical trial operations or trial sites by the FDA or other regulatory authorities;
- delays in reaching agreement on acceptable terms with prospective contract research organizations (CROs) and clinical trial sites;
- delays in obtaining required institutional review board (IRB) approval at each site;
- delays in recruiting suitable patients to participate in a trial;
- delays in having subjects complete participation in a trial or return for post-treatment follow-up;
- delays caused by subjects dropping out of a trial due to side effects or otherwise;
- delays caused by clinical sites dropping out of a trial;

Table of Contents

- time required to add new clinical sites; and
- delays by our contract manufacturers to produce and deliver sufficient supply of clinical trial materials.

If initiation or completion of any of our clinical trials for our product candidates, including arhalofenate, is delayed for any of the above reasons, our development costs may increase, the approval process could be delayed, any periods during which we may have the exclusive right to commercialize our product candidates may be reduced and our competitors may bring products to market before us. Any of these events could impair our ability to generate revenues from product sales and impair our ability to generate regulatory and commercialization milestones and royalties, all of which could have a material adverse effect on our business.

Our product candidates may cause adverse effects or have other properties that could delay or prevent their regulatory approval or limit the scope of any approved label or market acceptance.

Arhalofenate has been studied in a total of 17 clinical trials with over a thousand subjects. The emergence of adverse events (AEs) caused by arhalofenate in future studies could cause us, other reviewing entities, clinical study sites or regulatory authorities to interrupt, delay or halt clinical studies and could result in the denial of regulatory approval. There is also a risk that our other product candidates, including MBX-8025, may induce AEs, many of which may be unknown at this time. If an unacceptable frequency and/or severity of AEs are reported in our clinical trials for our product candidates, our ability to obtain regulatory approval for product candidates, including arhalofenate and MBX-8025, may be negatively impacted.

Furthermore, if any of our approved products cause serious or unexpected side effects after receiving market approval, a number of potentially significant negative consequences could result, including the following:

- regulatory authorities may withdraw their approval of the product or impose restrictions on its distribution in a form of a modified risk evaluation and mitigation strategy;
- regulatory authorities may require the addition of labeling statements, such as warnings or contraindications that could diminish the usage of the product or otherwise limit the commercial success of the affected product;
- we may be required to change the way the product is administered or to conduct additional clinical studies;
- we may choose to discontinue sale of the product;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product candidate and could substantially increase the costs of commercializing our product candidates.

We have obtained orphan drug designation for some of the targeted indications for MBX-8025 but not all possible indications for which we may seek approval and we may not be able to obtain or maintain orphan designation or obtain the benefits associated with orphan drug status, including market exclusivity.*

Regulatory authorities in some jurisdictions, including the United States and the European Union, or EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, as amended, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes the FDA or the European Medicines Agency, or EMA, from approving another marketing application for the same drug for that time period. The applicable period is seven years in the United States and ten years in the European Union. The EU 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 so 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. In addition, the orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process. Also, regulatory approval for any product candidate may be withdrawn and other candidates may obtain approval before us.

We have obtained orphan-drug designations for MBX-8025 for the treatments of HoFH and Frederickson Type I or V hyperlipoproteinemia, respectively. That exclusivity, or any other orphan exclusivity we may receive for another product candidate or indication, may not effectively protect the candidate from competition because: different drugs can be approved for the same condition; the same drugs can be approved for different indications and prescribed off-label; and the first entity with an orphan drug designation to receive regulatory approval for a particular indication will receive marketing exclusivity. If one of our product candidates that receives an orphan drug designation, including MBX-8025, is approved for a particular indication or use within the rare disease or condition, the FDA may later approve the same product for additional indications or uses within that rare disease or condition that are not protected by our exclusive approval. Even after an orphan drug is approved, the FDA can subsequently approve another 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.

Table of Contents

If any product candidate that we successfully develop does not achieve broad market acceptance among physicians, patients, health care payors and the medical community, the revenues that it generates from its sales will be limited.

Even if arhalofenate, MBX-8025 or any other product candidates receive regulatory approval, the products may not gain market acceptance among physicians, patients, health care payors and the medical community. Coverage and reimbursement of our product candidates by third-party payors, including government payors, generally is also necessary for commercial success. The degree of market acceptance of any of our approved products will depend upon a number of factors, including:

- the efficacy and safety, as demonstrated in clinical studies;
- the risk/benefit profile of our products such as arhalofenate;
- the prevalence and severity of any side effects;
- the clinical indications for which the product is approved;
- acceptance of the product by physicians, other health care providers and patients as a safe and effective treatment;
- the potential and perceived advantages of product candidates over alternative treatments;
- the safety of product candidates seen in a broader patient group, including its use outside the approved indications;
- the cost of treatment in relation to alternative treatments;
- the timing of market introduction of competitive products;
- the availability of adequate reimbursement and pricing by third parties and government authorities;
- relative convenience and ease of administration; and
- the effectiveness of our or our partners' sales, marketing and distribution efforts.

If any product candidate is approved but does not achieve an adequate level of acceptance by physicians, hospitals, health care payors and patients, we may not generate sufficient revenue from these products and we may not become or remain profitable.

Potential conflicts of interest arising from relationships and any related compensation with respect to clinical studies could adversely affect the process.

Principal investigators for our clinical studies may serve as scientific advisors or consultants to us from time to time and receive cash compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, the integrity of the data generated at the applicable clinical study site may be questioned or jeopardized.

We may be subject to costly claims related to our clinical studies and may not be able to obtain adequate insurance.

Because we conduct clinical studies in humans, we face the risk that the use of arhalofenate, MBX-8025 or future product candidates, will result in adverse side effects. We cannot predict the possible harms or side effects that may result from our clinical studies. Although we have clinical study liability insurance, our insurance may be insufficient to cover any such events. There is also a risk that we may not be able to continue to obtain clinical study coverage on acceptable terms. In addition, we may not have sufficient resources to pay for any liabilities resulting from a claim excluded from, or beyond the limit of, our insurance coverage. There is also a risk that third parties that we have agreed to indemnify could incur liability. Any litigation arising from our clinical studies, even if we are ultimately successful, would consume substantial amounts of our financial and managerial resources and may create adverse publicity.

After the completion of our clinical trials, we cannot predict whether or when we will obtain regulatory approval to commercialize our product candidates and we cannot, therefore, predict the timing of any future revenue from our product candidates. Regulatory approval of an NDA is not guaranteed, and the approval process is expensive, uncertain and lengthy.

We cannot commercialize our product candidates, including arhalofenate and MBX-8025, until the appropriate regulatory authorities, such as the FDA, have reviewed and approved the product candidate. The regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval for our product candidates. Additional delays may result if a product candidate is brought before an FDA advisory committee, which could recommend restrictions on approval or recommend non-approval of the product candidate. In addition, we may experience delays or rejections based upon

Table of Contents

additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical studies and the review process. As a result, we cannot predict when, if at all, we will receive any future revenue from commercialization of any of our product candidates, including arhalofenate and MBX-8025. The FDA has substantial discretion in the drug approval process, including the ability to delay, limit or deny approval of a product candidate for many reasons, including the following:

- we may be unable to demonstrate to the satisfaction of regulatory authorities that a product candidate is safe and effective for any indication;
- regulatory authorities may not find the data from nonclinical studies and clinical studies sufficient or may differ in the interpretation of the data;
- regulatory authorities may require additional nonclinical or clinical studies;
- the FDA or foreign regulatory authority might not approve our third party manufacturers' processes or facilities for clinical or commercial product;
- the FDA or foreign regulatory authority may change its approval policies or adopt new regulations;
- the FDA or foreign regulatory authorities may disagree with the design or implementation of our clinical studies;
- the FDA or foreign regulatory authority may not accept clinical data from studies that are conducted in countries where the standard of care is potentially different from that in the U.S.;
- the results of clinical studies may not meet the level of statistical significance required by the FDA or foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks; and
- the data collection from clinical studies of our product candidates may not be sufficient to support the submission of a NDA or other submission or to obtain regulatory approval in the U.S. or elsewhere.

In addition, events raising questions about the safety of certain marketed pharmaceuticals may result in increased caution by the FDA and other regulatory authorities in reviewing new pharmaceuticals based on safety, efficacy or other regulatory considerations and may result in significant delays in obtaining regulatory approvals.

Even if we obtain regulatory approval for arhalofenate, MBX-8025 and our other product candidates, we will still face extensive regulatory requirements and our products may face future development and regulatory difficulties.

Even if we obtain regulatory approval in the U.S., the FDA may still impose significant restrictions on the indicated uses or marketing of our product candidates, including arhalofenate and MBX-8025, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. For example, the labeling ultimately approved for our product candidates, including arhalofenate and MBX-8025, may include restrictions on use due to the specific patient population and manner of use in which the drug was evaluated and the safety and efficacy data obtained in those evaluations.

Arhalofenate, MBX-8025 and our other product candidates will also be subject to additional ongoing FDA requirements governing the labeling, packaging, storage, distribution, safety surveillance, advertising, promotion, record-keeping and reporting of safety and other post-market information. The holder of an approved NDA is obligated to monitor and report AEs and any failure of a product to meet the specifications in the NDA. The holder of an approved NDA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws. Furthermore, promotional materials must be approved by the FDA prior to use for any drug receiving accelerated approval, the pathway we are pursuing for arhalofenate in the U.S.

In addition, manufacturers of drug products and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with current Good Manufacturing Practices (cGMP), and adherence to commitments made in the NDA. If we, or a regulatory agency, discover previously unknown problems with a product, such as quality issues or AEs of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions relative to that product or the manufacturing facility, including requesting recall or withdrawal of the product from the market or suspension of manufacturing.

If we, or our third party contractors, fail to comply with applicable regulatory requirements following approval of our product candidate, a regulatory agency may:

- issue an untitled or warning letter asserting violation of the law;
- seek an injunction or impose civil or criminal penalties or monetary fines;

Table of Contents

- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending NDA or supplements to an NDA; or
- recall and/or seize product.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize arhalofenate and our other product candidates and inhibit our ability to generate revenues.

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products. If we are found to have improperly promoted our products for off-label uses, we may become subject to significant fines and other liability. *

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. If we receive marketing approval for our product candidates, physicians may nevertheless prescribe such products to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off-label uses, we may become subject to significant government fines and other related liability. For example, the federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA also has requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

Even if we obtain FDA approval for arhalofenate, MBX-8025 or any of our other product candidates in the U.S., we may never obtain approval for or commercialize arhalofenate, MBX-8025 or any of our other product candidates outside of the U.S., which would limit our ability to realize their full market potential.

In order to market any products outside of the U.S., we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional preclinical studies or clinical trials which could be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of our products will be unrealized.

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

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

- the federal health care 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 either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal health care programs such as Medicare and Medicaid;
- the federal False Claims Act imposes criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

Table of Contents

- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil liability for executing a scheme to defraud any health care benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for health care benefits, items or services;
- the federal transparency requirements under the PPACA require manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician ownership and investment interests; and
- analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving health care items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures.

Efforts to ensure that our business arrangements with third parties will comply with applicable health care 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 health care 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, exclusion from government funded health care programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded health care programs.

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

In the U.S. and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the health care system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any products for which we obtain marketing approval.

In the U.S., the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (Medicare Modernization Act) changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the Medicare Modernization Act may result in a similar reduction in payments from private payors.

More recently, in March 2010, the Affordable Care Act was enacted to broaden access to health insurance, reduce or constrain the growth of health care spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The Affordable Care Act revises the definition of "average manufacturer price" for reporting purposes, which could increase the amount of Medicaid drug rebates to states. Further, the new law imposes a significant annual fee on companies that manufacture or import branded prescription drug products. New provisions affecting compliance have also been enacted, which may affect our business practices with health care practitioners. We will not know the full effects of the Affordable Care Act until applicable federal and state agencies issue regulations or guidance under the new law. Although it is too early to determine the effect of the Affordable Care Act, the new 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.

[Table of Contents](#)

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

Risks Related to Our Reliance on Third Parties

We rely on third-party manufacturers to produce our preclinical and clinical drug supplies, and we intend to rely on third parties to produce commercial supplies of any approved product candidates.

We do not own or operate, and we do not expect to own or operate, facilities for product manufacturing, storage and distribution, or testing. In the past we have relied on third-party manufacturers for supply of our preclinical and clinical drug supplies. We expect that in the future we will continue to rely on such manufacturers for drug supplies that will be used in clinical trials of our product candidates, including arhalofenate, and for commercialization of any of our product candidates that receive regulatory approval.

The facilities used by our contract manufacturers to manufacture the product candidates must be approved by the FDA pursuant to inspections that will be conducted only after we submit an NDA to the FDA, if at all. We do not control the manufacturing process of our product candidates and are completely dependent on our contract manufacturing partners for compliance with the FDA's requirements for manufacture of finished pharmaceutical products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the FDA's strict regulatory requirements of safety, purity and potency, we will not be able to secure and/or maintain FDA approval for our product candidates. In addition, we have no direct control over the ability of the contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If our contract manufacturers cannot meet FDA standards, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates. No assurance can be given that our manufacturers can continue to make clinical and commercial supplies of arhalofenate, or future product candidates, at an appropriate scale and cost to make it commercially feasible.

In addition, we do not have the capability to package and distribute finished products to pharmacies and other customers. Prior to commercial launch, we will enter into agreements with one or more pharmaceutical product packager/distributor to ensure proper supply chain management once we are authorized to make commercial sales of our product candidates. If we receive marketing approval from the FDA, we intend to sell pharmaceutical product packaged and distributed by such suppliers. Although we have entered into agreements with our current contract manufacturers and packager/distributor for clinical trial material, we may be unable to maintain an agreement on commercially reasonable terms, which could have a material adverse impact upon our business.

We rely on limited sources of supply for the drug substance for our lead product candidate, arhalofenate, and any disruption in the chain of supply may cause delay in developing and commercializing arhalofenate.

It is our expectation that only one supplier of drug substance and one supplier of drug product will be qualified by the FDA. If supply from an approved vendor is interrupted, there could be a significant disruption in commercial supply of arhalofenate. An alternative vendor would need to be qualified through an NDA supplement which would be expensive and could result in further delay. The FDA or other regulatory agencies outside of the U.S. may also require additional studies if a new drug substance or drug product supplier is relied upon for commercial production. These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of arhalofenate, and cause us to incur additional costs. Furthermore, if our suppliers fail to deliver the required commercial quantities of active pharmaceutical ingredient on a timely basis and at commercially reasonable prices, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our supply chain for arhalofenate may be delayed, which could inhibit our ability to generate revenues.

Manufacturing issues may arise that could increase product and regulatory approval costs or delay commercialization of arhalofenate.

We are increasing the manufacturing batch size in preparation of our Phase 3 study and commercial supplies. As the process is scaled up it may reveal manufacturing challenges or previously unknown impurities which could require resolution in order to proceed with our planned clinical trials and obtain regulatory approval for the commercial marketing of arhalofenate. In the future, we may identify manufacturing issues or impurities which could result in delays in the clinical program and regulatory approval for arhalofenate, increases in our operating expenses, or failure to obtain or maintain approval for arhalofenate.

Table of Contents

Our reliance on third-party manufacturers entails risks, including the following:

- the inability to meet our product specifications and quality requirements consistently;
- a delay or inability to procure or expand sufficient manufacturing capacity;
- manufacturing and product quality issues related to scale-up of manufacturing;
- costs and validation of new equipment and facilities required for scale-up;
- a failure to comply with cGMP and similar foreign standards;
- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- the reliance on a limited number of sources, and in some cases, single sources for key materials, such that if we are unable to secure a sufficient supply of these key materials, we will be unable to manufacture and sell our product candidates in a timely fashion, in sufficient quantities or under acceptable terms;
- the lack of qualified backup suppliers for those materials that are currently purchased from a sole or single source supplier;
- operations of our third-party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier;
- carrier disruptions or increased costs that are beyond our control; and
- the failure to deliver our products under specified storage conditions and in a timely manner.

Any of these events could lead to clinical study delays, failure to obtain regulatory approval or impact our ability to successfully commercialize our products. Some of these events could be the basis for FDA or other regulatory authorities' action, including injunction, recall, seizure, or total or partial suspension of production.

We rely on third parties to conduct, supervise and monitor our clinical studies, and if those third parties perform in an unsatisfactory manner, it may harm our business.

We rely on contract service providers (CSPs) including clinical research organizations, clinical trial sites, central laboratories and other service providers to ensure the proper and timely conduct of our clinical trials. While we have agreements governing their activities, we have limited influence over their actual performance. We have relied and plan to continue to rely upon CSPs to monitor and manage data for our ongoing clinical programs for arhalofenate and our other product candidates, as well as the execution of nonclinical studies. We control only certain aspects of our CSPs' activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards and our reliance on the CSPs does not relieve us of our regulatory responsibilities.

We and our CSPs are required to comply with the FDA's guidance, which follows the International Conference on Harmonization Good Clinical Practice (ICH GCP), which are regulations and guidelines enforced by the FDA for all of our product candidates in clinical development. The FDA enforces the ICH GCP through periodic inspections of trial sponsors, principal investigators and clinical trial sites. If we or our CSPs fail to comply with the ICH GCP, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving our marketing applications. For example, upon inspection, the FDA may determine that our Phase 3 clinical trial for arhalofenate, does not comply with the ICH GCP. In addition, our Phase 3 clinical trials for arhalofenate will require a sufficiently large number of test subjects to evaluate the safety and effectiveness of arhalofenate. Accordingly, if our CSPs fail to comply with these regulations or fail to recruit a sufficient number of subjects, we may be required to repeat these Phase 3 clinical trials, which would delay the regulatory approval process.

Our CSPs are not our employees, and we cannot control whether or not they devote sufficient time and resources to our ongoing clinical and nonclinical programs. These CSPs may also have relationships with other entities, including our competitors, for whom they may also be conducting clinical studies, or other drug development activities which could harm our competitive position. We face the risk of potential unauthorized disclosure or misappropriation of our intellectual property by CSPs, which may reduce our trade secret protection and allow our potential competitors to access and exploit our proprietary technology. If our CSPs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize arhalofenate or our other product candidates. As a result, our financial results and the commercial prospects for arhalofenate and any other product candidates that we develop would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

[Table of Contents](#)

Risks Related to Commercialization of Our Product Candidates

The commercial success of arhalofenate, MBX-8025 and our other product candidates will depend upon the acceptance of these products by the medical community, including physicians, patients and health care payors.

If any of our product candidates, including arhalofenate and MBX-8025, receive marketing approval, they may nonetheless not gain sufficient market acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of any of our product candidates, including arhalofenate and MBX-8025, will depend on a number of factors, including the following:

- demonstration of clinical safety and efficacy in our clinical trials;
- the risk/benefit profile of our products such as arhalofenate;
- the relative convenience, ease of administration and acceptance by physicians, patients and health care payors;
- the prevalence and severity of any side effects;
- the safety of product candidates seen in a broader patient group, including its use outside the approved indications;
- limitations or warnings contained in the FDA and other regulatory authorities approved label for the relevant product candidate;
- acceptance of the product by physicians, other health care providers and patients as a safe and effective treatment;
- the potential and perceived advantages of product candidates over alternative treatments;
- the timing of market introduction of competitive products;
- pricing and cost-effectiveness;
- the effectiveness of our or any future collaborators' sales and marketing strategies;
- our ability to obtain formulary approval;
- our ability to obtain and maintain sufficient third-party coverage or reimbursement, which may vary from country to country; and
- the effectiveness of our or any future collaborators' sales, marketing and distribution efforts.

If any of our product candidates, including arhalofenate, is approved but does not achieve an adequate level of acceptance by physicians, patients and health care payors, we may not generate sufficient revenue and we may not become or remain profitable.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenue.

We currently do not have an organization for the sales, marketing and distribution of pharmaceutical products and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order to market any products that may be approved, including arhalofenate and MBX-8025, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. We may enter into strategic partnerships with third parties to commercialize our product candidates, including arhalofenate and MBX-8025.

If we are unable to build our own sales force or negotiate a strategic partnership for the commercialization of arhalofenate, we may be forced to delay the potential commercialization of arhalofenate, or reduce the scope of our sales or marketing activities for arhalofenate. If we elect to increase our expenditures to fund commercialization activities ourselves, we will need to obtain additional capital, which may not be available to us on acceptable terms, or at all. If we do not have sufficient funds, we will not be able to bring arhalofenate to market or generate product revenue.

If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate sufficient product revenue and may not become profitable. We will be competing with companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

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

Table of Contents

If we obtain approval to commercialize any products outside of the U.S., a variety of risks associated with international operations could materially adversely affect our business.

If our product candidates are approved for commercialization, we intend to enter into agreements with third parties to market those product candidates outside the U.S., including for arhalofenate and MBX-8025. We expect that we will be subject to additional risks related to international operations, including the following:

- different regulatory requirements for drug approvals in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, pandemics, or natural disasters including earthquakes, typhoons, volcanic eruptions, floods and fires.

We have no prior experience in these areas. In addition, there are complex regulatory, tax, labor and other legal requirements imposed by both the European Union and many of the individual countries in Europe with which we will need to comply. Many U.S.-based biopharmaceutical companies have found the process of marketing their own products in Europe to be very challenging.

If our competitors develop and market products that are more effective, safer or less expensive than arhalofenate, our commercial opportunities will be negatively impacted.

The life sciences industry is highly competitive, and we face significant competition from other pharmaceutical, biopharmaceutical and biotechnology companies and possibly from academic institutions, government agencies and private and public research institutions that are researching, developing and marketing products designed to address the treatment of gout. Our competitors may have significantly greater financial, manufacturing, marketing and drug development resources. Large pharmaceutical companies, in particular, have extensive experience in the clinical testing of, obtaining regulatory approvals for, and marketing of, drugs. New developments, including the development of other pharmaceutical technologies and methods of treating disease, occur in the pharmaceutical and life sciences industries at a rapid pace.

These developments may render our product candidates obsolete or noncompetitive. Compared to us, potential competitors may have substantially greater:

- research and development resources, including personnel and technology;
- regulatory experience;
- experience in pharmaceutical development and commercialization;
- ability to negotiate competitive pricing and reimbursement with third-party payors;
- experience and expertise in exploitation of intellectual property rights; and
- capital resources.

As a result of these factors, our competitors may obtain regulatory approval of their products more rapidly than we do or may obtain patent protection or other intellectual property rights that limit our ability to develop or commercialize our product candidates. The competitors may also develop products that are more effective, better tolerated, more useful and less costly than our products and they may also be more successful in manufacturing and marketing their products.

Formulary approval and reimbursement may not be available for arhalofenate, MBX-8025 and our other product candidates, which could make it difficult for us to sell our products profitably.

Obtaining formulary approval can be an expensive and time consuming process. We cannot be certain if and when we will obtain formulary approval to allow us to promote our product candidates, including arhalofenate and MBX-8025, into our target markets. Failure to obtain timely formulary approval will limit our commercial success.

Table of Contents

Furthermore, market acceptance and sales of arhalofenate, MBX-8025 or any other product candidates that we develop, will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A prevailing trend in the U.S. health care industry and elsewhere is cost containment. Government authorities and these third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Reimbursement may impact the demand for, or the price of, any product for which we obtain marketing approval. We cannot be sure that reimbursement will be available for arhalofenate, or any other product candidates. Also, reimbursement amounts may reduce the demand for, or the price of, our products. If reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize arhalofenate, or any other product candidates that we develop.

There have been a number of legislative and regulatory proposals to change the health care system in the U.S. and in some foreign jurisdictions that could affect our ability to sell any future products profitably. These legislative and regulatory changes may negatively impact the reimbursement for any future products, following approval. The availability of generic treatments may also substantially reduce the likelihood of reimbursement for any future products, including arhalofenate. The application of user fees to generic drug products will likely expedite the approval of additional generic drug treatments. We expect to experience pricing pressures in connection with the sale of arhalofenate and any other product candidate that we develop, due to the trend toward managed health care, the increasing influence of health maintenance organizations and additional legislative changes.

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

If we are unable to promptly obtain coverage and profitable payment rates from both government funded and private payors for any of our product candidates, including arhalofenate, it could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Even if we receive regulatory approval for arhalofenate or MBX-8025, we will be subject to ongoing FDA and other regulatory obligations and continued regulatory review, which may result in significant additional expense and limit our ability to commercialize arhalofenate or MBX-8025.

Any regulatory approvals that we or potential collaboration partners receive for arhalofenate, MBX-8025 or future product candidates, may also be subject to limitations on the indicated uses for which the product may be marketed or contain requirements for potentially costly post-marketing studies. In addition, even if approved, the labeling, packaging, adverse event reporting, storage, advertising, promotion and recordkeeping for any product will be subject to extensive and ongoing regulatory requirements. The subsequent discovery of previously unknown problems with a product, including AEs of unanticipated severity or frequency, may result in restrictions on the marketing of the product, and could include withdrawal of the product from the market. Depending on any safety issues associated with our product candidates that are approved, the FDA may require a REMS, thereby imposing certain restrictions on the sale and marketability of such products or additional post-marketing requirements.

Regulatory policies may change and additional government regulations may be enacted that could prevent or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the U.S. or abroad. If we are not able to maintain regulatory compliance, we might not be permitted to market arhalofenate or future products, if any, and we may not achieve or sustain profitability.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical studies, and will face an even greater risk if we sell our product candidates commercially. An individual may bring a liability claim against us if one of our product candidates causes, or merely appears to have caused, an injury. If we cannot successfully defend ourselves against product liability claims, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in the following:

Table of Contents

- decreased demand for our product candidates;
- impairment to our business reputation;
- withdrawal of clinical study participants;
- distraction of management's attention from our primary business;
- substantial monetary awards to patients or other claimants;
- the inability to commercialize our product candidates; and
- loss of revenues.

We do carry product liability insurance for our clinical studies. Further, we intend to expand our insurance coverage to include the sale of commercial products if marketing approval is obtained for any of our product candidates. However, we may be unable to obtain this product liability insurance on commercially reasonable terms and with insurance coverage that will be adequate to satisfy any liability that may arise. On occasion, large judgments have been awarded in class action or individual lawsuits relating to marketed pharmaceuticals. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

The success of our business depends primarily upon our ability to identify, develop and commercialize product candidates. Because we have limited financial and managerial resources, we focus on product candidates for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or other indications that later prove to have greater commercial potential. We may focus our efforts and resources on product candidates that ultimately prove to be unsuccessful.

If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to retain sole development and commercialization rights.

We are planning to study the combination of arhalofenate plus febuxostat in our planned Phase 3 program and if the results of these studies are positive, we will only be able to commercialize this combination if we are able to obtain febuxostat from an FDA qualified supplier, which we may not be able to do.

In order to commercialize a fixed dose combination product containing arhalofenate and febuxostat we would need to obtain febuxostat drug substance from a supplier that has been qualified by the FDA. If we are not able to identify a supplier, or if the supplier is not able to receive approval, we will not be able to receive approval for our fixed-dose combination product. In addition, we may need a license if the supplier's manufacturing process or final product infringes another party's valid patent. If we are not successful at obtaining a required license our ability to commercialize arhalofenate may be significantly diminished.

Risks Related to Our Intellectual Property

If we are unable to obtain or protect intellectual property rights related to our products and product candidates, we may not be able to compete effectively in our market.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our products and product candidates. The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own, co-own or in-license may fail to result in issued patents with claims that cover the products in the U.S. or in other countries. If this were to occur, early generic competition could be expected against arhalofenate and other product candidates in development. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing based on a pending patent application. Even if patents do successfully issue, third parties may challenge their validity, enforceability, scope or ownership, which may result in such patents, or our rights to such patents, being narrowed or invalidated. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property or prevent others from designing around our claims. If the patent applications we hold or license with respect to arhalofenate fail to issue or if their breadth or strength of protection is threatened, it could dissuade companies from collaborating with us and threaten our ability to commercialize our products. We cannot offer any assurances about which, if any, patents will issue or whether any issued patents will be found invalid or unenforceable, will be challenged by third parties or will adequately protect our products

Table of Contents

and product candidates. Further, if we encounter delays in development or regulatory approvals, the period of time during which we could market arhalofenate under patent protection could be reduced. Since patent applications in the U.S. and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we or our licensors were the first to file any patent application related to arhalofenate or our other product candidates. Furthermore, if third parties have filed such patent applications, an interference proceeding in the U.S. can be provoked by a third party or instituted by us to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license it from the prevailing party, which may not be available on commercially reasonable terms or at all.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable, processes for which patents are difficult to enforce and other elements of our drug discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. Although we expect all of our employees to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed, that such agreements provide adequate protection and will not be breached, that our trade secrets and other confidential proprietary information will not otherwise be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

Further, the laws of some foreign countries do not protect patents and other proprietary rights to the same extent or in the same manner as the laws of the U.S. As a result, we may encounter significant problems in protecting and defending our intellectual property abroad. We may also fail to pursue or obtain patents and other intellectual property protection relating to our products and product candidates in all foreign countries.

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts or otherwise affect our business.

Our commercial success depends in part on our avoiding infringement and other violations of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the U.S., involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions and inter party re-examination proceedings before the U.S. Patent and Trademark Office (U.S. PTO) and its foreign counterparts. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we and our collaborators are developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, and as we gain greater visibility and market exposure as a public company, the risk increases that our product candidates or other business activities may be subject to claims of infringement of the patent and other proprietary rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of arhalofenate and/or our other product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patent may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all. In addition, we may be subject to claims that we are infringing other intellectual property rights, such as trademarks or copyrights, or misappropriating the trade secrets of others, and to the extent that our employees, consultants or contractors use intellectual property or proprietary information owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful infringement or other intellectual property claim against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our

Table of Contents

affected products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize one or more of our product candidates, which could harm our business significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our products or product candidates, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

We license certain key intellectual property from third parties, and the loss of our license rights could have a materially adverse effect on our business.

We are a party to a number of technology licenses that are important to our business and expect to enter into additional licenses in the future. For example, we rely on an exclusive license to certain patents, proprietary technology and know-how from DiaTex, which include arhalofenate. During the term of the exclusive license with DiaTex we may perform research and development of compounds and products for the treatment of human disease based on the patents, proprietary technology and know-how from DiaTex. If we fail to comply with our obligations under our agreement with DiaTex, including our obligations to pay royalty payments during the development and commercialization of arhalofenate, or our other license agreements, or if we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to develop or market products covered by the license, including in the case of the DiaTex license, arhalofenate, which would have a materially adverse effect on our business.

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

Competitors may infringe or otherwise violate our patents, the patents of our licensors or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing. The initiation of a claim against a third party may also cause the third party to bring counter-claims against us.

We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the U.S. Our business could be harmed if in a litigation if the prevailing party does not offer us a license on commercially reasonable terms. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the U.S. PTO and foreign patent agencies in several stages over the lifetime of the patent. The U.S. PTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors that control the prosecution and maintenance of our licensed patents fail to maintain the patents and patent applications covering our product candidates, we may lose our rights and our competitors might be able to enter the market, which would have a material adverse effect on our business.

[Table of Contents](#)

Risks Related to Our Business Operations and Industry

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

We are highly dependent on principal members of our executive team listed under “Business—Executive Officers of Registrant” of our Annual Report on Form 10-K as filed with the SEC on March 23, 2015. While we have entered into employment agreements or offer letters with each of our executive officers, any of them could leave our employment at any time, as all of our employees are “at will” employees. We do not maintain “key person” insurance for any of our executives or other employees. Recruiting and retaining other qualified employees for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of skilled executives in our industry, which is likely to continue. We also experience competition from universities and research institutions for the hiring of scientific and clinical personnel. As a result, competition for skilled personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. In addition, failure of any of our clinical studies may make it more challenging to recruit and retain qualified personnel. If we are unable to successfully recruit key employees or replace the loss of services of any executive or key employee, it may adversely affect the progress of our research, development and commercialization objectives.

In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us, which could also adversely affect the progress of our research, development and commercialization objectives.

We will need to expand our organization, and we may experience difficulties in managing this growth, which could disrupt our operations.

As of March 31, 2015, we had 18 full-time employees. As our company matures, we expect to expand our employee base to increase our managerial, clinical, scientific and engineering, operational, sales, and marketing teams. Future growth would impose significant additional responsibilities on our management, including the need to identify, recruit, maintain, motivate and integrate additional employees, consultants and contractors. Also, our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize arhalofenate and our other product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

Our internal computer systems, or those used by our contract research organizations or other contractors or consultants, may fail or suffer security breaches.

Despite the implementation of security measures, our internal computer systems and those of our contract research organizations and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our product candidate development programs. For example, the loss of clinical study data from completed or ongoing clinical studies for a product candidate could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach was to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of any product candidates could be delayed.

Risks Relating to Owning Our Common Stock

An active trading market for our common stock may not develop and the market price for our common stock may decline in value.*

Our common stock is listed on the NASDAQ Capital Market under the symbol “CBAY”. Historically, trading volume for our common stock has been very limited. The historical trading prices of our common stock on the NASDAQ Capital Market may not be indicative of the price levels at which our common stock will trade in the future, and we cannot predict the extent to which investor interest in us generally will lead to the development of an active public trading market for our common stock or how liquid that public market may become.

Table of Contents

Our stock price may be volatile, and our stockholders' investment in our stock could decline in value.

The trading price of our common stock is likely to be volatile. Our stock price could be subject to wide fluctuations in response to a variety of factors, including:

- adverse results or delays in preclinical testing or clinical trials;
- inability to obtain additional funding;
- any delay in filing an IND or NDA for any of our future product candidates or any adverse development or perceived adverse development with respect to the FDA's review of that IND or NDA;
- failure to maintain our existing collaborations or enter into new collaborations;
- failure of our collaboration partners to elect to develop or commercialize product candidates under our collaboration agreements or the termination of any programs under our collaboration agreements;
- failure by us or our licensors and collaboration partners to prosecute, maintain or enforce our intellectual property rights;
- failure to successfully develop and commercialize our future product candidates;
- changes in laws or regulations applicable to future products;
- inability to obtain adequate product supply for our future product candidates or the inability to do so at acceptable prices;
- adverse regulatory decisions;
- introduction of new products, services or technologies by our competitors;
- failure to meet or exceed financial projections we may provide to the public;
- failure to meet or exceed the estimates and projections of the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us, our collaboration partners or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- additions or departures of key scientific or management personnel;
- significant lawsuits, including patent or stockholder litigation;
- changes in the market valuations of similar companies;
- sales of our common stock by us or our stockholders in the future; and
- trading volume of our common stock.

In addition, companies trading in the stock market in general have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

Our executive officers, directors and principal stockholders own a significant percentage of our stock and will be able to exert significant control over matters submitted to our stockholders for approval.

Our executive officers, directors and stockholders who own more than 5% of our outstanding common stock, together beneficially own a significant number of shares of our common stock. Therefore, these stockholders will have the ability to influence us through this ownership position as well as influence all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to substantially influence elections of directors, amendments to our organizational documents, or approval of any merger, sale of assets, or other major corporate action. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

Table of Contents

We have identified material weaknesses in our internal controls over financial reporting.

Effective internal controls are necessary for us to produce reliable financial reports and are important to help prevent financial fraud. Our independent registered public accounting firm will not be required to attest to the effectiveness of our internal control over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act until the later of the year following our first annual report required to be filed with the SEC or the date we are no longer an “emerging growth company” as defined in the JOBS Act, because we are taking advantage of the exemptions contained in the JOBS Act. We continue to build the infrastructure to allow us to assess the effectiveness of our internal control over financial reporting by hiring qualified personnel and specialists to assist us in executing phased project efforts to improve our accounting systems, disclosure policies, procedures and controls. This effort is on-going and will be costly and time consuming. If we are unsuccessful in building an appropriate accounting infrastructure, we may not be able to prepare and disclose, in a timely manner, our financial statements and other required disclosures, or comply with existing or new reporting requirements. We previously identified material weaknesses in our internal control over financial reporting in 2014, which we had remediated by the end of 2014. We cannot assure you that there will not be material weaknesses in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we are unable to maintain effective internal control over financial reporting, or if our independent registered public accounting firm determines we have a material weakness in our internal control over financial reporting, we could lose investor confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by The NASDAQ Stock Market, the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

Sales of a substantial number of shares of our common stock in the public market by our existing stockholders could cause our stock price to fall.

Sales of a substantial number of shares of our common stock in the public market, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that sales may have on the prevailing market price of our common stock.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that significant additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. For example, on July 28, 2014, we consummated a public offering of our common stock on a registration statement on Form S-1 pursuant to which we sold 4,600,000 shares of our common stock, including shares sold in connection with the exercise by the underwriters in the offering of an over-allotment of 600,000 shares, at a price of \$5.50 per share, for aggregate net proceeds of \$23.0 million. On November 7, 2014, we filed a \$100 million registration statement on Form S-3 with the SEC and also entered into an ATM to sell up to \$25 million of common stock under the registration statement under which we have sold additional shares of our

Table of Contents

common stock for net proceeds to us of \$4.3 million during the period January 1, 2015 through March 1, 2015. If in the future, we sell common stock, convertible securities or other equity securities, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders. Pursuant to our equity incentive plans, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. The number of shares available for future grant under our equity incentive plans as of March 31, 2015 was 330,192 shares.

We do not anticipate paying cash dividends, and accordingly, stockholders must rely on stock appreciation for any return on their investment.

We do not anticipate paying cash dividends in the future. As a result, only appreciation of the price of our common stock, which may never occur, will provide a return to stockholders. Investors seeking cash dividends should not invest in our common stock. In addition, our ability to pay cash dividends is currently prohibited without the prior consent of the lender pursuant to the terms of our loan and security agreement with Silicon Valley Bank and Oxford Finance LLC.

We may be subject to securities litigation, which is expensive and could divert management attention.

Our share price may be volatile, and in the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our amended and restated certificate of incorporation and our bylaws may delay or prevent an acquisition of us. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, who are responsible for appointing the members of our management team. In addition, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits, with some exceptions, stockholders owning in excess of 15% of our outstanding voting stock from merging or combining with us. Finally, our charter documents establish advance notice requirements for nominations for election to our board of directors and for proposing matters that can be acted upon at stockholder meetings. Although we believe these provisions together provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders.

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

Unregistered Sale of Equity Securities

From January 1, 2015, to March 31, 2015, we issued an aggregate of 122,592 shares of common stock to 56 of our stockholders upon the exercise of warrants exercisable for shares of our common stock. The 122,592 shares of common stock were issued pursuant to both cash and net exercise provisions as provided in the warrants. Specifically, 74,136 shares of our common stock were issued in exchange for \$426,282 and 48,456 shares of our common stock were issued in exchange for cancellation of 52,793 shares of our common stock. The issuances were in reliance on Rule 506 and Regulation D under the Securities Act and, with respect to net exercises, Section 3(a)(9) of Securities Act.

Use of Proceeds

We consummated a public offering of our common stock on a registration statement on Form S-1 (File No. 333-195127) that was declared effective by the SEC on July 21, 2014, pursuant to which we sold 4,600,000 shares of our common stock, including shares sold in connection with the exercise by the underwriters in the offering of an over-allotment of 600,000 shares, at a price of \$5.50 per share, for aggregate gross proceeds of \$25.3 million which we refer to as our 2014 public offering. The offering was made pursuant to a prospectus dated July 21, 2014. Cowen and Company, LLC and Stifel, Nicolaus & Company, Incorporated were the managing underwriters in the offering.

[Table of Contents](#)

As of March 31, 2015, we estimate that we had used approximately \$12.5 million of the proceeds on the development of MBX-8025 and ongoing development of arhalofenate and approximately \$3.8 million for working capital, capital expenditures and other general corporate purposes. The remaining \$6.8 million is held in cash, cash equivalents and short term investments. There has been no material change in the expected use of the net proceeds from our public offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b) on July 22, 2014 (File No. 333-195127).

Item 6. Exhibits

See the Exhibit Index which follows the signature page of this Quarterly Report on Form 10-Q, which is incorporated herein by reference.

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.

CYMABAY THERAPEUTICS, INC.

By: /s/ Harold Van Wart
Harold Van Wart
Chief Executive Officer
(Duly Authorized Officer and Principal Executive Officer)

Date: May 11, 2015

By: /s/ Sujal Shah
Sujal Shah
Chief Financial Officer
(Principal Financial and Accounting Officer)

Date: May 11, 2015

INDEX TO EXHIBITS

<u>Exhibit Number</u>	<u>Description of Document</u>
3.1	Amended and Restated Certificate of Incorporation (Filed with the SEC as Exhibit 3.1 to our Amendment No. 2 to Registration Statement on Form 10, filed with the SEC on October 17, 2013, SEC File No. 000-55021).
3.2	Amended and Restated By-Laws. (Filed with the SEC as Exhibit 3.2 to our Amendment No. 2 to Registration Statement on Form 10, filed with the SEC on October 17, 2013, SEC File No. 000-55021).
4.1	Reference is made to Exhibits 3.1 and 3.2.
4.2	Form of Registration Rights Agreement (Filed with the SEC as Exhibit 4.2 to our Amendment No. 2 to Registration Statement on Form 10, filed with the SEC on October 17, 2013, SEC File No. 000-55021).
4.3	Form of 2013 Financing Warrant (Filed with the SEC as Exhibit 4.3 to our Amendment No. 2 to Registration Statement on Form 10, filed with the SEC on October 17, 2013, SEC File No. 000-55021).
4.4	Amendment No. 1 to Registration Rights Agreement. (Filed with the SEC as Exhibit 4.4 to our Form 10-K, filed with the SEC on March 31, 2014, SEC File No. 000-55021).
10.1	Amended and Restated Non-Employee Director Compensation Policy (Filed with the SEC as Exhibit 10.20 to our Form 10-K, filed with the SEC on March 23, 2015, SEC File No 001-36500).
31.1	Certification of Chief Executive Officer pursuant to Rule 13-a-14(a) or Rule 15(d)-14(a) of the Exchange Act
31.2	Certification of Chief Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Exchange Act
32.1	Certification of Chief Executive Officer and Chief Financial Officer pursuant to 13a-14(b) or 15d-14(b) of the Exchange Act
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Schema Linkbase Document
101.CAL	XBRL Taxonomy Calculation Linkbase Document
101.DEF	XBRL Taxonomy Definition Linkbase Document
101.LAB	XBRL Taxonomy Labels Linkbase Document
101.PRE	XBRL Taxonomy Presentation Linkbase Document

CERTIFICATIONS

I, Harold Van Wart, certify that:

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

Date: May 11, 2015

/s/ Harold Van Wart

Harold Van Wart

Chief Executive Officer

CERTIFICATIONS

I, Sujal Shah, certify that:

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

Date: May 11, 2015

/s/ Sujal Shah

Sujal Shah

Chief Financial Officer

CERTIFICATION

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Harold Van Wart, Chief Executive Officer of CymaBay Therapeutics, Inc. (the “Company”), and Sujal Shah, Chief Financial Officer of the Company, each hereby certifies that, to the best of his knowledge:

1. The Company’s Quarterly Report on Form 10-Q for the period ended March 31, 2015, to which this Certification is attached as Exhibit 32.1 (the “Periodic Report”), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act, and
2. The information contained in the Periodic Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

In Witness Whereof, the undersigned have set their hands hereto as of May 11, 2015.

/s/ Harold Van Wart

Harold Van Wart
Chief Executive Officer

/s/ Sujal Shah

Sujal Shah
Chief Financial Officer

This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of CymaBay Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.