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4,000,000 Shares



**Common Stock**

We are offering shares of our common stock. Our common stock is listed on the NASDAQ Capital Market under the symbol "CBAY." The last reported sale price of our common stock on the NASDAQ Capital Market on July 21, 2014, was \$5.97 per share.

We are an "emerging growth company" as that term is used in the Jumpstart Our Business Startups Act of 2012 and, as such, have elected to comply with certain reduced public company reporting requirements for this prospectus and future filings. See "Prospectus Summary—We are an "Emerging Growth Company."

**Our business and an investment in our common stock involve significant risks. These risks are described under the caption "[Risk Factors](#)" beginning on page 9 of this prospectus.**

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

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	<i>Per Share</i>	<i>Total</i>
<b>Public offering price</b>	\$ 5.50	\$22,000,000
<b>Underwriting discount<sup>(1)</sup></b>	\$ 0.33	\$ 1,320,000
<b>Proceeds, before expenses, to us</b>	\$ 5.17	\$20,680,000

(1) See "[Underwriting](#)" beginning on page 112 for a full description of compensation payable to the underwriters.

The underwriters may also purchase up to an additional 600,000 shares from us at the public offering price, less the underwriting discount, within 30 days from the date of this prospectus to cover over-allotments.

The underwriters expect to deliver the shares against payment in New York, New York on July 25, 2014.

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**Cowen and Company**

**Stifel**

**Roth Capital Partners**

**National Securities Corporation**

July 21, 2014

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[Table of Contents](#)

[Index to Financial Statements](#)

TABLE OF CONTENTS

<a href="#">Prospectus Summary</a>	1	<a href="#">Business</a>	54
<a href="#">Risk Factors</a>	9	<a href="#">Management</a>	88
<a href="#">Special Note Regarding Forward-Looking Statements</a>	36	<a href="#">Executive and Director Compensation</a>	92
<a href="#">Market, Industry and Other Data</a>	36	<a href="#">Transactions With Related Persons</a>	101
<a href="#">Use of Proceeds</a>	38	<a href="#">Principal Stockholders</a>	102
<a href="#">Market Price of Common Stock</a>	38	<a href="#">Description of Capital Stock</a>	105
<a href="#">Dividend Policy</a>	38	<a href="#">Material U.S. Federal Income and Estate Tax Considerations</a>	
<a href="#">Dilution</a>	39	<a href="#">For Non-U.S. Holders</a>	109
<a href="#">Capitalization</a>	40	<a href="#">Underwriting</a>	112
<a href="#">Selected Financial Data</a>	41	<a href="#">Legal Matters</a>	117
<a href="#">Management's Discussion and Analysis of Financial</a>		<a href="#">Experts</a>	117
<a href="#">Condition and Results of Operations</a>	43	<a href="#">Where You Can Find Additional Information</a>	117
		<a href="#">Index To Financial Statements</a>	F-1

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You should rely only on the information contained in this prospectus and any related free writing prospectus that we may provide to you in connection with this offering. We have not, and the underwriters have not, authorized any other person to provide you with different information. If anyone provides you with different or inconsistent information, you should not rely on it. We are not, and the underwriters are not, making an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should assume that the information appearing in this prospectus is accurate only as of the date on the front cover of this prospectus. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside the United States: neither we nor any of the underwriters has done anything that would permit this offering or possession or distribution of this prospectus or any free writing prospectus we may provide to you in connection with this offering in any jurisdiction where action for that purpose is required, other than in the United States. You are required to inform yourselves about and to observe any restrictions relating to this offering and the distribution of this prospectus and any such free writing prospectus outside of the United States.

## PROSPECTUS SUMMARY

*The following summary highlights selected information contained elsewhere in this prospectus and does not contain all of the information that you should consider in making your investment decision. Before investing in our common stock, you should carefully read this entire prospectus, including our financial statements and the related notes included in this prospectus and the information set forth under the headings “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.”*

### **Our Company**

CymaBay Therapeutics, Inc. is focused on developing therapies to treat serious rare and orphan metabolic diseases or more prevalent diseases with high unmet medical need. Arhalofenate, our lead product candidate, is being developed for the treatment of gout. Arhalofenate has successfully completed three 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 arhalofenate’s ability to prevent or reduce flares while also lowering sUA differentiates it from currently available treatments for gout. Arhalofenate has established a favorable safety profile in clinical trials involving nearly 1,000 patients exposed to date. We are currently investigating arhalofenate in a 12-week Phase 2b clinical trial in patients with gout and expect to report data from this trial in the first half of 2015. 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 considering pursuing MBX-8025 in a number of orphan diseases in which these attributes would be beneficial, such as homozygous familial hypercholesterolemia (HoFH), severe hypertriglyceridemia (SHTG) and primary biliary cirrhosis (PBC). We also believe that MBX-8025 would have utility in the treatment of the more prevalent, but high unmet need, indication of nonalcoholic steatohepatitis (NASH). We plan to initiate one or more proof-of-concept studies for MBX-8025 in the first half of 2015.

We believe arhalofenate has the potential to address unmet needs in the treatment of gout. Of the eight million patients with gout in the U.S., we estimate that over three million are on urate lowering therapy (ULT). Approximately one million of these patients on ULT continue to experience three or more flares per year, with significant impact to patient quality of life and the health care system. The two primary goals of gout treatment are the prevention of flares and lowering of sUA. The fundamental limitation in achieving these goals is that all currently available ULTs cause an increase in flares upon initiation of treatment, leading many patients to discontinue or avoid therapy. Given this increase in flares, standard of care includes prophylaxis with colchicine and use of anti-inflammatory medications, which are often poorly tolerated or inadvisable for use in gout patients due to their side effects. Despite prophylaxis with colchicine, many patients continue to experience flares. We believe that by decreasing flares while lowering sUA, arhalofenate has the potential to treat patients with gout without the need for colchicine or other anti-inflammatory medications and would thus be differentiated from all currently available gout therapies.

**Our Pipeline**

Our pipeline includes three unpartnered clinical stage product candidates and a number of preclinical programs.

Program	Indication	Partner	Research	Preclinical	P1	P2
Arhalofenate	Gout					
MBX-8025	Orphan Disease					
MBX-2982	Diabetes					
Target	Diabetes	Johnson & Johnson				
Targets	Diabetes	Johnson & Johnson				

**Arhalofenate**

Arhalofenate has demonstrated a favorable safety profile and a pattern of reductions in flare parameters (incidence, duration and severity) and lowering of sUA in three Phase 2 clinical trials in patients with gout. These trials consisted of a monotherapy study, and one study each in combination with febuxostat and allopurinol, the most widely used ULTs. Allopurinol and febuxostat reduce sUA by inhibiting its production. In contrast, arhalofenate lowers sUA by increasing the excretion of uric acid. While all patients in these trials received colchicine as a treatment to prevent ULT-initiated flares, those groups treated with arhalofenate experienced a decrease in the number, duration and severity of flares. In addition, patients treated with arhalofenate experienced reductions in sUA across all three trials.

Based on our three completed Phase 2 gout clinical trials, we have demonstrated that arhalofenate:

- has a safety and tolerability profile appropriate for continued development for gout;
- provides dose-dependent improvements in flare parameters and reductions in sUA;
- provides a rationale for the product profile as a differentiated single agent to reduce flares and sUA without colchicine or other anti-inflammatory drugs; and
- can be combined with febuxostat to offer patients greater sUA lowering without causing an increase in flares.

The goal of our ongoing Phase 2b clinical trial is to investigate the potential benefit of arhalofenate monotherapy with regard to flare prevention and sUA lowering in a more robust, longer trial. Importantly, this trial will also study the benefits of two doses of arhalofenate monotherapy, including a higher dose than we studied in previous gout trials, without colchicine. This randomized, double-blind, active comparator- and placebo-controlled trial will evaluate the safety, flare prevention and sUA-lowering activity of arhalofenate in approximately 225 patients with a diagnosis of gout, hyperuricemia (elevated sUA levels) and a history of three or more flares in the last 12 months. The study has five arms including placebo, arhalofenate 600 mg, arhalofenate 800 mg, allopurinol 300 mg and allopurinol 300 mg plus colchicine 0.6 mg. The primary endpoint of the study is the flare incidence rate for the arhalofenate 800 mg arm versus allopurinol 300 mg following twelve weeks of treatment. A key secondary endpoint is the sUA responder rate (the percentage of patients that achieve sUA levels below 6 mg/dL) for the treatment arms. The study is designed to assess whether arhalofenate can provide sUA lowering comparable to the most commonly prescribed dose of allopurinol 300 mg and flare reduction similar to colchicine.

### ***MBX-8025***

MBX-8025 is a selective, potent peroxisome proliferator-activated receptor-delta (PPAR $\delta$ ) agonist that has potential therapeutic application for disorders linked to defects in lipid storage, handling and utilization and for certain diseases effecting liver function. The pharmacological action of MBX-8025 has been established in a Phase 2 clinical trial in patients with mixed dyslipidemia, which is characterized by elevated levels of low density lipoprotein cholesterol (LDL-C) and triglycerides (TGs) and below normal levels of high density lipoprotein cholesterol (HDL-C). In this trial, MBX-8025 demonstrated favorable effects on LDL-C, HDL-C, TGs and markers of liver health. However, we believe that its greatest benefit to patients and the best path for regulatory approval is likely to be in an orphan or other high unmet need indication. We have identified a range of indications linked to both lipid and hepatic disorders that may be applicable for treatment with MBX-8025, including HoFH (a rare genetic disorder characterized by extremely high levels of LDL-C), SHTG (a disorder characterized by extremely high levels of triglycerides) and PBC (an orphan autoimmune disease of the liver characterized by portal inflammation and destruction of intrahepatic bile ducts). We are currently exploring the feasibility and potential for use of MBX-8025 in HoFH and other orphan diseases. We plan to initiate one or more proof-of-concept studies for MBX-8025 in the first half of 2015.

### ***Other Programs and Product Candidates***

MBX-2982 is a potent selective agonist of G-coupled protein receptor 119 (GPR119), which has potential for use in the treatment of Type 2 diabetes. MBX-2982 has a dual-action in which it stimulates the release of incretin hormones in the gastrointestinal tract and promotes glucose-regulated insulin secretion in the pancreas. Based on the results from four Phase 1 and one Phase 2 clinical trials, we believe MBX-2982 has the potential to be used as a combination therapy for lowering glucose in patients with Type 2 diabetes. We do not anticipate conducting an additional study in diabetes until a suitable partner is identified.

We have licensed a lead optimization program targeted towards an undisclosed target for Type 2 diabetes to Johnson & Johnson. In addition, we have also licensed several lead discovery programs for undisclosed targets to Johnson & Johnson.

### **Our Strategy**

Our goal is to become a leading biopharmaceutical company focused on developing and commercializing proprietary new medicines for metabolic and rare diseases with high unmet need. Key elements of our strategy are to:

- develop arhalofenate as a dual-acting treatment to prevent or reduce flares and lower sUA in patients with gout;
- develop MBX-8025 for high unmet need or orphan indications linked to defects in lipid storage, handling and utilization and certain diseases effecting liver function;
- pursue partnerships to advance and commercialize arhalofenate and potentially other clinical candidates; and
- strengthen our patent portfolio and other means of protecting exclusivity.

### **Our Intellectual Property**

The arhalofenate portfolio consists of approximately 130 issued patents and 78 pending patent applications relating to composition, method of use or methods of manufacture. We believe our issued patents protect arhalofenate through at least 2019-2029 before accounting for any potential patent term extension. The MBX-8025 portfolio consists of approximately 83 issued patents and 41 pending patent applications related to composition and method of use that we believe protect it through at least 2024-2026 before accounting for any potential patent term extension.

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[Table of Contents](#)

[Index to Financial Statements](#)

**Risks Related to Our Business**

Our business is subject to numerous risks, as more fully described in the section entitled “Risk Factors” immediately following this prospectus summary. You should read these risks before you invest in our common stock. In particular, our risks include, but are not limited to, the following:

- We will need additional capital in the future to sufficiently fund our operations and research;
- 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 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;
- 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;
- 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 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;
- 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; and
- Our stock price may be volatile, and our stockholders’ investment in our stock could decline in value.

**Corporate Information**

CymaBay Therapeutics, Inc., was incorporated under the laws of the State of Delaware on October 5, 1988, originally under the name Transtech Corporation. Our executive offices are located at 7999 Gateway Blvd., Suite 130 Newark, CA 94560. The telephone number at our executive office is (510) 293-8800. Our corporate website address is [www.cymabay.com](http://www.cymabay.com). We do not incorporate the information contained on, or accessible through, our website into this prospectus, and you should not consider it part of this prospectus.

As used in this prospectus, “CymaBay,” “we,” “us,” and “our” refer to CymaBay Therapeutics, Inc. and its subsidiaries taken as a whole. The word trademark “CymaBay” is registered on the Principal Register of the United States Patent and Trademark Office. This prospectus also contains trademarks and trade names of other companies, and those trademarks and trade names are the property of their respective owners. We do not intend our use or display of other companies’ trademarks or trade names to imply a relationship with, or endorsement or sponsorship of us by, any other companies or products.

**We are an “Emerging Growth Company”**

We qualify as an “emerging growth company” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. As an “emerging growth company,” we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

- only two years of audited financial statements in addition to any required unaudited interim financial statements with correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure;
- reduced disclosure about our executive compensation arrangements;
- omitted compensation discussion and analysis;

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[Table of Contents](#)

[Index to Financial Statements](#)

- no requirement that we solicit non-binding advisory votes on executive compensation or golden parachute arrangements; and
- exemption from the auditor attestation requirement in the assessment of our internal control over financial reporting.

We intend to take advantage of the reduced disclosure obligations. Section 107 of the JOBS Act also provides that an emerging growth company can take advantage of the extended transition period provided in the Securities Act of 1933, as amended, or the Securities Act, for complying with new or revised accounting standards. In other words, an emerging growth company can elect to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to avail ourselves of this exemption to take advantage of the extended transition period for complying with new or revised accounting standards.

We could remain an emerging growth company until the earliest of (i) the last day of the first fiscal year in which our annual gross revenues exceed \$1 billion, (ii) the date that we become a “large accelerated filer” as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended, or the Exchange Act, which would occur if the market value of our common stock that are held by non-affiliates exceeds \$700 million as of the last business day of our most recently completed second fiscal quarter, (iii) the date on which we have issued more than \$1 billion in non-convertible debt during the preceding three-year period and (iv) the last day of the fiscal year following the fifth anniversary of the date of the first sale of our common equity securities pursuant to an effective registration statement under the Securities Act. At this time we expect to remain an “emerging growth company” for the foreseeable future.

CymaBay also qualifies as a “smaller reporting company” and thus has the advantage of not being required to provide the same level of disclosure as larger public companies.

On September 30, 2013, we engaged in a 1-for-79.5 reverse split of our preferred stock and common stock, which we refer to as the reverse stock split, and all of the shares of our outstanding preferred stock converted to common stock. Unless otherwise noted in this registration statement on Form S-1 of which this prospectus forms a part and except as set forth in the financial statements included in this prospectus, all share numbers and prices are presented on a reverse stock split basis.

[Table of Contents](#)

[Index to Financial Statements](#)

**The Offering**

Common stock offered by us:	4,000,000 shares
Common stock to be outstanding after this offering:	14,064,495 shares
Underwriters' over-allotment option:	The underwriters have an option to purchase up to 600,000 additional shares of common stock to cover over-allotments as described in "Underwriting."
Use of Proceeds	<p>We estimate that the net proceeds from the issuance of our common stock in this offering will be approximately \$19.8 million, or approximately \$22.8 million if the underwriters exercise their over-allotment option in full, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.</p> <p>We intend to use the net proceeds from this offering for the ongoing development of arhalofenate, the development of MBX-8025, working capital, capital expenditures and other general corporate purposes. See "Use of Proceeds" for additional information.</p>
Risk Factors	See "Risk Factors" beginning on page 9 in this prospectus for a discussion of factors that you should carefully consider before deciding to invest in shares of our common stock.
<b>NASDAQ Capital Market Symbol</b>	"CBAY"

The number of our shares of common stock outstanding is based on 10,064,495 shares of common stock outstanding as of March 31, 2014, and excludes the following:

- 906,796 shares issuable upon the exercise of outstanding stock options at a weighted average exercise price of \$6.13 per share;
- 222,861 shares issuable upon the exercise of outstanding incentive awards at a weighted average exercise price of \$5.00 per share;
- 643,251 additional shares reserved for future issuance under our equity incentive plan, including 500,000 shares approved for future issuance by our stockholders at our annual meeting on June 3, 2014; and
- 1,848,487 shares issuable upon the exercise of warrants held by our stockholders and lenders at a weighted average exercise price of \$5.70 per share.

Unless otherwise indicated, all information in this prospectus reflects and assumes no exercise of the underwriters' over-allotment option to purchase up to 600,000 additional shares of our common stock.



[Table of Contents](#)[Index to Financial Statements](#)

### Summary Financial Data

The following tables summarize our financial data and should be read together with the sections in this prospectus entitled “Selected Financial Data” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and our financial statements and related notes included elsewhere in this prospectus.

We have derived the statements of operations data for the years ended December 31, 2013 and 2012, from our audited financial statements included elsewhere in this prospectus. The statements of operations data for the three months ended March 31, 2014 and 2013, and the balance sheet data as of March 31, 2014, are derived from our unaudited interim financial statements included elsewhere in this prospectus. We have prepared the unaudited interim financial statements on the same basis as the audited financial statements and have included, in our opinion, all adjustments, consisting only of normal recurring adjustments, that we consider necessary for a fair presentation of the financial information set forth in those statements. You should read this data together with our audited financial statements and related notes appearing elsewhere in this prospectus and the information under the captions “Selected Financial Data” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.” Our historical results are not necessarily indicative of the results that should be expected in the future, and our interim results are not necessarily indicative of the results that should be expected for the full year or any other period.

	Three Months Ended		Year Ended December 31,	
	March 31, 2014	2013	2013	2012
Contract revenue	\$ —	\$ —	\$ —	\$ 3,050
Operating expenses:				
Research and development	2,615	1,490	4,525	9,280
General and administrative	2,500	925	4,871	4,208
Total operating expenses	5,115	2,415	9,396	13,488
Loss from operations	(5,115)	(2,415)	(9,396)	(10,438)
Other income (expense):				
Interest income	12	1	10	22
Interest expense	(184)	(212)	(822)	(841)
Other income (expense), net	(4,775)	(2)	135	2
Net loss	\$ (10,062)	\$ (2,628)	\$ (10,073)	\$ (11,255)
Net income (loss) attributable to common stockholders	\$ (10,062)	\$ (5,737)	\$ 243,994	\$ (23,899)
Net loss	(10,062)	(2,628)	(10,073)	(11,255)
Other comprehensive loss/income:				
Unrealized gains (losses) on marketable securities	1	—	2	(2)
Other comprehensive income (loss)	1	—	2	(2)
Comprehensive loss	\$ (10,061)	\$ (2,628)	\$ (10,071)	\$ (11,257)
Basic net income (loss) per common share	\$ (1.02)	\$ (985.06)	\$ 103.52	\$ (4,128.71)
Weighted average common shares outstanding used to calculate basic net income (loss) per common share	9,873,687	5,824	2,357,036	5,788
Diluted net loss per common share	\$ (1.02)	\$ (985.06)	\$ (3.54)	\$ (4,128.71)
Weighted average common shares outstanding used to calculate diluted net loss per common share	9,873,687	5,824	2,845,609	5,788

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[Table of Contents](#)

[Index to Financial Statements](#)

	As of March 31, 2014	
	Actual	As adjusted <sup>(1)</sup>
	(unaudited)	
	(in thousands)	
<b>Balance sheet data:</b>		
Cash, cash equivalents and marketable securities	\$28,533	\$ 48,290
Working capital	15,412	35,169
Total assets	31,196	50,953
Warrant liability	11,638	11,638
Facility loan	4,509	4,509
Total liabilities	19,820	19,820
Total stockholders' equity	11,376	31,133

(1) The as adjusted balance sheet data as of March 31, 2014, reflects receipt of the estimated net proceeds of \$19.8 million from the sale of common stock in this offering (assuming no exercise of the underwriter's option to purchase additional shares), after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

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[Table of Contents](#)

[Index to Financial Statements](#)

## RISK FACTORS

*Investing in our common stock involves a high degree of risk. You should consider carefully the risks and uncertainties described below, together with all of the other information in this prospectus, including the financial statements and the related notes appearing at the end of this prospectus, before deciding to invest in shares of our common stock. If any of the following risks actually occurs, our business, financial condition, results of operations and future prospects could be harmed. In that event, the market price of our common stock could decline and you could lose part or even all of your investment.*

### Risks Related to Our Financial Condition and Capital Requirements

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

We have consumed substantial amounts of capital to date as we continue our research and development activities, including our Phase 2b study of arhalofenate. As of March 31, 2014, we had cash and cash equivalents of approximately \$22.3 million and marketable securities of \$6.2 million. These funds were obtained through recent equity and debt financings including approximately \$28.8 million which we raised in aggregate net proceeds on September 30, 2013, \$2.2 million of additional net proceeds which we raised on October 31, 2013, and \$2.7 million of additional net proceeds which we raised on January 29, 2014. We believe that our existing cash will allow us to continue operation through the second quarter of 2015, and after giving effect to the estimated net proceeds of \$19.8 million in connection with this offering, we believe that our anticipated cash will allow us to continue operations through the end of 2015. 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 planned Phase 2b study of arhalofenate in patients with gout 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;

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## [Table of Contents](#)

### [Index to Financial Statements](#)

- 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 net losses of approximately \$10.1 million for the three months ended March 31, 2014, and \$10.1 million and \$11.3 million for the fiscal years ended 2013 and 2012, respectively. As of March 31, 2014, we had an accumulated deficit of \$358.9 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:

- 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 or those of its collaborators 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.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

***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, obtain the necessary regulatory approvals 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 successfully initiating and completing our Phase 2b and 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.

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, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a stockholder. 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.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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.

***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 seven Phase 2 clinical trials, including three 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 conducting a Phase 2b clinical trial for arhalofenate in preventing flares and reducing serum uric acid in gout patients prior to initiation of a Phase 3 program. There is no guarantee that our clinical trials will be completed or, if completed, will be successful. For example, the 800 mg dose of arhalofenate to be used in our Phase 2b gout trial is higher than doses of arhalofenate previously administered in our gout and T2DM programs, and may demonstrate unacceptable toxicities or lack of efficacy. We also plan to initiate one or more proof-of-concept studies for

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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;
- 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.

***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 to conduct this study. We believe that our existing cash will be sufficient to enable us to complete our Phase 2b study, which we anticipate completing the second quarter of 2015.

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

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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 three 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;
- 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 and 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.



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## [Table of Contents](#)

## [Index to Financial Statements](#)

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 arhalofenate as a monotherapy for the treatment of gout flares without the use of colchicine. If arhalofenate does not demonstrate efficacy in the treatment of such flares in our Phase 2b clinical trial, our ability to successfully commercialize arhalofenate may be adversely affected.***

We have not previously conducted a clinical trial of arhalofenate for the purpose of measuring its effect on flare reduction and control without the use of colchicine. We are conducting a Phase 2b clinical trial to investigate the potential benefit of arhalofenate monotherapy with regard to flare prevention and serum uric acid (sUA) lowering. In addition, our Phase 2b study will investigate the benefits of two doses of arhalofenate monotherapy, including a higher dose than we studied in previous gout studies, without colchicine. If we do not obtain favorable efficacy and safety results in the Phase 2b trial, our ability to successfully market arhalofenate could be adversely affected.

***We have never conducted a clinical trial of MBX-8025 for the indications which we are considering for MBX-8025. 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. As a result, although we believe that MBX-8025 may be beneficial to address the diseases for which we are considering redirecting the development of MBX-8025, 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. For example, we have completed two-year rodent carcinogenicity studies with MBX-8025 as well as some additional follow-up studies requested by the FDA, and are awaiting a response from the FDA. The results of these studies may determine whether or not we will be able to initiate any clinical trials of MBX-8025, or if we are, then 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;

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## [Table of Contents](#)

### [Index to Financial Statements](#)

- 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;
- 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, are 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 15 clinical trials with nearly 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 not obtained orphan drug designation for MBX-8025 for any indication 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, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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 not obtained orphan designation for MBX-8025 for any indication, and may not be able to obtain designation or any of the potential benefits associated with it. Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the candidate from competition because different drugs can be approved for the same condition and the first entity with an orphan drug designation to receive regulatory approval for a particular indication will receive marketing exclusivity. 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.

***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.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

***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 its 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 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 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.;

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## [Table of Contents](#)

### [Index to Financial Statements](#)

- 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 requiring 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;
- 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.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

***Even if we obtain FDA approval for arhalofenate, MBX-8025 or any of our other products in the U.S., we may never obtain approval for or commercialize arhalofenate, MBX-8025 or any of our other products 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;
- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology 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

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## [Table of Contents](#)

### [Index to Financial Statements](#)

- 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 Health Care Reform Law 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 Health Care Reform Law 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 Health Care Reform Law 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 Health Care Reform Law, 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.

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.

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[Table of Contents](#)

[Index to Financial Statements](#)

**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.***

We are currently transferring the drug substance manufacturing process to our selected contractor that will produce the supplies needed to meet clinical development, registration and forecasted commercial demand. 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 modifying the drug substance production process for arhalofenate at the selected commercial manufacturer to cost effectively remove impurities. As the modified process is scaled up it may reveal previously



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## [Table of Contents](#)

### [Index to Financial Statements](#)

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 impurities, which could result in increased scrutiny by the regulatory agencies, delays in the clinical program and regulatory approval for arhalofenate, increases in our operating expenses, or failure to obtain or maintain approval for arhalofenate.

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

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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.

### **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

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## [Table of Contents](#)

## [Index to Financial Statements](#)

- 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.

***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.;

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## [Table of Contents](#)

### [Index to Financial Statements](#)

- 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.

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

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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.

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.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

### ***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:

- 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.

## **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

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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 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

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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 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



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## [Table of Contents](#)

## [Index to Financial Statements](#)

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.

***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 “Management.” 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.

---

## [Table of Contents](#)

## [Index to Financial Statements](#)

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

As of June 30, 2014, we had 16 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.

### **Risks Relating to This Offering and 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 below the offering price of our common stock in this offering.***

Our common stock is listed on the NASDAQ Capital Market under the symbol "CBAY". 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 following this offering, and we cannot predict the extent to which the consummation of this offering or 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. The offering price for our common stock in this offering will be determined by negotiation between the representative of the underwriters and us based upon several factors, and may not be indicative of prices that will prevail in the open market after this offering. Consequently, you may be unable to sell your shares of our common stock at prices equal to or greater than the prices you paid for them, if at all.

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

The trading price of our common stock, if one develops, 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;

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## [Table of Contents](#)

### [Index to Financial Statements](#)

- 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.***

As of June 30, 2014, our executive officers, directors and stockholders who own more than 5% of our outstanding common stock, together own shares representing approximately 35.0% of our common stock. Therefore, these stockholders will have the ability to influence us through this ownership position. These stockholders may be able to influence all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to 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.

***We have recently become a public company and we will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial new time to compliance initiatives.***

We became a public company in October 2013, and as a result, we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act, as well as rules subsequently implemented by the Securities and Exchange Commission, or the SEC, and any stock market upon which we may list, have imposed various requirements on public companies. In July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as “say on pay” and proxy access. Recent legislation permits smaller “emerging growth companies” to implement many of these requirements over a longer period and up to five years from the pricing of their public offerings. We intend to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and

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## [Table of Contents](#)

## [Index to Financial Statements](#)

disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain our current levels of such coverage.

***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. If we sell common stock, convertible securities or other equity securities in more than one transaction, 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 June 30, 2014, was 325,617 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.

***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

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[Table of Contents](#)

[Index to Financial Statements](#)

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.

***If you purchase shares of common stock in this offering, you will experience immediate dilution in your investment. You will experience further dilution if we issue additional equity securities in future fundraising transactions.***

Purchasers of common stock in this offering will pay a price per share in this offering that exceeds the net tangible book value per share of our common stock. If you purchase shares of our common stock in this offering, you will experience immediate dilution of \$3.28 per share, representing the difference between the public offering price and our as adjusted net tangible book value per share as of March 31, 2014, after giving effect to this offering. See the section entitled "Dilution" below for a more detailed illustration of the dilution you would incur if you purchase common stock in this offering.

If we issue additional common stock, or securities convertible into or exchangeable or exercisable for common stock, our stockholders, including investors who purchase shares of common stock in this offering, may experience additional dilution, and any such issuances may result in downward pressure on the price of our common stock. We also cannot assure you that we will be able to sell shares or other securities in any other offering at a price per share that is equal to or greater than the price per share paid by investors in this offering, and investors purchasing shares or other securities in the future could have rights superior to existing stockholders.

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[Table of Contents](#)

[Index to Financial Statements](#)

**SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS**

This prospectus, including the sections titled “Prospectus Summary,” “Risk Factors,” “Use of Proceeds,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” “Market, Industry and Other Data,” “Business” and “Shares Eligible for Future Sale,” contains forward-looking statements. In some cases you can identify these statements by forward-looking words, such as “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “goal,” “intend,” “may,” “plan,” “potential,” “seek,” “will,” “would,” or the negative or plural of these words or similar expressions. These forward-looking statements include, but are not limited to, statements concerning the following:

- our expectations with respect to the clinical development of arhalofenate and our other product candidates, our clinical trials and the regulatory approval process;
- statements regarding the steps, timing and costs of our development programs;
- any projections of earnings, revenue, sufficiency of cash resources or other financial items;
- our expected uses of the net proceeds to us from this offering, and how long they will last;
- the plans and objectives of management for future operations;
- the availability of additional financing and access to capital;
- the formation of a trading market for our common stock;
- discussions and approvals of regulatory agencies; and
- the period of time for which we will be able to fund our operations.

These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in “Risk Factors.” Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors 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 we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this prospectus may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

You should not rely upon forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur. Moreover, except as required by law, we undertake no obligation to update publicly any forward-looking statements for any reason after the date of this prospectus to conform these statements to actual results or to changes in our expectations.

You should read this prospectus, and the documents that we reference in this prospectus and have filed with the Securities and Exchange Commission as exhibits to the registration statement of which this prospectus is a part, with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

**MARKET, INDUSTRY AND OTHER DATA**

We obtained the industry, market and other data throughout this prospectus from our own internal estimates and research, as well as from industry publications and research, surveys and studies conducted by third parties. Industry publications, studies and surveys generally state that they have been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe that each of these studies and publications is reliable, we have not independently verified market and industry data from third-party sources. While we believe our internal company research is reliable and the definitions of our market and industry are appropriate, neither this research nor these definitions have been verified by any independent source.

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[Table of Contents](#)

[Index to Financial Statements](#)

Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances that are assumed in this information. In some cases, we do not expressly refer to the sources from which this data is derived. In that regard, when we refer to one or more sources of this type of data in any paragraph, you should assume that other data of this type appearing in the same paragraph is derived from the same sources, unless otherwise expressly stated or the context otherwise requires.

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[Table of Contents](#)

[Index to Financial Statements](#)

### **USE OF PROCEEDS**

We estimate that the net proceeds from our issuance and sale of the 4,000,000 shares of our common stock in this offering will be approximately \$19.8 million, or approximately \$22.8 million if the underwriters exercise their over-allotment option in full, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

As of March 31, 2014, we had cash, cash equivalents and marketable securities of approximately \$28.5 million. We currently estimate that we will use the net proceeds from this offering, together with our cash and cash equivalents as follows:

- Approximately \$15- \$20 million on the development of MBX-8025 and ongoing development of arhalofenate; and
- the balance to fund working capital, capital expenditures and other general corporate purposes.

This expected use of the net proceeds from this offering and our existing cash and cash equivalents represents our intentions based upon our current plans and business conditions. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development and commercialization efforts and the status of and results from clinical studies, as well as any collaborations that we may enter into with third parties and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering.

Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation investments, including short-term, investment grade, interest bearing instruments and U.S. government securities.

### **MARKET PRICE OF COMMON STOCK**

Our common stock was quoted on the OTCQB Market Place under the symbol "CYMA" from January 24, 2014, to June 17, 2014, and has been listed on the NASDAQ Capital Market under the symbol "CBAY" since June 18, 2014. From January 24, 2014, to March 31, 2014, the closing price of our common stock has ranged from a high of \$9.00 to a low of \$5.00. From April 1, 2014, to June 30, 2014, the closing price of our common stock has ranged from a high of \$8.00 to a low of \$5.50. From July 1, 2014, to July 21, 2014, the closing price of our common stock has ranged from a high of \$7.78 to a low of \$5.69. As of July 21, 2014, the closing price of our common stock as reported on the NASDAQ Capital Market was \$5.97. As of June 30, 2014, there were approximately 522 holders of record of our common stock.

### **DIVIDEND POLICY**

We have never declared or paid any cash dividends to our stockholders. Our board of directors will make any future decisions regarding dividends. We currently intend to retain and use any future earnings, if any, for the development and expansion of our business and do not anticipate paying any cash dividends in the foreseeable future. Our board of directors has complete discretion on whether to pay dividends. Even if our board of directors decides to pay dividends, the form, frequency and amount will depend upon our future operations and earnings, capital requirements and surplus, general financial condition, contractual restrictions and other factors that the board of directors may deem relevant. Further, we may not pay dividends or redeem shares of our capital stock without the prior consent of the lenders pursuant to the terms of our current loan and security agreement with Silicon Valley Bank and Oxford.



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[Table of Contents](#)

[Index to Financial Statements](#)

**DILUTION**

Our net tangible book value as of March 31, 2014, was approximately \$11.4 million, or \$1.13 per share. Net tangible book value per share is determined by dividing our total tangible assets, less total liabilities, by the number of shares of our common stock outstanding as of March 31, 2014. Dilution with respect to net tangible book value per share represents the difference between the amount per share paid by purchasers of shares of common stock in this offering and the net tangible book value per share of our common stock immediately after this offering.

After giving effect to the sale of the 4,000,000 shares of our common stock in this offering, at the public offering price of \$5.50 per share, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us, our as adjusted net tangible book value as of March 31, 2014, would have been approximately \$31.2 million or \$2.22 per share. This represents an immediate increase in net tangible book value of \$1.09 per share to existing stockholders and immediate dilution of \$3.28 per share to investors purchasing our common stock in this offering. The following table illustrates this dilution on a per share basis:

Public offering price per share	\$5.50
Net tangible book value per share as of March 31, 2014	\$1.13
Increase in net tangible book value per share attributable to investors purchasing our common stock in this offering	<u>1.09</u>
As adjusted net tangible book value per share after this offering	<u>2.22</u>
Dilution per share to investors purchasing our common stock in this offering	<u>\$3.28</u>

The foregoing discussion and table do not take into account further dilution to new investors that could occur upon the exercise of the underwriters' option to purchase up to an additional 600,000 shares of our common stock within 30 days of the date of this prospectus. If the underwriters exercise in full their option to purchase 600,000 additional shares of our common stock, our net tangible book value on March 31, 2014, after giving effect to this offering, would have been approximately \$34.2 million, or approximately \$2.33 per share, representing an immediate dilution of \$3.17 per share to new investors purchasing shares of common stock in this offering.

The above discussion and table do not take into account further dilution to investors purchasing our common stock in this offering that could occur upon the exercise of outstanding options and warrants having a per share exercise price less than the public offering price per share in this offering. To the extent that outstanding options or warrants outstanding as of March 31, 2014, are exercised, or other shares are issued, investors purchasing our common stock in this offering will experience further dilution. In addition, we may choose to raise additional capital due to market conditions or strategic considerations even if we believe that we have sufficient funds for our current or future operating plans. To the extent that additional capital is raised through the sale of our common stock, including through the sale of securities convertible into or exchangeable or exercisable for common stock, the issuance of these securities could result in further dilution to our stockholders, including investors purchasing our common stock in this offering.

The number of our shares outstanding in the discussion and table above is based on 10,064,495 shares outstanding as of March 31, 2014, and excludes the following:

- 906,796 shares issuable upon the exercise of outstanding stock options at a weighted average exercise price of \$6.13 per share;
- 222,861 shares issuable upon the exercise of outstanding incentive awards at a weighted average exercise price of \$5.00 per share;
- 643,251 additional shares reserved for future issuance under our equity incentive plan, including 500,000 shares approved for future issuance by our stockholders at our annual meeting on June 3, 2014; and
- 1,848,487 shares issuable upon the exercise of warrants held by our stockholders and lenders at a weighted average exercise price of \$5.70 per share.

[Table of Contents](#)

[Index to Financial Statements](#)

**CAPITALIZATION**

The following table sets forth our cash, cash equivalents and marketable securities, and capitalization as of March 31, 2014:

- on an actual basis; and
- on an as adjusted basis to further reflect the sale by us of the 4,000,000 shares of our common stock in this offering (assuming no exercise of the underwriters' option to purchase additional shares) at the public offering price of \$5.50 per share, after deducting the underwriting discount and estimated offering expenses payable by us.

You should read this table together with the sections in this prospectus entitled "Selected Financial Data," and "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes included elsewhere in this prospectus.

	As of March 31, 2014	
	Actual	As adjusted
	(unaudited)	
	(in thousands, except share data)	
Cash, cash equivalents and marketable securities	\$ 28,533	\$ 48,290
Warrant liability	11,638	11,638
Facility loan, less current portion	4,099	4,099
Stockholders' equity:		
Preferred stock, \$0.0001 par value; 10,000,000 shares authorized, no shares issued and outstanding, actual and as adjusted	—	—
Common stock, \$0.0001 par value; 100,000,000 shares authorized, 10,064,495 shares issued and outstanding, actual; 14,064,495 shares issued and outstanding as adjusted	1	1
Additional paid-in capital	370,276	390,033
Accumulated other comprehensive income	3	3
Accumulated deficit	(358,904)	(358,904)
Total stockholders' equity	11,376	31,133
Total capitalization	\$ 27,113	\$ 46,870

The number of our shares outstanding in the table above is based on 10,064,495 shares outstanding as of March 31, 2014, and excludes the following:

- 906,796 shares issuable upon the exercise of outstanding stock options at a weighted average exercise price of \$6.13 per share;
- 222,861 shares issuable upon the exercise of outstanding incentive awards at a weighted average exercise price of \$5.00 per share;
- 643,251 additional shares reserved for future issuance under our equity incentive plan, including 500,000 shares approved for future issuance by our stockholders at our annual meeting on June 3, 2014; and
- 1,848,487 shares issuable upon the exercise of warrants held by our stockholders and lenders at a weighted average exercise price of \$5.70 per share.

[Table of Contents](#)[Index to Financial Statements](#)**SELECTED FINANCIAL DATA**

You should read the following selected financial data together with the section of this prospectus titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and our financial statements and the related notes included elsewhere in this prospectus. The statement of operations data for the years ended December 31, 2013 and 2012, and the balance sheet data as of December 31, 2013 and 2012, are derived from the audited financial statements that are included elsewhere in this prospectus. The statements of operations data for the three months ended March 31, 2014 and 2013, and the balance sheet data as of March 31, 2014, are derived from our unaudited interim financial statements included elsewhere in this prospectus. We have prepared the unaudited interim financial statements on the same basis as the audited financial statements and have included, in our opinion, all adjustments, consisting only of normal recurring adjustments, that we consider necessary for a fair presentation of the financial information set forth in those statements. Our historical results are not necessarily indicative of the results to be expected in the future.

(in thousands, except per share data)	Three Months Ended March 31,		Year Ended December 31,	
	2014	2013	2013	2012
<b>Statements of Operations Data:</b>				
Contract revenue	\$ —	\$ —	\$ —	\$ 3,050
Operating expenses:				
Research and development	2,615	1,490	4,525	9,280
General and administrative	2,500	925	4,871	4,208
Total operating expenses	5,115	2,415	9,396	13,488
Loss from operations	(5,115)	(2,415)	(9,396)	(10,438)
Other income (expense):				
Interest income	12	1	10	22
Interest expense	(184)	(212)	(822)	(841)
Other income (expense), net	(4,775)	(2)	135	2
Net loss	\$ (10,062)	\$ (2,628)	\$ (10,073)	\$ (11,255)
Net income (loss) attributable to common stockholders	\$ (10,062)	\$ (5,737)	\$ 243,994	\$ (23,899)
Net loss	(10,062)	(2,628)	(10,073)	(11,255)
Other comprehensive loss/income:				
Unrealized gains (losses) on marketable securities	1	—	2	(2)
Other comprehensive income (loss)	1	—	2	(2)
Comprehensive loss	\$ (10,061)	\$ (2,628)	\$ (10,071)	\$ (11,257)
Basic net income (loss) per common share	\$ (1.02)	\$ (985.06)	\$ 103.52	\$ (4,128.71)
Weighted average common shares outstanding used to calculate basic net income (loss) per common share	9,873,687	5,824	2,357,036	5,788
Diluted net loss per common share	\$ (1.02)	\$ (985.06)	\$ (3.54)	\$ (4,128.71)
Weighted average common shares outstanding used to calculate diluted net loss per common share	9,873,687	5,824	2,845,609	5,788

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[Table of Contents](#)

[Index to Financial Statements](#)

(in thousands)	As of March 31,	As of December 31,	
	2014	2013	2012
	(unaudited)		
<b>Balance sheet data:</b>			
Cash, cash equivalents and marketable securities	28,533	\$ 31,244	\$ 7,726
Working capital	15,412	22,751	(9,960)
Total assets	31,196	32,500	8,116
Warrant liability	11,638	6,466	—
Facility loan	4,509	4,445	—
Convertible notes	—	—	13,737
Total liabilities	19,820	13,904	17,986
Redeemable convertible preferred stock	—	—	318,697
Accumulated deficit	(358,904)	(348,842)	(329,480)
Total stockholders' equity (deficit)	11,376	18,596	(328,567)

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[Table of Contents](#)

[Index to Financial Statements](#)

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

**Forward-Looking Statements**

*Some of the statements under in this "Management's Discussion and Analysis of Financial Condition and Results of Operations" are forward-looking statements. These forward-looking statements are based on management's beliefs and assumptions and on information currently available to our management and involve significant elements of subjective judgment and analysis. Words such as "anticipate," "believe," "continue," "could," "estimate," "expect," "goal," "intend," "may," "plan," "potential," "seek," "will," "would," or the negative or plural of these words or similar expressions are intended to identify forward-looking statements. Our actual results and the timing of events may differ significantly from the results discussed in the forward-looking statements. Factors that might cause such a difference include those discussed under the caption "Special Note Regarding Forward Looking Statements" and in "Risk Factors" and elsewhere in this prospectus. These and many other factors could affect our future financial and operating results. We undertake no obligation to update any forward-looking statement to reflect events after the date of this prospectus.*

**Overview**

CymaBay Therapeutics, Inc. is focused on developing therapies to treat serious rare and orphan metabolic diseases as well as more prevalent diseases with high unmet medical need. Arhalofenate, our lead product candidate, is being developed for the treatment of gout. Arhalofenate has successfully completed three 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 arhalofenate's ability to prevent or reduce flares while also lowering sUA differentiates it from currently available treatments for gout. Arhalofenate has established a favorable safety profile in clinical trials involving nearly 1,000 patients exposed to date. We are currently investigating arhalofenate in a 12-week Phase 2b clinical trial in patients with gout and expect to report data from this trial in the first half of 2015. 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 considering pursuing MBX-8025 in a number of orphan diseases in which these attributes would be beneficial, such as homozygous familial hypercholesterolemia (HoFH), severe hypertriglyceridemia (SHTG) and primary biliary cirrhosis (PBC). We also believe that MBX-8025 would have utility in the treatment of the more prevalent, but high unmet need, indication of nonalcoholic steatohepatitis (NASH). We plan to initiate one or more proof-of-concept studies for MBX-8025 in the first half of 2015.

We have reported net losses of \$10.1 million, \$10.1 million and \$11.3 million for the three months ended March 31, 2014, and for the years ended December 31, 2013 and 2012, respectively. Our cash, cash equivalents and marketable securities balances as of March 31, 2014, and December 31, 2013, were \$28.5 million and \$31.2 million respectively. Our average monthly cash usage for the three months ended March 31, 2014, and for the year ended December 31, 2013, was approximately \$1.9 million and \$0.5 million, respectively. On September 30, 2013, 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 on September 30, 2013, resulting in aggregate net proceeds to us of \$28.8 million after deducting placement agent fees and estimated 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 of \$2.2 million after deducting placement agent fees and estimated 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 as part of the private placement for net proceeds of \$2.7 million, which sales occurred on

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## [Table of Contents](#)

## [Index to Financial Statements](#)

January 29, 2014, after the listing of our common stock on the OTCQB Market Place. 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. After giving effect to the 2013 financing, we believe that our existing cash will allow us to continue operation through the second quarter of 2015 and after giving effect to the estimated net proceeds of \$19.8 million in connection with this offering, we believe that our anticipated cash will allow us to continue operations through the end of 2015. The discussion in this registration statement gives retroactive effect to the 1 for 79.5 reverse stock split that occurred on September 30, 2013.

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 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.”

### **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. Actual results may materially differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 of our financial statements included in this prospectus, we believe the following accounting policies to be critical to the judgments and estimates used in the preparation of our financial statements.

#### ***Revenue Recognition***

Our contract revenues are generated primarily through research and development collaboration agreements, which may include nonrefundable, non-creditable upfront fees, funding for research and development efforts, and milestone or other contingent payments for achievements with regards to our licensed products. We have not materially modified any previous collaboration agreements or entered into any new agreements in 2013 or the first quarter of 2014, nor have we received any milestone payments in 2013 or the first quarter of 2014.

We recognize revenue when pervasive evidence of an arrangement exists, transfer of technology has been completed, services are performed or products have been delivered, the fee is fixed and determinable, and collection is reasonably assured.

Upfront payments for licensing our intellectual property to date have not been separable from the activity of providing research and development services because the license has not been assessed to have stand-alone value separate from the research and development services provided. Such upfront payments are recorded as deferred revenue in the balance sheet and are recognized as contract revenue over the contractual or estimated substantive performance period, which is consistent with the term of the research and development obligations contained in the research and development collaboration agreement.

Payments resulting from our research and development efforts under license agreements are recognized as the activities are performed.

Substantive, at-risk milestone payments are recognized as revenue when the milestone is achieved and collectability is reasonably assured. When contingent payments are not for substantive and at-risk milestones, revenue is recognized over the estimated remaining term of the related service period or, if there are no continuing performance obligations under the arrangement, upon receipt provided that collection is reasonably assured and other revenue recognition criteria have been satisfied.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

### ***Research and Development Expenses***

As part of the process of preparing our financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing contracts, reviewing the terms of our license agreements, communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. The majority of our service providers invoice us monthly in arrears for services performed. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. Examples of estimated accrued research and development expenses include fees to:

- contract research organizations and other service providers in connection with clinical studies;
- contract manufacturers in connection with the production of clinical trial materials; and
- vendors in connection with preclinical development activities.

We base our expenses related to clinical studies on our estimates of the services received and efforts expended pursuant to contracts with multiple research institutions and contract research organizations that conduct and manage clinical studies on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows and expense recognition. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual accordingly. Our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in our reporting changes in estimates in any particular period. Adjustments to prior period estimates have not been material for the three months ended March 31, 2014, or the years ended December 31, 2013 and 2012.

### ***Stock-Based Compensation***

We expense stock-based compensation to employees over the requisite service period based on the estimated grant-date fair value-based measurement of the awards and considering estimated forfeiture rates. For stock-based compensation awards to non-employees, we re-measure the fair value-based measurement of the non-employee awards at each reporting period prior to vesting and finally at the vesting date of the award. Changes in the estimated fair value-based measurement of these non-employee awards are recognized as compensation expense in the period of change.

Determining the appropriate fair value-based measurement of stock-based awards requires the use of subjective assumptions. In the absence of a public trading market for our common stock prior to becoming a publicly traded company, we conducted periodic assessments of the valuation of our common stock. These valuations were performed concurrently with the achievement of significant milestones, with major financing transactions or when prior valuations became stale under Section 409A of the Internal Revenue Code. The determination of the fair value-based measurement of options using an option-pricing model is affected by our estimated common stock fair value as well as assumptions regarding a number of other subjective variables. These other variables include the expected term of the options, our expected stock price volatility over the expected term of the options, stock option exercise and cancellation behaviors, risk-free interest rates, and expected dividends, which are estimated as follows:

- Fair Value of our Common Stock: Our common stock became publicly traded on the OTCQB Marketplace on January 24, 2014. After this date, we began to determine the fair value of our common stock based on closing market prices as reflected on the OTCQB Marketplace. Prior to this date, we estimated the fair value of our common stock, as discussed in “Common Stock Valuations” below.
- Expected Term: We do not believe we are currently able to rely on our historical exercise and post-vesting termination activity to provide accurate data for estimating the expected term for use in

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## [Table of Contents](#)

### [Index to Financial Statements](#)

determining the fair value-based measurement of our options. Therefore, we have opted to use the “simplified method” for estimating the expected term of options.

- **Volatility:** We have a limited trading history for our common stock, and as such, the expected stock price volatility for our common stock was estimated by taking an average weighted historic price volatility for comparable industry peers based on daily price observations over a period equivalent to the expected term of the stock option grants. Industry peers consist of several public companies in the biopharmaceutical industry similar in size, stage of life cycle and financial leverage. We did not rely on implied volatilities of traded options in our industry peers’ common stock because the volume of activity was relatively low. We intend to continue to consistently apply this process using the same or similar public companies until a sufficient amount of historical information regarding the volatility of our own common stock share price becomes available, or unless circumstances change such that the identified companies are no longer similar to us, in which case, more suitable companies whose share prices are publicly available would be utilized in the calculation.
- **Risk-free Rate:** The risk-free interest rate is based on the yields of U.S. Treasury securities with maturities similar to the expected term of the options for each option group.
- **Dividend Yield:** We have never declared or paid any cash dividends and do not presently plan to pay cash dividends in the foreseeable future. Consequently, we used an expected dividend yield of zero.

The estimation of the number of stock awards that will ultimately vest requires judgment, and to the extent actual results or updated estimates differ from our current estimates, such amounts will be recorded as a cumulative adjustment in the period in which estimates are revised. Forfeitures are estimated such that we only recognize expense for those shares expected to vest, and adjustments are made if actual forfeitures differ from those estimates.

If any of the assumptions used in a Black-Scholes model changes significantly, stock-based compensation for future awards may differ materially compared with the awards granted previously.

#### ***Common Stock Valuations***

Prior to January 24, 2014, the date our common stock first became publicly traded on the OTCQB Marketplace, the fair value of the common stock underlying our stock options and restricted stock at the date of grant was determined by our board of directors, which intended all options granted to be exercisable at a price per share not less than the per share fair value of our common stock underlying those options on the date of grant. All stock awards previously granted or to be granted in the future were or are expected to be granted at the grant date fair value of the award. The valuations of our common stock were determined in accordance with the guidelines outlined in the American Institute of Certified Public Accountants Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*. Valuation analysis of our common stock was performed on our behalf by third party valuation specialists. The methodology used by the third party valuation specialists to determine the fair value of our common stock included estimating the fair value of the enterprise, subtracting the fair value of debt from this enterprise value, and then allocating this value using the Option Pricing Method to all of the equity interests. The assumptions used in the valuation model to determine the fair value of our common stock as of the date of each option and restricted stock award, are based on numerous objective and subjective factors combined with management judgment including the following:

- progress of research and development activities;
- our operating and financial performance;
- market conditions;
- developmental milestones achieved;
- sales of our convertible preferred stock in arms-length transactions;
- business risks; and
- management and board of director experience.



[Table of Contents](#)[Index to Financial Statements](#)

We have granted stock options during the period from January 1, 2012, through March 31, 2014, as summarized below:

<u>Date of Issuance</u>	<u>Number of Shares Subject to Options Granted</u>	<u>Exercise Price per Share</u>	<u>Fair Value Estimate per Common Share</u>	<u>Estimated Total Fair Value-Based Measurement of Options Granted (In thousands)</u>
January 25, 2012	15,094	\$ 4.77	\$ 3.97	\$ 58
October 31, 2013	321,574	\$ 5.00	\$ 3.75	\$ 1,207
December 23, 2013	166,123	\$ 5.00	\$ 3.77	\$ 600
January 6, 2014	320,991	\$ 5.00	\$ 3.75	\$ 1,203
January 22, 2014	19,459	\$ 5.00	\$ 3.77	\$ 73
March 18, 2014	2,500	\$ 7.99	\$ 6.01	\$ 15

Prior to January 24, 2014, the date our common stock first began trading on the OTCQB Marketplace, management and our board of directors performed valuation analyses with the assistance of independent valuation specialists to determine the then current fair value of our common stock. To facilitate these valuation analyses, we developed projections of our future revenues and operating expenses. Key assumptions reflected in the income approach calculations included the anticipated timing of a potential liquidity event, the estimated volatility of our common stock, and the discount for lack of marketability of our common stock. These income approach assumptions are set forth below for each of the valuations performed as of December 31, 2013 and 2012:

	<u>December 31,</u>	
	<u>2013</u>	<u>2012</u>
Common Stock Value per Share	\$5.00	\$0.80
Time to Liquidity (in years)	1.25	2.0
Volatility	64.6%	94.7%
Risk-Free Interest Rate	0.02%	0.30%
Marketability Discount Rate	12.8%	49.2%

For grants of stock awards made on dates for which there was no valuation performed by an independent valuation specialist, our board of directors determined the fair value of our common stock on the date of grant based upon the immediately preceding valuation and other pertinent information available to it at the time of grant.

#### *Warrant Liabilities*

We have issued freestanding warrants to purchase shares of our common stock. Our outstanding common stock warrants issued in connection with our 2013 financing are classified as liabilities in the balance sheet as they contain terms for redemption of the underlying security that are outside our control. The fair value of all warrants is re-measured at each financial reporting date with any changes in fair value being recognized in other income (expense), net in the statements of operations and comprehensive income (loss). We will continue to re-measure the fair value of the warrant liabilities until: (i) exercise, or (ii) expiration of the related warrant.

#### *JOBS Act*

In April 2012, the JumpStart Our Business Startups Act of 2012, or the JOBS Act, was signed into law. The JOBS Act contains provisions that, among other things, reduce certain reporting requirements for an “emerging growth company.” As an “emerging growth company,” and as disclosed in our Form 10 filed with the SEC on August 12, 2013, as amended, we are electing to take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards, and as a result, we will not comply with new or revised accounting standards until adoption of such standards is required for non-emerging growth companies. Section 107 of the JOBS Act provides that our decision to take advantage of the extended transition period is irrevocable. In addition, we are in the process of evaluating the benefits of relying on the other exemptions and reduced reporting requirements provided by the JOBS Act. Subject to certain conditions set forth in the JOBS Act, if as an “emerging growth company” we choose to rely on such exemptions, we may not be

## [Table of Contents](#)

## [Index to Financial Statements](#)

required to, among other things, (i) provide an auditor's attestation report on our system of internal controls over financial reporting pursuant to Section 404, (ii) provide all of the compensation disclosure that may be required of non-emerging growth public companies under the Dodd-Frank Wall Street Reform and Consumer Protection Act, (iii) comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements (auditor discussion and analysis), and (iv) disclose certain executive compensation-related items such as the correlation between executive compensation and performance and comparisons of the Chief Executive Officer's compensation to median employee compensation. These exemptions will apply for a period of five years following the completion of our initial public offering or until we no longer meet the requirements of being an "emerging growth company," whichever is earlier.

### Results of Operations

#### *General*

To date, we have not generated any net income from operations. As of March 31, 2014, we have an accumulated deficit of \$358.9 million, primarily as a result of expenditures for research and development and general and administrative expenses. While we may in the future generate revenue from a variety of sources, including license fees and milestone payments in connection with strategic partnerships, our product candidates are at a mid-level stage of 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 sufficient revenue to achieve and sustain profitability.

#### *Research & Development Expenses*

Conducting research and development is central to our business model. For the three months ended March 31, 2014 and 2013, and for the years ended December 31, 2013 and 2012, research and development expenses were \$2.6 million, \$1.5 million, \$4.5 million and \$9.3 million, respectively. Research and development expenses are detailed in the table below:

	Three Months Ended		Year ended	
	March 31,		December 31,	
	2014	2013	2013	2012
	(unaudited)			
Arhalofenate—Phase 2b Randomized Study	\$1,486	\$ 4	\$ 461	\$ 39
Arhalofenate Gout—Drug manufacturing	122	380	9	986
Arhalofenate—Three Phase 2 Randomized Studies	(90)	9	631	2,716
MBX-8025	1	—	—	21
Other Projects	16	27	68	157
<b>Total Project Costs</b>	<u>1,535</u>	<u>420</u>	<u>1,169</u>	<u>3,919</u>
<b>Internal Research and Development Costs</b>	<u>1,080</u>	<u>1,070</u>	<u>3,356</u>	<u>5,361</u>
<b>Total Research and Development</b>	<u>\$ 2,615</u>	<u>\$1,490</u>	<u>\$4,525</u>	<u>\$9,280</u>

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.

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[Table of Contents](#)

[Index to Financial Statements](#)

We expect to continue to incur substantial expenses related to our development activities for the foreseeable future as we continue product development and initiate our next clinical study for arhalofenate. 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, rent and other general operating expenses not otherwise included in research and development. For the three months ended March 31, 2014 and 2013, and for the years ended December 31, 2013 and 2012, general and administrative expenses were \$2.5 million, \$0.9 million, \$4.9 million and \$4.2 million, respectively. We anticipate general and administrative expenses will increase in future periods, reflecting an expanding infrastructure and increased professional fees associated with being a public reporting company under the Exchange Act.

**Comparison of Three Months Ended March 31, 2014 and 2013**

	Three Months Ended March 31,		Variance
	2014	2013	
<i>(\$ in thousands)</i>			
Operating expenses:			
Research and development	\$ 2,615	\$ 1,490	\$ 1,125
General and administrative	2,500	925	1,575
Loss from operations	(5,115)	(2,415)	(2,700)
Interest expense, net	(172)	(211)	39
Other expense, net	(4,775)	(2)	(4,773)
Net loss	<u>\$(10,062)</u>	<u>\$(2,628)</u>	<u>\$(7,434)</u>

Research and development expenses increased \$1.1 million, from \$1.5 million to \$2.6 million for the three months ended March 31, 2013 and 2014, respectively. Total project costs increased by \$1.1 million during the three months ended March 31, 2014, as compared to the three months ended March 31, 2013, due to the commencement of clinical trial activities for arhalofenate, our lead product candidate. Internal research and development cost remained consistent at \$1.1 million for the three months ended March 31, 2014, and March 31, 2013, as cost reductions from the closure of our research labs in mid 2013 were offset by consulting and stock compensation expenses incurred in 2014 primarily to support the expansion of our clinical development activities for arhalofenate.

For the three months ended March 31, 2014 and 2013, general and administrative expenses were \$2.5 million, and \$0.9 million, respectively. The \$1.6 million increase for the three months ended March 31, 2014, as compared to the three months ended March 31, 2013, was due primarily to an increase in headcount related expenses, an increase in professional fees and compliance costs of \$0.5 million associated with operating as a public company, and an increase of \$0.6 million for employee and director stock-based compensation expense.

Other expense, net for the three months ended March 31, 2014, was \$4.8 million due to the remeasurement of our warrant liabilities at fair value as of March 31, 2014. We use a binomial lattice option pricing model to value our warrants and the valuation increase was due primarily to an increase in the value of our common stock from \$5 at December 31, 2013, to \$8 at March 31, 2014, which is an input to the valuation model. No warrants subject to liability accounting were outstanding during the three months ended March 31, 2013.

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[Table of Contents](#)

[Index to Financial Statements](#)

*Comparison of Years Ended December 31, 2013 and 2012*

(\$ in thousands)	For the Year Ended		Variance
	December 31,		
	2013	2012	
Contract revenue	\$ —	\$ 3,050	\$(3,050)
Operating expenses:			
Research and development	4,525	9,280	(4,755)
General and administrative	4,871	4,208	663
Loss from operations	(9,396)	(10,438)	1,042
Interest income (expense), net	(812)	(819)	7
Other income, net	135	2	133
Net loss	<u>\$(10,073)</u>	<u>\$(11,255)</u>	<u>\$ 1,182</u>

Contract revenue as of December 31, 2012, was related to specific research and development funding with Takeda San Francisco, Inc. (“Takeda”) of \$0.1 million and a final contract revenue payment of \$2.9 million from Sanoif-Aventis. There was no contract revenue as of December 31, 2013, since all revenue contracts were terminated in 2012.

Research and development expenses decreased \$4.8 million, from \$9.3 million to \$4.5 million for the year ended December 31, 2012 and 2013, respectively. Total project costs decreased by \$2.8 million for the year ended December 31, 2013, as compared to December 30, 2012, due to a hold placed on all projects until financing could be obtained. Internal research and development cost decreased by \$2.0 million for year ended December 30, 2013, as compared to December 31, 2012, due to cost cutting measures in 2013. There was an involuntary reduction in the research and development workforce at the end of May 2013 and a subsequent shutdown of the labs from June to September 2013 due to the company’s decision to place its primary focus on fundraising.

General and administrative expenses consist principally of personnel-related costs, professional fees for legal, consulting, audit services, rent and other general operating expenses not otherwise included in research and development. General and administrative expenses increased by \$0.7 million from \$4.2 million for the year ended December 31, 2012, to \$4.9 million for the year ended December 31, 2013, primarily due to a \$0.6 million increase in stock compensation expense.

Other income, net increased by approximately \$0.1 million for the year ended December 31, 2013, compared to the year ended December 31, 2012, due primarily to a \$0.6 million gain on the sale of lab equipment and furniture and fixtures which was partially offset by a \$0.5 million increase in the fair value of our warrant liability.

**Income Taxes**

As of December 31, 2013, we had federal and state net operating loss carryforwards of approximately \$152.1 million and \$152.2 million, respectively, to offset future taxable income, if any. In addition, we had federal and state research and development tax credit carry forwards of approximately \$6.2 million and \$ 3.2 million, respectively. If not utilized, the federal net operating loss and tax credit carryforwards will expire beginning in 2024 through 2033 and the state net operating loss carryforwards will expire beginning in 2014 through 2033. The state tax credit will carry forward indefinitely. Current federal and state tax laws include substantial restrictions on the utilization of net operating losses and tax credits in the event of an ownership change. Even if the carryforwards are available, they may be subject to annual limitations, lack of future taxable income, or future ownership changes that could result in the expiration of the carryforwards before they are utilized. At December 31, 2013, we recorded a 100% valuation allowance against our deferred assets of approximately \$90.8 million as our management believes it is uncertain that they will be fully realized. If we determine in the future that we will be able to realize all or a portion of our net operating loss carryforwards, an adjustment to our net operating loss carryforwards would increase net income in the period in which we make such a determination.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

### **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, 2014, we had cash and cash equivalents of \$22.3 million and marketable securities of \$6.2 million. At December 31, 2013, we had cash and cash equivalents of \$24.4 million and marketable securities of \$6.8 million. As stated above under “Reverse Stock Split and Conversion of Preferred Stock,” we initiated a series of transactions we refer to as our 2013 financing. Specifically, on September 30, 2013, we issued common stock and warrants to purchase our common stock and we secured a term loan facility which together enabled us to raise aggregate net proceeds of \$28.8 million. In addition, on September 30, 2013, we issued common stock in cancellation of \$16.9 million of debt owed to the holder of that debt, and on October 31, 2013, we issued common stock and warrants to purchase our common stock to raise additional net proceeds of \$2.2 million. Furthermore, on November 22, 2013, we entered into an agreement with investors to purchase shares of our common stock and warrants to purchase our common stock as part of the private placement for net proceeds of \$2.7 million, which sales occurred shortly after our listing of our common stock on the OTCQB Marketplace on January 24, 2014.

As part of the 2013 financing, we entered into a term loan facility with Silicon Valley Bank and Oxford Finance LLC, collectively referred to as the lenders, for an aggregate amount of \$10.0 million. Of this total amount, \$5.0 million was made available to us as of September 30, 2013, and the remaining \$5.0 million, which we refer to as the second tranche, shall be made available to us upon the achievement of positive data and successful completion of all primary endpoints for either the 600mg or 800mg dose of arhalofenate in our current Phase 2b study (the “second draw milestone”). The second tranche shall be available to us until the earlier of 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.

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, the term loan facility contains financial covenants that require us to maintain a certain cash liquidity. The term loan facility also contains performance covenants that require that: (a) within one hundred twenty (120) days of us becoming eligible to file a registration statement with the United States Securities and Exchange Commission on Form S-3, we must have access to an At The Market facility; and (b) by no later than March 31, 2015, the lenders must have received evidence of the occurrence of the second draw milestone; provided that our failure to comply with these performance covenants shall not be an event of

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## [Table of Contents](#)

## [Index to Financial Statements](#)

default under the term loan facility so long as we deposit an amount equal to 100% of the aggregate outstanding term loans in a segregated, blocked deposit account at Silicon Valley Bank.

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.

### 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,		Years Ended December 31,	
	2014	2013	2013	2012
Net cash used in operating activities	\$(5,011)	\$ (2,159)	\$ (8,458)	\$ (11,293)
Net cash provided by (used in) investing activities	550	—	(6,231)	11,010
Net cash provided by (used in) financing activities	2,402	—	31,364	(12)
Net (decrease) increase in cash and cash equivalents	\$(2,059)	\$ (2,159)	\$ 16,675	\$ (295)

*Operating Activities:* Net cash used in operating activities for the three months ended March 31, 2014 was \$5.0 million and was primarily due to a net loss of \$10.1 million which resulted from the planned commencement of clinical trial activities for arhaolfenate, our lead product candidate. This net loss was partially offset by a \$4.8 million noncash adjustment incurred to revalue our warrant liability. Cash used in operating activities for the years ended December 31, 2013 and December 31, 2012 was \$8.5 million and \$11.3 million, respectively. The decrease of \$2.8 million in cash used in operating activities is due primarily to operating cost containment measures taken throughout 2013 until the 2013 financing occurred.

*Investing Activities:* Net cash provided by investing activities was \$0.5 million for the three months ended March 31, 2014, and was primarily due to the sale of marketable securities. No investing activities occurred during the three months ended March 31, 2013. Net cash used in investing activities was \$6.2 million for the year ended December 31, 2013, and was primarily due to the purchase of marketable securities as we sought to invest funds raised in the 2013 financing. Net cash provided by investing activities was \$11.0 million for the year ended December 31, 2012, and was due primarily to proceeds received from sales of marketable securities.

*Financing Activities:* Cash provided by financing activities was \$2.4 million for the three months ended March 31, 2014, primarily as a result of the consummation of an agreement with investors to purchase shares of our common stock and warrants to purchase shares of our common stock in January 2014 as part of the 2013 financing. Net cash provided by financing activities increased by \$31.4 million in the year ended December 31, 2013, of which \$26.5 million was due to proceeds received from the sale of equity securities and \$4.9 million which was due to proceeds received from our new facility loan.

Management believes that cash and cash equivalents as of March 31, 2014, including the funds raised in the 2013 financing, are sufficient to sustain our operations through the second quarter of 2015. We expect to incur substantial expenditures in the foreseeable future for the development and potential commercialization of our product candidates. We will continue to require additional financing to develop our products and fund operating losses. We will seek funds through equity financings, debt, collaborative or other arrangements with corporate sources, or through other sources of financing, including a public offering. Adequate additional funding may not be available to us on acceptable 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 close our business.

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[Table of Contents](#)

[Index to Financial Statements](#)

**Off Balance Sheet Arrangements**

As of March 31, 2014, and December 31, 2013, we had no off-balance sheet arrangements (as defined in Item 303(a)(4)(ii) of Regulation S-K under the Exchange Act) that create potential material risks for us and that are not recognized on our balance sheets.

**Contractual Obligations**

The following table summarizes our long-term contractual obligations as of December 31, 2013 (in thousands):

(in thousands)	Payments Due by Period			
	Total	Less than 1 Year	1-3 Years	3-5 Years
<b>Contractual Obligations</b>				
Operating lease obligations	\$1,212	\$ 337	\$ 647	\$228
Facility term loan, including interest	<u>6,392</u>	<u>681</u>	<u>5,711</u>	<u>—</u>
Contractual Commitments	<u>\$7,604</u>	<u>\$1,018</u>	<u>\$6,358</u>	<u>\$228</u>

There were no material changes to our contractual obligations in the first quarter of 2014.

## BUSINESS

### CymaBay Overview

CymaBay Therapeutics, Inc. is focused on developing therapies to treat serious rare and orphan metabolic diseases as well as more prevalent diseases with high unmet medical need. Arhalofenate, our lead product candidate, is being developed for the treatment of gout. Arhalofenate has successfully completed three 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 arhalofenate's ability to prevent or reduce flares while also lowering sUA differentiates it from currently available treatments for gout. Arhalofenate has established a favorable safety profile in clinical trials involving nearly 1,000 patients exposed to date. We are currently investigating arhalofenate in a 12-week Phase 2b clinical trial in patients with gout and expect to report data from this trial in the first half of 2015. 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 considering pursuing MBX-8025 in a number of orphan diseases in which these attributes would be beneficial, such as homozygous familial hypercholesterolemia (HoFH), severe hypertriglyceridemia (SHTG) and primary biliary cirrhosis (PBC). We also believe that MBX-8025 would have utility in the treatment of the more prevalent, but high unmet need, indication of nonalcoholic steatohepatitis (NASH). We plan to initiate one or more proof-of-concept studies for MBX-8025 in the first half of 2015.

We believe arhalofenate has the potential to address unmet needs in the treatment of gout. Of the eight million patients with gout in the U.S., we estimate that over three million are on urate lowering therapy (ULT). Approximately one million of these patients on ULT continue to experience three or more flares per year, with significant impact to patient quality of life and the health care system. This patient population is poorly served by available therapies. The two primary goals of gout treatment are the prevention of flares and lowering of sUA. The fundamental limitation in achieving these goals is that all currently available ULTs cause an increase in flares upon initiation of treatment, leading many patients to discontinue or avoid therapy. Given this increase in flares, standard of care includes prophylaxis with colchicine and use of anti-inflammatory medications, which are often poorly tolerated or inadvisable for use in gout patients due to their side effects. Despite prophylaxis with colchicine, many patients continue to experience flares. We believe that by decreasing flares while lowering sUA, arhalofenate has the potential to treat patients with gout without the need for colchicine or other anti-inflammatory medications and would thus be differentiated from all currently available gout therapies.

### CymaBay Strategy

Our goal is to become a leading biopharmaceutical company focused on developing and commercializing proprietary new medicines for metabolic and rare diseases with high unmet need. Key elements of our strategy are to:

- develop arhalofenate as a dual-acting treatment to prevent or reduce flares and lower sUA in patients with gout;
- develop MBX-8025 for high unmet need or orphan indications linked to defects in lipid storage, handling and utilization and certain diseases effecting liver function;
- pursue partnerships to advance and commercialize arhalofenate and potentially other clinical candidates; and
- strengthen our patent portfolio and other means of protecting exclusivity.

### CymaBay Pipeline Overview

Our pipeline includes three unpartnered clinical stage product candidates and a number of preclinical programs.



[Table of Contents](#)

[Index to Financial Statements](#)

Program	Indication	Partner	Research	Preclinical	P1	P2
Arhalofenate	Gout					
MBX-8025	Orphan Disease					
MBX-2982	Diabetes					
Target	Diabetes	Johnson & Johnson				
Targets	Diabetes	Johnson & Johnson				

**Arhalofenate—Gout**

Gouty arthritis, or simply gout, is the most common form of inflammatory arthritis in men and affects more than eight million people in the United States (U.S.). The hallmark symptom of gout is a flare, characterized by debilitating pain, along with tenderness and inflammation of affected joints. Gout has a significant impact on patients’ quality of life and health care utilization. Patients experiencing gout flares miss an average of 4.6 more days of work per year than those without gout. Gout flares also result in increased health care utilization with approximately 35% of moderate and 50% of severe gout patients who experience a flare having at least one acute care visit per year.

Gout flares are recurring and painful episodes of joint inflammation that are triggered by the presence of monosodium urate (MSU) crystals. MSU crystals are formed in tissues when the concentration of serum uric acid (sUA) exceeds its solubility limit of approximately 6.8 milligrams per deciliter (mg/dL). Elevated levels of sUA, or hyperuricemia, most commonly results from the under excretion of uric acid in the kidney. This is caused by its reabsorption from urine and transport back to the blood by specialized urate transporters/exchangers in the proximal renal tubule. Long term accumulation of MSU crystals in the body leads to the progression of gout with an increase in the frequency of flares, the involvement of multiple joints, the formation of visible masses of MSU crystals (tophi) and the debilitation that results from deformation of joints.

Many scientific surveys and large clinical studies in gout indicate that gout patients have a high incidence of cardiovascular and metabolic comorbidities, such as hypertension (50% or more), coronary artery disease (>35%), chronic kidney disease (~40%), and diabetes (~20%). Managing patients with these comorbidities is challenging because many of them are contraindicated in the medication currently used to treat gout. Examples include corticosteroids which can cause hypertension and worsening of dysglycemia and non-steroidal anti-inflammatory drugs (NSAIDs) which have renal toxicity.

**Market Opportunity**

*Unmet Needs in the Treatment of Gout*

Of the eight million patients with gout in the U.S., we estimate that over three million are on urate lowering therapy (ULT) and of these patients on ULTs, about one million will continue to experience three or more flares per year, with significant impact to patient quality of life and the health care system. According to a 2012 study, patients having three or more flares per year typically incur \$10,000 more in annual health care costs than patients without gout. In order to halt the progression of the disease and provide long term reduction in flares, MSU crystals must be eliminated from the body. Therefore, the two major goals of gout treatment are to prevent flares and lower sUA to below 6 mg/dL in order to dissolve MSU crystals present in tissue. The most important limitation in achieving these goals is that all existing ULTs paradoxically cause an increase in flares upon initiation of treatment, leading many patients to discontinue or avoid therapy. Non-adherence to therapy is a significant problem. In one long term study, only about 40% of allopurinol patients reached the goal of sUA < 6

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## [Table of Contents](#)

## [Index to Financial Statements](#)

mg/dL (Febuxostat Briefing Package FDA Advisory Committee Meeting November 24, 2008). Failure to get to goal results in progression of the disease and continued flaring.

### ***Limitations of Current Therapies***

Allopurinol and febuxostat (marketed by Takeda Pharmaceutical Company Limited as Uloric®), the most common drugs prescribed to lower sUA, increase flares for up to 6 – 12 months following initiation of treatment. The ULT-initiated flare phenomenon is common to marketed ULTs and leads to increased health care utilization and high patient discontinuation with progression of disease.

To address the increase in flare rate associated with initiation of ULT therapy, anti-inflammatory drugs such as colchicine and NSAIDs are co-prescribed with ULTs. However, use of these agents carries a risk for causing adverse effects. Some known adverse effects of colchicine include diarrhea, nausea, vomiting, destruction of skeletal muscle, neuromuscular toxicity, and decreased blood cell production. Chronic use of NSAIDs, which only provide symptom relief, is associated with increased risk of renal toxicity, gastrointestinal (GI) bleeding and cardiovascular events. Similarly, steroids are linked to hypertension and a worsening of blood glucose, which is problematic for diabetics and patients with hypertension and/or heart disease, respectively. Given the prevalence of cardiovascular and metabolic comorbidities in gout patients, the use of these agents can be problematic in a significant number of gout patients.

### ***Anti-Flare Competition***

The largest selling branded gout drug in the U.S. is Colcrys® (branded colchicine), marketed by Takeda for the prevention and treatment of gout flares. Despite the availability of low cost generic NSAIDs and steroids, Colcrys had total U.S. sales of approximately \$629 million in 2013 per IMS Health data highlighting the importance of preventing and treating gout flares effectively. While colchicine has been shown to reduce the percentage of patients experiencing flares by 57%, it carries limitations in terms of safety and tolerability.

The biologic drugs Ilaris (developed by Novartis) and Arcalyst (developed by Regeneron) which neutralize the proinflammatory cytokine IL-1 $\beta$ , the trigger for flares, have been shown in clinical trials to suppress gout flares. However, there are safety risks associated with these drugs, and neither drug has gained approval in the U.S. for gout.

### ***Serum Uric Acid Lowering Competition***

Xanthine oxidase (XO) inhibitors, allopurinol and febuxostat, dominate the ULT market with generic allopurinol at doses up to 300 mg accounting for about 90% of ULT prescriptions in the U.S. Allopurinol may potentially lead to undertreatment because of the occurrence of skin rash and a rare but serious hypersensitivity reaction which can be fatal. In addition, it must be used with caution in renally impaired patients, a common comorbidity in gout, and is recommended to undergo dose escalation. Febuxostat, approved by the Food and Drug Administration (FDA) in 2009, was the first new treatment approved for gout in more than 40 years.

Lesinurad is a drug in Phase 3 development by AstraZeneca PLC. Like arhalofenate, it lowers sUA by promoting the excretion of uric acid by the kidney. However, lesinurad, like all other ULTs, has been shown to increase flares upon initiation of treatment. Lesinurad is being studied as an add-on treatment to allopurinol patients not reaching target sUA levels, as an add-on to febuxostat in tophaceous gout patients and as monotherapy (given as a single drug) for patients who are intolerant to XO inhibitors.

While medically important, we believe the case for sUA lowering alone is not sufficient to ensure success in the market because hyperuricemia is asymptomatic and patients usually seek treatment for their flares.

### ***Arhalofenate Addresses the Unmet Needs in Gout***

We believe that a significant opportunity exists for arhalofenate as a result of its combined anti-flare and sUA lowering profile for the treatment of gout. Arhalofenate has the potential to address key unmet needs by

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## [Table of Contents](#)

## [Index to Financial Statements](#)

preventing flares and achieving sUA target goals as monotherapy. In patients who need additional sUA lowering, arhalofenate may be combined with other ULTs to significantly reduce sUA without the induction of flares seen with other ULTs.

We have undertaken an analysis of the gout market expected at the time of arhalofenate's launch. Arhalofenate has dual pharmacology, whereas other gout drugs on the market or in development, are limited to only either anti-flare or sUA lowering. Given arhalofenate has demonstrated the ability in our Phase 2 studies to reduce and prevent flares while also lowering sUA, we believe it has the potential to be the preferred alternative for the approximately 1 million patients who flare three or more times per year despite being on ULT. We believe the poor compliance of patients treated with existing ULTs also leads to more than one million discontinuations and restarts of therapy every year. The cycling of patients on and off ULTs would offer opportunities for physicians to switch patients on other therapies to arhalofenate.

As a monotherapy, we believe arhalofenate has the potential to be a single, safe, easy-to-use replacement for the combination of allopurinol and colchicine, which is the current standard of care.

For those patients needing additional sUA reduction, our clinical trial data have demonstrated that arhalofenate has the potential to be combined with febuxostat to provide large (~60%) reductions in sUA, but without the large increases in the incidence of flares seen with all other ULTs.

### **Arhalofenate Overview**

#### *Scientific Rationale*

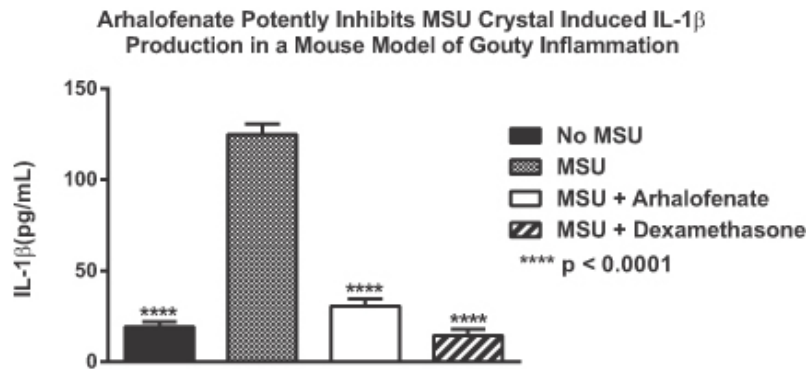
Arhalofenate is a prodrug which upon absorption is converted to its active form, arhalofenate acid. Arhalofenate acid's dual actions are to block the MSU crystal-stimulated production of IL-1 $\beta$  by macrophages (white blood cells that play an important role in the body's defense against pathogens and foreign matter) in joints and to inhibit uric acid reabsorption by urate transporters in the kidney.

#### *Anti-Inflammatory Activity*

We believe, arhalofenate (through arhalofenate acid) is unique among available anti-inflammatory drugs because it prevents the initiation of the inflammatory cascade and acts upstream from other therapies used for the prophylaxis and treatment of gout flares. The anti-inflammatory action comes from a unique trans-repression (a type of inhibition) of peroxisome proliferator-activated receptor-gamma (PPAR $\gamma$ ) which blocks the production of IL-1 $\beta$  and other inflammatory proteins by macrophages that produce a flare. Neutralization of IL-1 $\beta$  has been shown in clinical trials to reduce flares by about 70%. Because arhalofenate acid acts upstream of colchicine, it may be able to replace colchicine.

The anti-inflammatory mechanism of arhalofenate acid has been demonstrated in preclinical models. In experiments with isolated macrophages, arhalofenate acid is able to suppress MSU crystal-stimulated release of IL-1 $\beta$  protein by blocking expression of the precursor pro-IL-1 $\beta$  gene. Importantly, this activity is seen at concentrations that are achieved in humans.

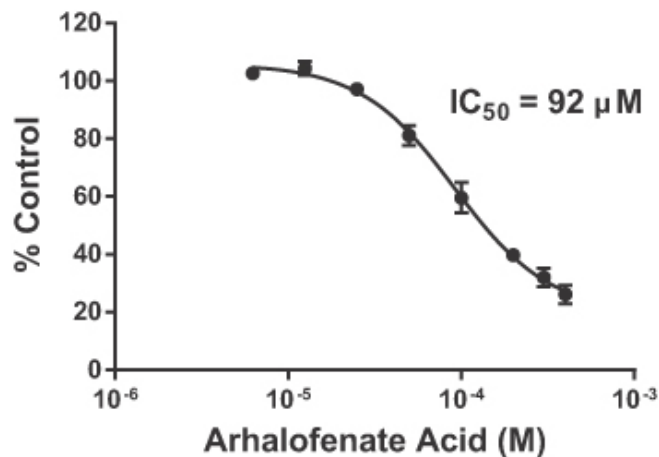
*In vivo* confirmation of this effect was seen in a mouse model of gouty inflammation. Injecting MSU crystals into mice produces many of the molecular and cellular steps involved in a gout flare. As shown below, administration of arhalofenate at doses that produce clinically relevant exposures was able to suppress the release of IL-1 $\beta$  in response to MSU crystals to a degree similar to that of dexamethasone, a potent anti-inflammatory steroid drug. Importantly, it also suppresses other important inflammatory mediators, such as CXCL1, CXCL2 and MCP-1 (chemokine (C-X-C motif) ligand 1 and ligand 2 and monocyte chemoattractant protein 1), that colchicine does not.



#### Uric Acid Lowering Activity

Uric acid is an anionic, or negatively charged, molecule that is removed from the body by filtration through the kidney into urine. For about 80-90% of patients, hyperuricemia is a result of under excretion of uric acid due to its reabsorption by organic anion transporters (OAT) in the proximal renal tubule. Arhalofenate acid blocks <sup>14</sup>C-uric acid uptake in an embryonic kidney cell line that expresses human urate transporter 1 (URAT1), one of the predominant renal transporters of urate. The inhibition is pharmacologically relevant because it occurs at concentrations that are less than those seen in human urine in clinical trials. Arhalofenate acid was shown to inhibit uric acid uptake by URAT1, OAT4 and OAT10, three of the transporters that play a critical role in uric acid reabsorption. This mechanism is consistent with the clinical pharmacology in which arhalofenate was shown to dose-dependently increase urate clearance into urine in gout patients.

#### Arhalofenate Acid Blocks <sup>14</sup>C Uric Acid Uptake by URAT1 in Human Kidney Cells



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[Table of Contents](#)

[Index to Financial Statements](#)

The available preclinical evidence provides an explanation for the dual mode-of-action observed for arhalofenate in treating gout patients. CymaBay has completed three clinical studies in gout patients which have shown that arhalofenate has the potential for both decreasing the incidence, severity and duration of gout flares, including those that often occur upon initiation of ULT, and reducing sUA.

CymaBay has completed a nonclinical program for arhalofenate, including genotoxicity, chronic repeat dose toxicology in rats and monkeys, safety pharmacology, reproductive toxicology and two-year rodent carcinogenicity studies. The results of these studies have all been submitted to and received by the FDA.

CymaBay has developed a manufacturing process for arhalofenate and ~200 kg of drug substance is available to initiate the Phase 3 program. Tablets for the Phase 2b study have already been manufactured. Both the drug substance and tablet manufacturing processes will be scaled up to support the registration and commercial chemistry, manufacturing and controls program.

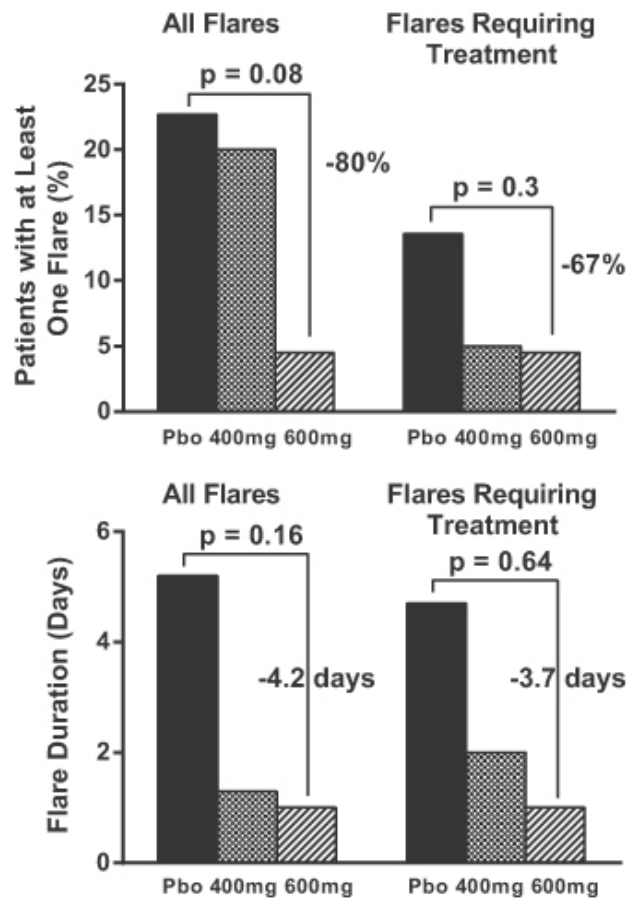
**Clinical Studies with Arhalofenate**

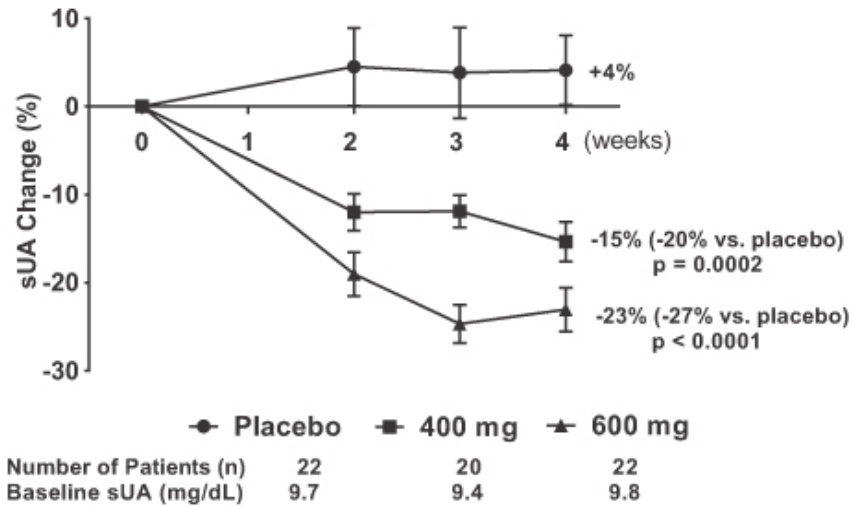
***The Gout Development Program***

Arhalofenate has been studied in three Phase 2 gout clinical trials including a monotherapy study, febuxostat combination study and an allopurinol combination study.

**Monotherapy Study**

The monotherapy study was a randomized, double-blind, placebo-controlled study evaluating the safety and efficacy of arhalofenate for the treatment of hyperuricemia in patients with gout. Arhalofenate was given daily at doses of 400 mg and 600 mg for four weeks. A total of 64 patients completed the treatment phase: 22 received placebo, 20 received arhalofenate 400 mg, and 22 received arhalofenate 600 mg. All randomized patients also received colchicine 0.6 mg daily as flare prophylaxis, a preventive treatment for flares. Compared to placebo, patients treated with arhalofenate demonstrated dose-dependent reductions in gout flare and sUA, as shown below. The proportion of patients reporting at least one flare during the treatment phase was 23% (5 of 22), 20% (4 of 20), and 5% (1 of 22) in the placebo, 400 mg, and 600 mg groups, respectively. In addition to flare frequency, both severity and duration of flare were lower in arhalofenate-treated patients. After 4 weeks of treatment, the mean sUA percent (and absolute) changes from Day 1 were: +4% (+0.2 mg/dL) in the placebo group, -15% (-1.4 mg/dL) in the 400 mg arhalofenate group and -23% (-2.3 mg/dL) in the 600 mg arhalofenate group. When compared to placebo, the sUA reductions in both arhalofenate treatment groups were statistically significant ( $p \leq 0.0002$ ).



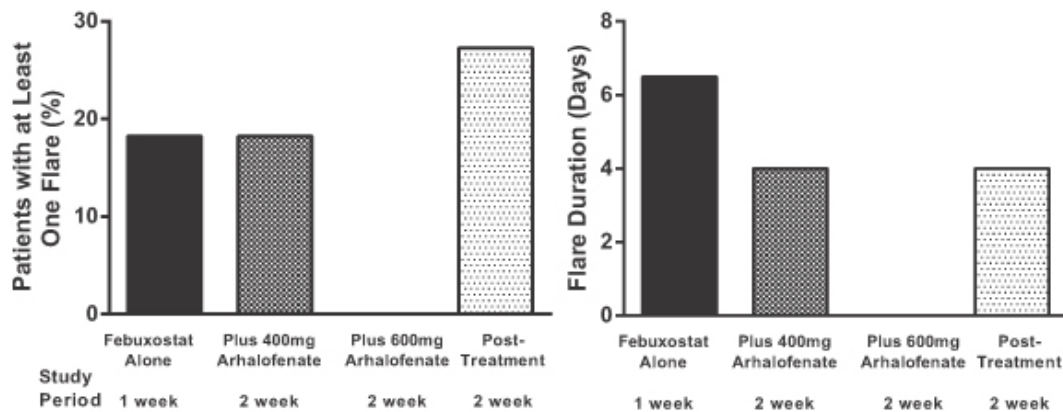


Overall, adverse events (AEs) were similar among the placebo and arhalofenate-treated groups. There were no severe or serious AEs, discontinuations due to AEs, or deaths during the study. Overall, the types and frequencies of AEs were similar among patients receiving placebo or arhalofenate 400 mg or 600 mg and there were no clinically meaningful differences observed in safety laboratory test results.

**Febuxostat Combination Study**

In the febuxostat combination study, arhalofenate up to 600 mg daily was added to febuxostat 80 mg in an open-label, in-patient study to determine the efficacy, safety, and tolerability of arhalofenate in combination with 80 mg febuxostat once daily. A total of 11 patients were dosed with 80 mg febuxostat during Week 1, 80 mg febuxostat plus 400 mg arhalofenate during Weeks 2-3 and 80 mg febuxostat plus 600 mg arhalofenate during Weeks 4-5. All patients also received 0.6 mg colchicine daily as prophylaxis for gout flare.

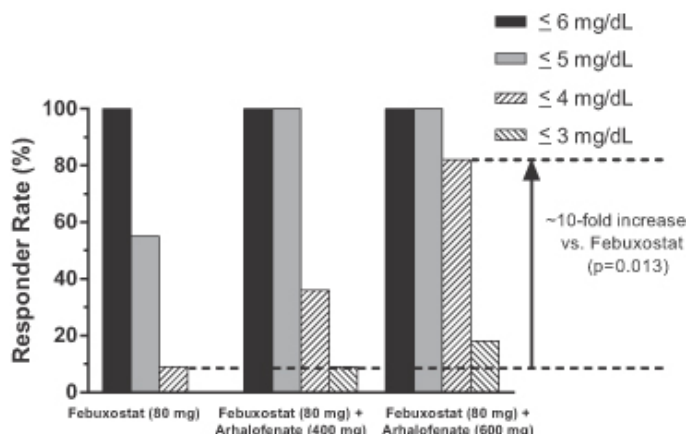
The proportion of these patients reporting at least one flare was 18% (2 of 11 patients) during Week 1 (febuxostat 80 mg) and 18% (2 of 11 patients) during Weeks 2-3 (febuxostat 80 mg plus arhalofenate 400 mg), respectively. No patient reported the initiation of a flare during Weeks 4-5 (febuxostat 80 mg plus arhalofenate 600 mg). The proportion of patients reporting at least one flare in the two-week follow-up period was 27% (3 of 11 patients).



[Table of Contents](#)

[Index to Financial Statements](#)

Mean sUA reductions were -48% at Day 8 (febuxostat 80 mg), -54% at Day 22 (febuxostat 80 mg plus arhalofenate 400 mg), and -60% at Day 36 (febuxostat 80 mg plus arhalofenate 600 mg). Historically, one week of dosing with febuxostat 80 mg has been shown to give the full effect of sUA reduction, and the mean reductions in this study at Day 8 are consistent with other reported study results. The proportion of patients who achieved various sUA target levels during treatment is shown below. Patients with advanced gout have large stores of MSU crystals in the body, and driving sUA levels to lower values (e.g., < 4 mg/dL) has been shown with other ULTs to accelerate clinical benefits such as the reduction of tophi (masses of MSU crystals).



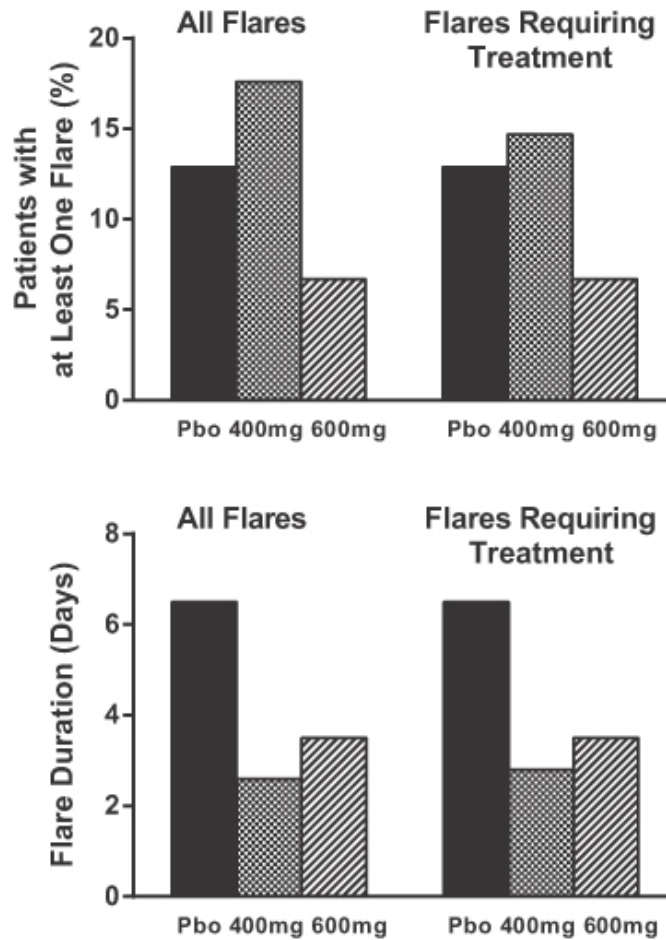
No patients experienced severe or serious AEs or deaths, and there were no discontinuations because of AEs. No clinically meaningful differences were observed among the study treatments in safety laboratory test results.

**Allopurinol Combination Study**

This study was a randomized, double-blind, placebo-controlled clinical trial designed to evaluate the efficacy, safety and tolerability of arhalofenate 400 mg and 600 mg when given in combination with allopurinol 300 mg and also to evaluate the effect of arhalofenate on the pharmacokinetics (PK, drug levels in the blood) of allopurinol and oxypurinol, (the product of metabolism or active metabolite of allopurinol) that forms in the body after ingestion of allopurinol. Arhalofenate (or placebo) was given once daily at doses of 400 mg and 600 mg, in addition to allopurinol 300 mg, for four weeks to patients who had failed to reach the sUA target of <6 mg/dL with allopurinol 300 mg. All randomized patients also received colchicine 0.6 mg daily as flare prophylaxis. A reduction in gout flares was observed in the arhalofenate 600 mg plus allopurinol group compared to the allopurinol only group. The proportion of patients in a pre-specified per protocol population reporting at least one flare during the 4-week treatment phase was 13% (4 of 31) in the allopurinol 300 mg only group, 18% (6 of 34) in the allopurinol 300 mg plus arhalofenate 400 mg group, and 7% (2 of 30) in the allopurinol 300 mg plus arhalofenate 600 mg group. The mean duration of flares was longer in the allopurinol plus placebo group (6.5 days) than in either the allopurinol plus 400 mg arhalofenate group (2.6 days) or the allopurinol plus 600 mg arhalofenate group (3.5 days).

There was no statistically significant difference in sUA reduction in the arhalofenate plus allopurinol groups compared to the allopurinol only group. In the per protocol population, the proportion of patients who reached a sUA target of <6 mg/dL at the end of the treatment phase was 35.5%, 52.9%, and 43.3% in the allopurinol plus placebo group, the allopurinol plus 400 mg arhalofenate group, and the allopurinol plus 600 mg arhalofenate group, respectively. The modest additional sUA reduction observed in the arhalofenate plus allopurinol groups in this study is attributable to an interaction in which arhalofenate reduces the concentration of oxypurinol, the active metabolite of allopurinol. Specifically, arhalofenate promotes the excretion of uric acid as well as oxypurinol given both are typically reabsorbed into the blood stream through the same renal transporters arhalofenate is responsible for blocking.





No severe or serious AEs were reported. Two patients discontinued from the study due to moderate AEs. Overall, the types and frequencies of AEs were similar among the treatment groups and there were no clinically meaningful differences observed among the study treatments in safety laboratory test results.

**Prior Clinical Experience with Arhalofenate**

Prior to the Phase 2 trials in gout described above, eight Phase 1 studies and four Phase 2 studies in patients with type 2 diabetes mellitus (T2DM) were conducted with arhalofenate. In these studies a total of 873 subjects were studied. Daily treatment with arhalofenate up to 600 mg for up to 24 weeks in T2DM patients was found to be safe and well tolerated. Prior to conducting the third and fourth Phase 2 clinical studies in patients with T2DM, we entered into an exclusive licensing agreement for arhalofenate with Ortho-McNeil in June 2006.

In these T2DM studies, daily treatment with arhalofenate with doses up to 600 mg for up to 24 weeks duration showed improvements in glucose parameters (hemoglobin A1c [HbA1c] and fasting plasma glucose), as well as a lowering of serum triglycerides in patients with elevated levels at baseline. However, given that the observed reductions in HbA1c and fasting plasma glucose were inferior for patients receiving arhalofenate versus for those receiving the comparator drug, Actos™, arhalofenate’s development for diabetes was abandoned. Ortho-McNeil terminated the license in March 2010 and has no further rights to arhalofenate. Arhalofenate was found to be well tolerated with no meaningful treatment group differences in AEs including those of special interest (edema, weight gain, and upper GI AEs), discontinuation due to AEs, serious AEs, and death. There were no reports of urinary tract stones in any of these studies. No clinically meaningful differences were observed in

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## [Table of Contents](#)

## [Index to Financial Statements](#)

safety laboratory test results including LFTs and serum creatinine values between placebo and arhalofenate-treated groups. Patients with LFT increase did not demonstrate any increase in serum bilirubin; therefore, no patient met the criteria of Hy's law of drug induced liver injury.

A pooled analysis of sUA data from these diabetes studies showed statistically significant dose dependent reductions from baseline in mean sUA with arhalofenate: +2% in the placebo group (n=252), -11% in the 200 mg group (n=125), -20% in the 400 mg group (n=174), and -27% in the 600 mg group (n=159);  $p < 0.0001$  for each active group vs. placebo comparison. A p-value is a statistical measure of the probability that the difference in two values could have occurred by chance. The smaller the p-value the greater the confidence that the results are significant. For example, in the preceding studies, there is less than a 0.01% probability that the difference between two values is due to chance and, conversely there is a 99.99% probability that the observed difference was not due to chance. Similar sUA reduction was observed in patients with mild to moderate renal impairment and without additional worsening of renal function. Comparable sUA reduction was also achieved with arhalofenate in patients on concomitant low-dose aspirin (up to 325 mg daily) and on diuretics (blood pressure lowering agents).

### *Conclusions of Arhalofenate's Clinical Experience*

Arhalofenate has been studied in a total of 15 clinical trials with nearly a thousand subjects. These include Phase 1 studies of safety, tolerability and PK, Phase 2 studies of blood glucose effects in diabetics, and Phase 2 studies of sUA and flare effects in gout patients. Arhalofenate has had a consistent pattern of good safety and tolerability. Despite having differing objectives across these studies, arhalofenate demonstrated comparable dose-dependent reductions in sUA.

In addition to its primary characteristics for reduction of flare incidence and duration and in sUA lowering, arhalofenate also has additional features which are important in the gout population. It has shown an ability to lower triglycerides in subsets of patients with elevated serum triglycerides and to improve blood glucose parameters in diabetics, which are common comorbidities in gout patients. In an exploratory analysis, it retained its ability to lower sUA in patients with impaired renal function, another highly prevalent comorbidity in gout patients. In addition, arhalofenate gave comparable reductions in sUA whether or not patients were on low dose aspirin or thiazide diuretic (first-line therapy for uncomplicated hypertension) therapies, these latter agents being known to exacerbate hyperuricemia and to sometimes trigger flares when their treatment is initiated.

In the treatment of over a hundred patients with hyperuricemia and a diagnosis of gout, arhalofenate was safe and well tolerated and produced a consistent reduction in flare incidence and duration and in lowering sUA whether administered alone or in combination with allopurinol 300 mg or febuxostat 80 mg. The time-course of reductions in sUA was gradual and favorable for those of a drug intended to treat gout in which rapid fluctuations in sUA levels are inadvisable. It was shown as a single agent to dose-dependently increase urate excretion and fractional urate clearance, establishing that its sUA mechanism is uricosuria (i.e., it is a uricosuric).

### **Clinical Development of Arhalofenate for Treatment of Gout**

#### *Current Phase 2b Study*

The goal of our current Phase 2b study is to investigate the full potential benefit of arhalofenate monotherapy with regard to flare prevention and sUA lowering in a more robust, longer trial. Importantly, we are investigating the benefits of two doses of arhalofenate monotherapy, including a higher dose than we studied in previous gout studies, without colchicine.

This randomized, double-blind, active comparator- and placebo-controlled study will evaluate the safety, flare prevention and sUA-lowering activity of arhalofenate in approximately 225 patients with a diagnosis of gout hyperuricemia and a history of 3 or more flares in the last 12 months. The study has 5 arms including placebo, arhalofenate (600 and 800 mg), allopurinol (300 mg) and allopurinol (300 mg) plus colchicine (0.6 mg). The primary endpoint of the study is the flare incidence rate for the arhalofenate (800 mg) arm vs. allopurinol (300 mg) following twelve weeks of treatment. A key secondary endpoint is the sUA responder rate (the

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## [Table of Contents](#)

## [Index to Financial Statements](#)

percentage of patients that achieve sUA levels below 6 mg/dL) for the treatment arms. The study is designed to assess whether arhalofenate can provide sUA lowering comparable to the most commonly prescribed dose of allopurinol (300 mg) and flare reduction similar to colchicine.

### ***Phase 3 Gout Program***

The details (design, size, duration, etc.) of the Phase 3 program will be the subject of discussion at an End-of-Phase 2 meeting with the FDA, and will be designed to support an indication for both arhalofenate monotherapy and combination treatment with febuxostat.

In order to support this indication, and the broad use of arhalofenate to both prevent flares and reduce sUA, the Phase 3 clinical program is currently planned to include two pivotal gout studies: one arhalofenate monotherapy study, and one study of arhalofenate in combination with febuxostat. These will both be randomized, double-blind studies, with appropriate controls and statistical power. The program will also include a single arm, open label safety study to accumulate additional longer term safety data needed for the New Drug Application (at least 100 patients dosed for at least one year at the proposed dose). A small number of Phase 1 studies, including necessary drug-drug interaction studies, or special population studies, will also be conducted prior to registration.

### **MBX-8025**

MBX-8025 is a selective agonist (a substance that elicits a response by binding to a receptor) for the peroxisome proliferator-activated receptor delta (PPAR $\delta$ ). PPAR $\delta$  is a nuclear receptor that regulates genes involved in lipid storage, transport and metabolism (particularly fatty acid oxidation) and in insulin signaling and sensitivity. MBX-8025 has the potential to treat a variety of disorders characterized by derangements in lipid metabolism and certain diseases of the liver. Previously, MBX-8025 had been in development as a treatment for mixed dyslipidemia (elevated LDL-C and triglycerides (TGs) and often associated with decreased HDL-C). Results from our Phase 2 clinical trial of MBX-8025 in patients with mixed dyslipidemia established a number of clinically and statistically significant effects of the drug that we believe have the potential to benefit patients affected with other conditions. In this trial, MBX-8025 demonstrated an anti-atherogenic profile in which it lowered LDL-C, decreased the more atherogenic (i.e. tending to promote the formation of fatty plaques in the arteries) small dense LDL-C particles and raised HDL-C. In addition, MBX-8025 decreased TGs and free fatty acids. Whereas other lipid lowering drugs lower either TGs or LDL-C or predominantly act on one of these parameters, MBX-8025 has been shown in this trial to lower both at the same time. Treatment with MBX-8025 also led to significant decreases in gamma-glutamyl transferase (GGT), an enzymatic biomarker that has been associated with the liver inflammation that is often associated with the accumulation of fat in the liver (steatosis). Finally, treatment with MBX-8025 resulted in significant reductions in alkaline phosphatase (AP), an enzymatic biomarker associated with liver cholestasis.

Despite these positive results, we have decided not to further develop MBX-8025 for mixed dyslipidemia because of the requirement by the FDA to conduct a preapproval cardiovascular outcome study for all novel drugs in mixed dyslipidemia. This significantly increases the risk, time and cost of development for this indication.

Another factor in our decision to redirect development relates to an issue specific to compounds that work by interacting with the PPAR class of receptors (PPAR $\alpha$ , PPAR $\gamma$  and PPAR $\delta$ ), including MBX-8025. These compounds are subject to a FDA partial clinical hold which limits clinical studies to durations of less than six months until the two-year rodent carcinogenicity studies are completed and evaluated, and the hold is lifted. The decision by the FDA to lift the partial hold involves an assessment of the human relevance and perceived risk of the rodent carcinogenicity findings in relation to the benefit to the patient for the intended indication. We have completed the two-year rodent carcinogenicity studies with MBX-8025 as well as some additional follow-up studies requested by the FDA. After completion of clinical studies for HoFH or other indications described below, the FDA has indicated that they will determine whether to lift the partial hold based on the risk-benefit profile for the patient.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

For these reasons, we have decided to redirect the development of MBX-8025 for serious rare and orphan diseases or more prevalent diseases with high unmet medical need for which the risk/benefit assessment of the carcinogenicity findings would be more favorable to the patient and where an outcome study would not be necessary. We have identified a number of such indications in which there is a clear scientific rationale to suggest that the beneficial effects of MBX-8025 observed in our mixed dyslipidemia trial may be retained in that disease population. We believe MBX-8025 may provide a significant benefit for patients across a wide range of rare diseases associated with disorders of lipid metabolism, such as homozygous familial hypercholesterolemia (HoFH) and severe hypertriglyceridemia (SHTG) syndromes, and disorders of liver function, such as primary biliary cirrhosis (PBC). We also believe that MBX-8025 would have utility in the treatment of the more prevalent, but high unmet need, indication of nonalcoholic steatohepatitis (NASH).

### **Nonclinical Overview**

In *in vitro* studies with cells and animal tissues, MBX-8025 was shown to up-regulate genes involved in the metabolism and handling of lipids, most notably stimulation of fatty acid transport and oxidation.

In preclinical studies in rodents, dogs and primates, MBX-8025 demonstrated a variety of beneficial effects on the lipid profile and other metabolic parameters. MBX-8025 treatment increased peripheral oxidation of fatty acids leading to reduced levels of TGs and LDL-C, while raising HDL-C. MBX-8025 also inhibited fat mass accumulation, resulting in attenuation of body weight gain in rodent models of obesity.

Three-month toxicology studies in rodents (alone and in combination with atorvastatin, the generic name of the cholesterol lowering drug Lipitor®) and in monkeys have been completed. In addition, the two-year carcinogenicity studies in mice and rats have been completed. Johnson & Johnson Pharmaceutical Research & Development filed an IND for this compound with the FDA in July 2005 and subsequently transferred the application to CymaBay in March 2007.

### **Clinical Trials with MBX-8025**

Five Phase 1 and one Phase 2 clinical trials with MBX-8025 have been completed. The largest clinical trial was an eight-week, Phase 2 trial in which MBX-8025 was administered at doses of 50 or 100 mg/day both alone and in combination with 20 mg/day of atorvastatin in moderately obese patients with mixed dyslipidemia. This trial also had a placebo arm and a 20 mg/day atorvastatin only arm.

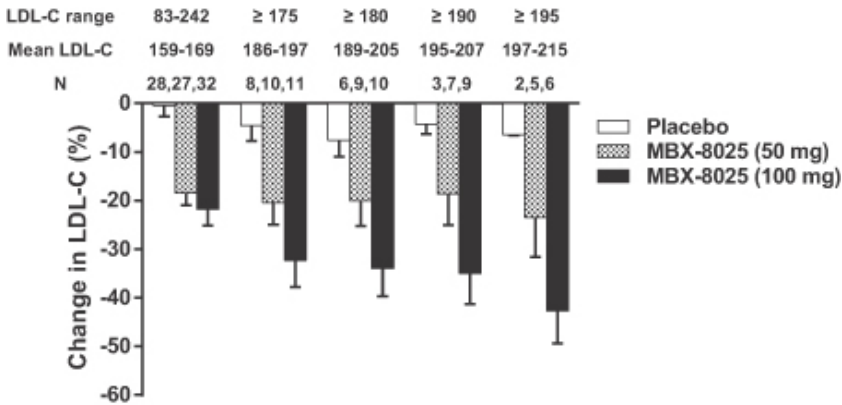
Treatment with MBX-8025 produced multiple beneficial effects on lipid parameters. First, there were significant overall reductions in total LDL-C (~20%), a parameter known to be correlated with risk of cardiovascular disease and death. The onset of the LDL-C lowering was rapid with a maximal effect seen by two weeks of treatment which was stably retained up to the end of the 8 weeks of treatment. LDL-C levels returned to pre-treatment levels within two weeks after treatment was stopped.

In addition, adding treatment with atorvastatin to MBX-8025 increased the percent change in LDL-C by approximately an additional 20% compared to that of MBX-8025 dosed alone in those patients with baseline LDL-C  $\geq$  175mg/dL. Decreases in LDL-C were correlated with baseline values, as shown in the figures below. Patients with higher baseline LDL-C values experienced larger reductions in LDL-C. Patients with baseline LDL-C in the 200 mg/dL range had reductions of approximately 40 to 50% with a dose response pattern between the 50 and the 100 mg doses. This suggests that higher doses of MBX-8025 (>100 mg) could potentially produce even larger decreases in LDL-C.

[Table of Contents](#)

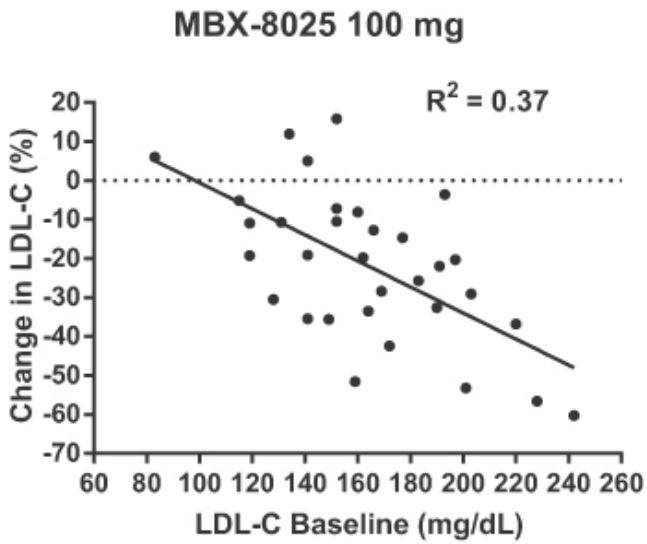
[Index to Financial Statements](#)

Change in LDL-C (%) according to baseline LDL-C



The correlation between baseline LDL-C levels and percentage change in LDL-C for subjects receiving 100 mg MBX-8025 is shown in the graph below and demonstrates a larger effect at higher baseline LDL-C values. These data suggest that MBX-8025 could potentially be a particularly effective treatment for diseases in which LDL-C is markedly elevated.

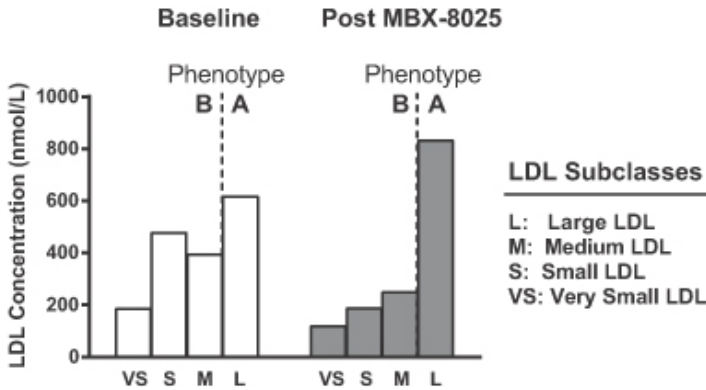
Individual Patient % Change from Baseline in LDL-C according to Baseline LDL-C



[Table of Contents](#)

[Index to Financial Statements](#)

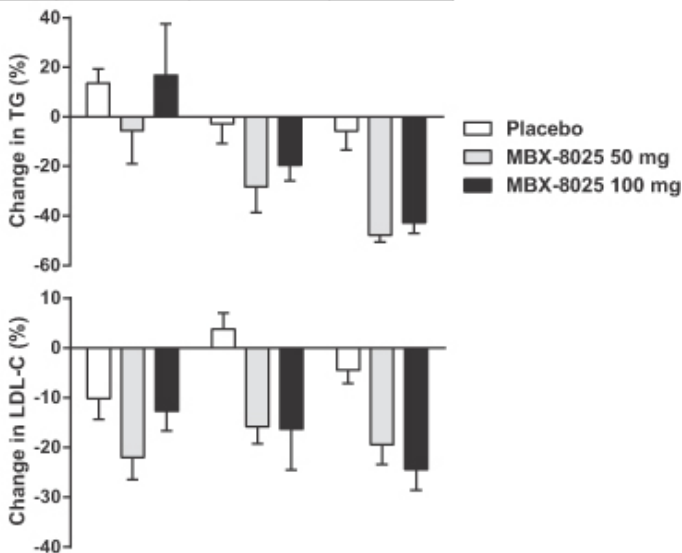
In this trial, lipoprotein particle size measurements were also performed to assess the effect of MBX-8025 on LDL particle subtype distribution. It is believed that small dense LDL particles (type B) are the more atherogenic subtype and that they confer a greater risk for atherogenesis (promotion of arterial plaque formation). As shown below, MBX-8025 selectively depleted the small dense LDL particles, converting them to the larger, more buoyant and less atherogenic phenotype A.



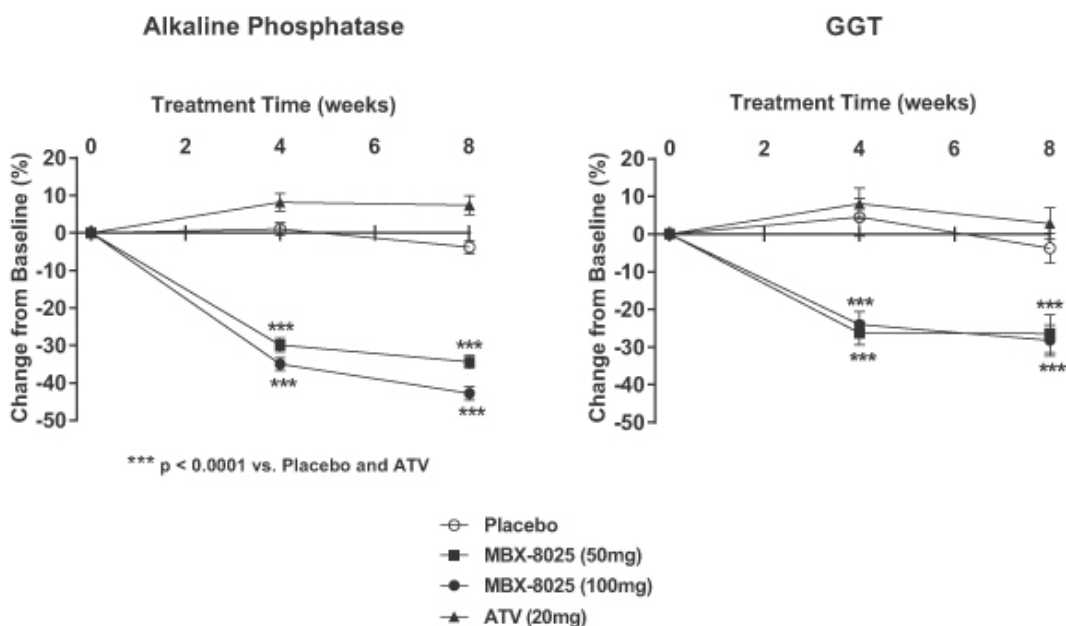
Another beneficial effect of MBX-8025 observed in this Phase 2 clinical trial was a decrease in both TGs (~30%) and free fatty acids (10-15%). The reductions in TGs are illustrated in the figure below where the effect is shown as a function of baseline TG concentration (subdivided into three groups as defined by the National Cholesterol Education Program Adult Treatment Panel III, or NCEP ATP III). At baseline values above 200 mg/dL, the reductions are approximately 50%. Also shown in this figure are the changes in LDL-C for the same patients that experienced the reductions in TGs. At all doses of MBX-8025, the reductions in TGs are associated with a concomitant reduction (15-25%) in LDL-C. Thus, MBX-8025 lowered both TGs and LDL-C in the same patients in this clinical trial. A similar pattern of simultaneous decreases in TGs and LDL-C were observed in the MBX-8025 plus atorvastatin arms of the trial.

Change in TG as a function of baseline TG by NCEP ATP III:

NCEP ATP III	Normal TG	Borderline TG	High TG
TG range	<150	150-199	≥ 200
Mean TG	119-133	171-187	264-295
N	2, 5, 3	10, 7, 6	14, 14, 23



MBX-8025 also produced statistically significant decreases in GGT and AP at both doses of 50 and 100 mg, whereas there were no changes with either placebo or atorvastatin. GGT has been described as a marker of liver inflammation that is associated with the deposition of fat in the liver and AP is a validated marker of liver cholestasis.



### Future Development Plan for MBX-8025

We have decided to redirect the development of MBX-8025 toward serious rare and orphan diseases or more prevalent diseases with higher unmet medical need. We have focused on diseases in which there is a clear scientific rationale or clinical data to suggest that the beneficial effects of MBX-8025 observed in our mixed dyslipidemia trial may be retained in that disease population. The indications of interest are HoFH, SHTG, PBC and NASH.

### Homozygous Familial Hypercholesterolemia (HoFH)

HoFH is a rare, life-threatening, genetic disease characterized by marked elevations in plasma levels of LDL-C leading to severe atherosclerosis and the development of premature cardiovascular diseases. While normal LDL-C levels are approximately 100 mg/dL, patients with HoFH may have levels in the 500 to 1000 mg/dL range. Symptomatic cardiovascular disease often presents during the first decades of life leading to myocardial infarction, ischemic stroke, and death. If untreated, most HoFH patients do not survive beyond the age of 30.

HoFH is caused by loss-of-function mutations in both genes of the low-density lipoprotein receptor (LDL-R) protein, leading to reduced or absent LDL-R function. The disease affects approximately one in one million persons. The loss of LDL-R function leads to impaired removal by the liver of LDL-C from the circulation, resulting in exceptionally high LDL-C blood concentrations.

Treatment of HoFH is focused on reducing LDL-C levels, as compelling evidence exists from randomized, double-blind, placebo-controlled studies to support the causality of LDL-C in atherosclerotic cardiovascular disease. Considerable evidence implicates LDL-C as a causal mediator of cardiovascular disease in HoFH patients and reductions in LDL-C can be expected to decrease the risk of cardiovascular disease. It is known that HoFH subjects undergoing LDL-C apheresis, have a reduction in cardiovascular disease events.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

Initial treatment of HoFH entails adoption of a low fat diet and exercise program, usually with limited effectiveness. This is followed by conventional pharmacological therapies for reducing LDL-C, including statins, cholesterol absorption inhibitors and bile acid sequestrants. Unfortunately, these conventional therapies work largely through up-regulation of the LDL-R. Thus, they are minimally effective in patients with HoFH in whom LDL-R activity is impaired or absent. Patients having a small amount of residual LDL-R activity may receive a modest reduction in LDL-C with maximal conventional therapy, but most patients with HoFH respond insufficiently.

Plasma apheresis is a selective mechanical filtration of blood that may be used to remove LDL-C and is currently a treatment of choice for HoFH. However, apheresis is a complex and inconvenient procedure that could require an arterio-venous fistula and has numerous side effects. The procedure is not widely available throughout the US and Europe. Apheresis reduces LDL-C levels transiently, but must be repeated every one to two weeks because LDL-C levels rebound.

Two new drugs have recently been approved for use in combination with diet, exercise and conventional lipid lowering therapy to treat HoFH. The first is lomitapide (Juxtapid, Aegerion® Pharmaceuticals) that lowers LDL-C by inhibiting microsomal triglyceride transfer protein (MTP), a protein whose activity is required for the production of very low density lipoprotein (VLDL-C), a precursor of LDL-C. Lomitapide produces decreases in LDL-C of approximately 40% from a baseline LDL-C level of 337 mg/dL and gets 28% of patients to the LDL-C target of <100 mg/dL. A side effect of lomitapide treatment is that fat accumulates in the liver, thereby causing hepatic steatosis, with or without concurrent increases in transaminases. For this reason, the drug carries a black box warning and a requirement for monthly liver function monitoring tests. Lomitapide also blocks MTP in enterocytes (cells lining the gastrointestinal tract), leading to an accumulation of fat in the intestinal mucosa. This can reduce the absorption of fat-soluble nutrients and causes gastrointestinal issues (diarrhea, abdominal pain). Subjects on lomitapide should be prescribed concomitant fat-soluble vitamin supplementation and should adhere to a restrictive diet supplying less than 20% of energy from fat.

The second drug is mipomersen (Kynamro, ISIS Pharmaceuticals). It lowers LDL-C by acting as an anti-sense oligonucleotide inhibitor that blocks the synthesis of apo B-100, the protein component of LDL-C. Mipomersen lowers LDL-C by approximately 25% from a baseline LDL-C of 439 mg/dL. Like lomitapide, mipomersen causes the accumulation of fat in the liver, confers a risk of hepatic steatosis and carries a black box warning and requirement for monthly liver function monitoring tests.

While these two newly registered drugs offer additional treatment options for patients with HoFH, there remains a high degree of unmet medical need. Even with an aggressive combination of available therapies, subjects with HoFH generally have LDL-C levels substantially above treatment targets. Many patients also have difficulty accessing or tolerating available treatments. We believe that MBX-8025 has attributes that are well suited to the treatment of HoFH and should be independent of the LDL-R activity. This is supported by studies on another PPAR $\delta$  agonist, GW501516, in mice that lack the LDL-R. Thus, we hypothesize that the LDL-C lowering effect observed in our earlier studies in patients with mixed dyslipidemia may be transferable to patients with HoFH. If MBX-8025 is able to reduce LDL-C in these patients and retains the favorable safety profile observed thus far in our clinical studies, we believe it has the potential to be the front line pharmacological treatment for HoFH. We plan to conduct a small placebo-controlled double-blind proof-of-concept Phase 2 study in patients with HoFH to test this hypothesis.

It is likely that many patients with HoFH will require combination therapy with LDL-C lowering agents in order to achieve enough lowering of LDL-C to reach goal of < 100 mg/dL. Thus we believe there may be opportunities to combine MBX-8025 with other therapies including lomitapide or mipomersen. In this scenario, we note that the ability of MBX-8025 to reduce hepatic fat may potentially mitigate or prevent the development of hepatic steatosis and steato-hepatitis associated with lomitapide and mipomersen.

### **Severe Hypertriglyceridemia (SHTG)**

Severe HTG (SHTG, TGs > 500 mg/dL) is associated with an increased risk of pancreatitis. As a result, management of HTG and SHTG is also an important goal of lipid therapy. Most patients with HTG can be managed



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## [Table of Contents](#)

### [Index to Financial Statements](#)

with available TG-lowering therapies including fibrates, niacin and fish oil components. However, there remains an unmet need for addressing SHTG which may arise from a variety of circumstances. It is estimated that there are approximately five million patients in the US with SHTG; however, the Fredrickson classification of hyperlipidemias further subdivides the overall population into several types, some of which can be classified as orphan diseases.

According to the Fredrickson classification of hyperlipidemias, several types of HTG have been identified. This includes Type 1a, a rare genetic disease also called familial chylomicronemia syndrome (FCS), in which chylomicrons are markedly elevated due to decreased activity of lipoprotein lipase (LPL), the enzyme that is primarily responsible for their metabolism. FCS affects about one in one million people worldwide. Type 1b is another form characterized by a deficiency in a protein component of chylomicrons called apo-CII which is needed to activate LPL and facilitate chylomicron metabolism. Another form is Type 5 in which very low density lipoprotein (VLDL) is elevated in addition to chylomicrons and is likely caused by yet incompletely defined variety of molecular defects.

The need for better treatments for SHTG has been recognized and several new therapies either have been brought to the market or are in development. One popular approach has been to develop components of fish oil. Lovaza is a marketed drug that is a mixture of the omega-3 fatty acids esters eicosapentaenoic acid (EPA) and docosahexaenoic acid (DHA) isolated from fish oil. In patients with SHTG (TGs > 500 mg/dL), it has been shown to reduce TGs by over 40%, but the reductions are accompanied by increases in LDL-C of over 40%. Vascepa, an ethyl ester of EPA, is also on the market for the treatment of SHTG and lowers TGs by approximately 30% with no significant effect on LDL-C. Epanova is a complex mixture of polyunsaturated free fatty acids derived from fish oils, including multiple long-chain omega-3 and omega-6 fatty acids, with EPA, DHA, and docosapentaenoic acid being the most abundant forms. In patients with SHTG, Epanova produced decreases in TGs of approximately 30% with increases of approximately 25% in LDL-C.

Other drugs are currently in earlier stage development for SHTG. ISIS-APOCIIIIRX is an oligonucleotide inhibitor of apo-CIII, a lipoprotein component that regulates TG metabolism. Loss-of-function mutations in apo-CIII are associated with lower levels of TGs. In a Phase 2 study in patients with SHTG, ISIS-APOCIIIIRX produced reductions in TGs of up to 70%. The effects on LDL-C were not reported. Another product candidate, CAT-2003, produced decreases in both fasting and post prandial (post meal) TGs in normal healthy volunteers and has been advanced into Phase 2 studies in SHTG.

We believe that MBX-8025 may be uniquely able to benefit patients with SHTG by virtue of its ability to simultaneously lower TGs and LDL-C. This benefit has been observed both in monotherapy as well as in combination with atorvastatin in patients with mixed dyslipidemia. Drugs currently marketed for the treatment of SHTG lower TGs with either a worsening or lack of meaningful improvement in LDL-C. Recognizing that SHTG is a heterogeneous collection of diseases, we are continuing our assessment of the best patient populations to study in a small Phase 2 clinical trial.

### **Primary Biliary Cirrhosis (PBC)**

PBC is a slowly progressive autoimmune disease of the liver characterized by portal inflammation and immune-mediated destruction of intrahepatic bile ducts. The loss of bile duct function leads to decreased bile secretion and the retention of toxic substances within the liver, resulting in further hepatic damage, fibrosis, cirrhosis and, eventually, liver failure. It is a common cause of liver transplantation.

PBC affects primarily women with peak incidence in the fifth decade of life. It has been recognized as an orphan disease both in the US and in the EU. It is a long-term debilitating and life-threatening disease. Fatigue and pruritus are the most common presenting symptoms. Pruritus (itching), which occurs in 20 to 70% of patients, can be extremely distressing for patients. Other common findings include jaundice, hyperlipidemia (notably hypercholesterolemia), hypothyroidism, osteopenia and osteoporosis, and coexisting autoimmune diseases. Portal hypertension is a late complication of the disease, as is malabsorption, deficiencies of fat-soluble vitamins, and steatorrhea (excess fat in feces).

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## [Table of Contents](#)

## [Index to Financial Statements](#)

Currently, the only FDA-approved treatment is ursodeoxycholic acid, also known as ursodiol, an isomer of chenodeoxycholic acid. Ursodiol decreases serum levels of AP, bilirubin, alanine aminotransferase, aspartate aminotransferase, cholesterol, and immunoglobulin M, all of which are elevated in patients with PBC and can serve as biochemical markers of the disease. In a study that combined data from three controlled trials with a total of 548 patients, ursodiol significantly reduced the likelihood of liver transplantation or death after four years. Ursodiol also delayed the progression of hepatic fibrosis in early-stage PBC, but was not effective in advanced disease. It is also known that up to 50% of PBC patients fail to respond to ursodiol therapy.

Other therapies, such as colchicine, methotrexate, prednisone and multiple immunosuppressive regimens have been attempted. However, their efficacy is controversial, limited, or unproven and they are associated with multiple side-effects impacting tolerance and safety. Liver transplantation improves survival in patients with PBC, and it is the only effective treatment for those with liver failure. However cirrhosis recurs in 15% of patients at three years and in 30% at 10 years. As a result, despite the previously mentioned therapeutic interventions, it is recognized that PBC continues to progress in many patients and additional medical treatment is needed to address this disease.

The bile acid analog obeticholic acid (OCA) is in development (Intercept Pharmaceuticals) for PBC. OCA has received orphan designations in US and EU and Fast Track status in the US. Clinical proof-of-concept has been established in two 12-week Phase 2 studies (one in ursodiol non-responders and one in treatment naïve or intolerant patients) using AP as the primary endpoint (<1.67 times the upper limit of normal with >15% reduction) + normal bilirubin. Approximately 40% of patients met the primary endpoint. A Phase 3 study has recently been completed that met its primary endpoint. It remains unclear what the criteria are for registration.

Both AP and GGT are common biochemical markers of cholestasis and their elevation is presumably a consequence of the toxic effects of retention of bile acids in cells in the biliary duct. AP levels in PBC patients have been used as a primary outcome measure in proof-of-concept clinical trials and as a key secondary outcome in pivotal trials. The observation that MBX-8025 produces significant reductions in these surrogate markers suggests that the drug may improve biliary function, ameliorate cholestasis and, hence, be a novel treatment for PBC. The coordinate decrease in AP and GGT levels indicates that the AP decrease is indeed hepatic in origin. The magnitude of the change in AP with MBX-8025 (~40%) is similar to that seen after treatment with ursodeoxycholic acid after eight weeks. In addition to the potential benefit to improving biliary function, we believe MBX-8025 may confer improvements in lipid parameters including reductions in LDL-C and TGs.

The precise mechanism by which MBX-8025 improves cholestasis by acting as a PPAR $\delta$  agonist is not fully understood. However, there is some supporting preclinical data. In the bile ligation model of cholestasis, the PPAR $\delta$  agonist KD3010 reduced hepatic injury, fibrosis and inflammation, while increasing survival. In addition, treatment of mice with the PPAR $\delta$  agonist GW610742 has been shown to produce significant and large increases in bile flow and the production of bile salts.

We are currently evaluating the initiation of a Phase 2 proof-of-concept study for MBX-8025 in patients with PBC.

### **Non-Alcoholic Fatty Liver Disease (NAFLD) / Nonalcoholic Steatohepatitis (NASH)**

NAFLD is a disease characterized by accumulation of fat in the liver of people who drink little or not at all. Approximately one-third of NAFLD patients develop NASH, which is characterized by inflammation in the liver that is often accompanied by fibrosis. This can progress to cirrhosis, followed by eventual liver failure and death. NASH is the third most common reason for liver transplantation in the United States. NASH is a major challenge to healthcare systems worldwide. NASH is initially a silent disease, the first sign of which may be elevations in transaminases such as alanine aminotransferase (ALT) or aspartate aminotransferase (AST) from routine blood test panels. When further evaluation rules out medications, viral hepatitis, alcohol, etc. as a cause, or when imaging studies of the liver show fat, NASH is suspected. A confirmation of a diagnosis of NASH requires a liver biopsy.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

There are currently no drugs approved by the FDA for the treatment of NASH. However, a number of clinical studies have been carried out or are underway with drug candidates that may affect disease outcomes in patients with NASH, including OCA (Intercept Pharmaceuticals) and GFT505, a PPAR $\alpha/\delta$  agonist (Genfit).

Based on data from our Phase 2 clinical trial in patients with mixed dyslipidemia and available data from other PPAR $\delta$  agonists, we believe MBX-8025 may have utility in treating patients with NASH. The decrease in GGT, a biochemical marker which has been recognized to be linked with hepatic fat accumulation, observed in our phase 2 mixed dyslipidemia trial is consistent with results reported for another PPAR $\delta$  agonist GW501516. A short term clinical trial with GW501516 investigators demonstrated that the compound decreased hepatic fat. In addition to our clinical experience with MBX-8025, along with that of other PPAR $\delta$  agonists, the well documented property that MBX-8025 induces the oxidation of fatty acid leads us to believe that our compound could potentially benefit patients affected with NAFLD who are further at risk of developing NASH. Although we do not currently anticipate near term development of MBX-8025 in NASH, we continue to evaluate the opportunity among a number of additional indications.

### **Cymabay Clinical Strategy for MBX-8025**

Our initial strategy is to evaluate and carry out proof-of-concept clinical trials in HoFH, SHTG and PBC to assess whether MBX-8025 is able to produce the predicted improvements in the relevant biomarkers associated with these diseases. In all three indications, clinically and statistically significant markers of disease status can be achieved in relatively small (10-20 patients) studies of three months or less duration. In cases where clinical proof-of-concept is achieved, we believe that we could move rapidly into a Phase 3 registration program based on the high unmet need in these indications. We continue to assess a variety of criteria (patient availability, regulatory pathway clarity, commercial attractiveness, etc.) with which to prioritize these indications and expect to complete our analysis in the second half of 2014. We intend to start proof-of-concept studies in at least two indications in the first half of 2015 from the use of proceeds from this offering.

### **MBX-2982**

Type 2 diabetes is a chronic debilitating disease characterized by a progressive loss of the normal control of glucose levels in the blood and other tissues. There are several established and emerging classes of drug therapies for diabetes. Over the last decade, injectable drugs have emerged as competing drugs with significant benefits in glucose control as well as effects on weight loss and the potential to protect the pancreas from the damage caused by the progression of diabetes. These drugs are primarily analogs of the natural hormone glucagon-like 1 peptide (GLP-1), and include exenatide, liraglutide and lixisenatide among others. These drugs are given by subcutaneous injection once or twice daily. Their action is to provide glucose-regulated insulin secretion with weight loss and the potential to preserve function of pancreatic islets. New members of this class with once weekly to once monthly dose schedules have been approved or are in late stage development. In spite of the variety of drugs available for the treatment of diabetes, the medications used to manage diabetes have not led to optimal control of hyperglycemia and many are associated with dose-limiting side effects. MBX-2982 is an oral, G-protein coupled receptor (GPR119) agonist being evaluated as a novel therapeutic agent for patients with T2DM, with a dual mechanism including direct effects and indirect effects mediated by gastrointestinal hormones known as incretins on glucose-dependent insulin secretion, as well as potentially beneficial effects on islet health.

GPR119 is expressed in pancreatic islet cells and gastrointestinal hormone secreting cells (enteroendocrine cells). Activation of GPR119 in pancreatic  $\beta$ -islets either by natural (endogenous) substances or by drugs developed to interact with it (GPR119 agonists) results in direct stimulation of glucose-dependent insulin secretion *in vitro*. Activation of GPR119 in intestinal enteroendocrine cells either by endogenous substances or by GPR119 agonists results in stimulation of glucagon-like peptide 1 (GLP-1) and gastrointestinal inhibitory peptide release, and subsequent enhanced glucose-dependent insulin secretion and suppression of glucagon, leading to improved acute glucose tolerance, both *in vitro* and *in vivo*. MBX-2982 was synthesized and screened as a GPR119 agonist, and is capable of activating endogenous GPR119 in a cell line over-expressing the receptor. MBX-2982 has been shown to increase glucose-dependent insulin secretion in both *in vitro* and in animal models. MBX-2982 also increases incretin hormone levels in animals, which may contribute to its glucose lowering effects.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

Nonclinical studies show that MBX-2982 has desirable effects on blood glucose levels, and this effect is additive to the effect of the dipeptidyl peptidase-4 (DPP-4) inhibitor, sitagliptin. Based on these results, there may be an important role for MBX-2982 as a novel therapeutic agent in the treatment of T2DM, alone or in combination with other anti-diabetic agents, including the DPP-4 inhibitors. Presently, there are no other agents approved in the U.S. within this pharmacologic class for the treatment of T2DM.

Extensive preclinical toxicological (up to 6 months in rats and dogs) have been completed, and PK profiling of MBX-2982 has shown low potential for safety risk. We filed an IND for MBX-2982 with the FDA in January 2008.

### **Clinical Studies with MBX-2982**

Four Phase 1 clinical studies and one Phase 2 clinical study with MBX-2982 have been completed and the safety and PK review showed no safety or tolerability concerns with MBX-2982 administered in escalating doses (25, 100, and 300 mg/day) tested for up to 4 weeks of dosing. A four-week study in type 2 diabetics can be summarized as follows:

- MBX-2982 generally lowered mean weighted glucose and post-meal glucose during an extended mixed-meal tolerance test (MMTT), although not always to a statistically significant degree and not to the extent of sitagliptin. The effect at the 300 mg dose may have been mitigated by the inclusion of a very small number of patients who experienced extreme worsening of glucose to the degree of being statistical outliers. Decreases in fasting glucose were generally not observed with MBX-2982.
- Four weeks of treatment with MBX-2982 tended to increase insulin, active GLP-1, and total GLP-1 during an extended MMTT. Decreases in glucagon were not as consistently observed. Changes in active GLP-1 were not as robust as those observed with sitagliptin. Four weeks of treatment with MBX-2982 also tended to increase fasting insulin and c-peptide, and decrease fasting triglycerides.
- Overall, the data suggest that MBX-2982 may decrease glucose, potentially through effects on GLP-1, glucagon, and insulin. Changes in HbA1c are difficult to assess over a 4-week treatment period, but trended in the downward direction. Glucose-lowering effects and mechanism of action will need to be explored more robustly in longer duration trials of MBX-2982.
- The PK results observed in this study are similar to those seen in the completed Phase 1 study that used the same formulation, demonstrating dose-dependent increases in drug exposure and a profile supporting once daily oral dosing.
- MBX-2982 at doses of 25, 100, and 300 mg was safe and well tolerated.

Based on these results, we believe further testing with MBX-2982 in combination with sitagliptin and/or metformin for the treatment of diabetes is warranted.

### **Next Steps in Development of MBX-2982**

Prior to conducting the fourth Phase 1 clinical study and the Phase 2 clinical study, we entered into an exclusive license agreement for MBX-2982 with Sanofi-Aventis in June 2010. In June 2011, Sanofi-Aventis terminated the license and has no further rights to MBX-2982. A proof-of-concept study has been designed to determine the effects of MBX-2982 on fasting and post-challenge blood glucose in patients with T2DM either as dual therapy in combination with either metformin or sitagliptin, or as triple therapy in combination with metformin and sitagliptin. Successful achievement of study goals would position the drug for a Phase 2b study, followed by a Phase 3 program.

We do not anticipate conducting this study until a suitable partner is found to contribute funding or resources for the project, or until sometime in the future when we have sufficient capital resources.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

### License Agreements and Intellectual Property

#### General

CymaBay actively seeks to obtain, where appropriate, patent protection and regulatory exclusivity for the proprietary technology that it considers important to its business, including compounds, compositions and formulations, their methods of use and processes for their manufacture both in the United States and other countries. CymaBay also relies on trade secrets, know-how, continuing technological innovation and in-licensing to develop and maintain its proprietary position. Our success depends in part on our ability to obtain, maintain and enforce proprietary protection for our product candidates, technology and know-how, to operate without infringing the proprietary rights of others, and to exclude others from infringing our proprietary rights. However, patent protection may not afford CymaBay complete protection against competitors who seek to circumvent CymaBay's patents.

CymaBay also depends upon the skills, knowledge, experience and know-how of its management, research and development personnel, as well as that of its advisors, consultants and other contractors. To help protect its proprietary know-how, which is not patentable, and for inventions for which patents may be difficult to enforce, CymaBay currently relies and will in the future rely on trade secret protection and confidentiality agreements to protect its interests. To this end, CymaBay requires all of its employees, consultants, advisors and other contractors to enter into confidentiality agreements that prohibit the disclosure of confidential information and, where applicable, require disclosure and assignment to it of the ideas, developments, discoveries and inventions important to its business.

#### Collaborations and Licensing Agreements

CymaBay has entered into various arrangements with licensors and licensees. The current collaborations are summarized below.

**Johnson and Johnson:** In August 2006, CymaBay entered into a strategic alliance with Ortho-McNeil, Inc. As part of the alliance, Janssen Pharmaceutical NV, an affiliate of Ortho-McNeil, granted to CymaBay an exclusive worldwide, royalty-bearing license to MBX-8025 and certain other PPAR $\delta$  compounds (the "PPAR $\delta$  Products") with the right to grant sublicenses to third parties to make, use and sell such PPAR $\delta$  Products. Under the terms of the agreement, CymaBay has full control and responsibility over the research, development and registration of any PPAR $\delta$  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 PPAR $\delta$  Products. Janssen has a right of first negotiation under the agreement to license a particular PPAR $\delta$  Product from CymaBay in the event that CymaBay elects to seek a third party corporate partner for the research, development, promotion, and/or commercialization of such PPAR $\delta$  Products. Under the terms of the agreement Janssen is entitled to receive up to an 8% royalty on net sales of PPAR $\delta$  Products. Under the terms of the agreement, if CymaBay does not expend more than a de minimus amount of effort and resources on the research and/or development of at least one PPAR $\delta$  product, such action would constitute a default under the agreement. In addition, if CymaBay fails to make any payment called for under the agreement, discloses any non-exempt confidential information related to the agreement, or fails to use diligent efforts to promote, market and sell any PPAR $\delta$  product under the agreement, such action would constitute a default under the agreement. In the event of such default, or upon CymaBay's termination of the agreement, CymaBay shall grant Janssen a worldwide, exclusive, irrevocable license under the agreement in all information that is controlled, developed or acquired by CymaBay which relate to a PPAR $\delta$  compound or PPAR $\delta$  product and in all patents that are filed during the term of the agreement with a priority date after the effective date of the agreement and relate to a PPAR $\delta$  compound or PPAR $\delta$  product.

In June 2010, CymaBay entered into two development and license agreements with Janssen Pharmaceuticals, Inc. (Janssen) 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. CymaBay is also eligible to receive up to \$228 million in contingent payments if certain development and commercial events are achieved as well as royalties on worldwide net sales of products. No

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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. A joint steering committee with equal representation from each party will oversee the development of products. Following June 2012, all decisions of the joint steering committee will be made by Janssen. CymaBay has the sole responsibility, for the preparation, filing, prosecution, maintenance of, and defense of the CymaBay patents with respect to, metabolic disease target agonists. Under the terms of the agreements, if CymaBay discloses any non-exempt confidential information related to the agreements, such action would constitute a default under the agreements. In addition, if CymaBay breaches any of its representations or warranties under the agreements, such action would constitute a default. In the event of a default, the agreements do not provide that CymaBay will lose any of its rights to the intellectual property developed under the agreement.

**DiaTex:** On June 30, 1998, we entered into a License and Development Agreement with DiaTex, Inc. Under the agreement, DiaTex granted us an exclusive license to develop and commercialize therapeutic products containing halofenate, its enantiomers (mirror images, including arhalofenate), derivatives, and analogs (the licensed products) for the treatment of diseases. Under terms of the agreement, DiaTex will work cooperatively and assist us in conducting a program for the research and development of halofenate and its enantiomers including the right to sublicense, to use and to practice all patents controlled by DiaTex that claim halofenate and its enantiomers, and all information, data, know-how, trade secrets, inventions, developments, results, techniques and materials, whether or not patentable, that are necessary or useful towards such commercialization. Under the agreement, we are obligated to use diligent efforts to conduct preclinical and clinical testing of halofenate and its enantiomers in order to determine its efficacy for use in the treatment or prevention of human diseases or conditions. On April 15, 1999 the agreement was amended by the parties to allow DiaTex to transfer to us their interest in an IND application that they filed with the FDA. The amendment also provided for DiaTex to indemnify us against any and all losses resulting or arising from any third party claims, actions or proceedings under the IND application, any negligent or wrongful acts or omissions of DiaTex in connection with the IND application, and any misrepresentations by DiaTex relating to the license agreement. Under the amendment, we will provide the same indemnifications to DiaTex with respect to any third party claims, actions, or proceedings in connection with negligent or wrongful conduct of clinical trials relating to the license agreement, provided the claims are not related to negligent or wrongful acts or omissions committed by DiaTex.

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. DiaTex is entitled to up to \$0.8 million for the future development of arhalofenate, as well as a 2% royalty payment on any net sales of products containing arhalofenate. A \$50,000 milestone payment was made in May 2005 but no other milestone or royalty payments have been made since then. The agreement will expire upon the expiration of the last of DiaTex's patents related to the license granted, or, if later, the expiration of all payment obligations under the agreement. The agreement may also terminate upon a material breach by DiaTex or us, if written notice of such breach is delivered to the breaching party, and the breaching party has not (i) cured the breach or (ii) initiated good faith efforts to cure the breach within a specified time period. Under the terms of the agreement, if we fail to use diligent efforts to conduct preclinical and clinical testing of halofenate and its enantiomers to determine its efficacy for use in the treatment or prevention of human diseases or conditions, fail to make any payment called for under the agreement, or disclose non-exempt confidential information under the agreement, such action would constitute a material breach under the agreement. In addition, if we fail to execute all instruments and assignments or fail to take any action to effect joint ownership of any enantiomer patent with DiaTex, such action would constitute a material breach under the agreement. We may terminate the agreement at any time if we determine we are no longer interested in DiaTex's license grant, provided we provide sufficient written notice within a specified time period.

### **Research and Development Agreements**

**INC Research:** In February 2014, we entered into a Master Services Agreement with INC Research, LLC and related initial work order for INC Research to provide contract clinical research and development services to us in connection with our Phase 2b study. The Agreement provides that we may engage INC Research from time

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## [Table of Contents](#)

## [Index to Financial Statements](#)

to time to provide services in accordance with work orders mutually agreed and budgeted between the parties for clinical research and development of arhalofenate which total is anticipated to exceed approximately \$8 million. The master services agreement provides customary terms and conditions, including those for performance of services by INC Research in compliance with work orders, standard operating procedures, FDA and ICH requirements and all applicable laws. We remain responsible for all regulatory responsibilities and the determination of any work orders, subject to mutual agreement on the specific terms of any such work orders. The master services agreement has a term of five years; provided that we may terminate the master services agreement or any individual work order on thirty (30) days written notice, or immediately in the event of any safety risk associated with the services the being performed. In addition, either party may terminate the master services agreement or any applicable work order upon thirty (30) days written notice for a material breach by the other party.

### **Intellectual Property**

CymaBay owns and co-owns approximately 29 United States patents, 160 foreign patents, as well as 26 United States patent applications and 185 foreign and Patent Cooperation Treaty applications which are counterparts to certain United States patents and patent applications. In addition, we license from third parties approximately 17 United States patents and 1 United States patent application, 221 foreign patents and 70 foreign and Patent Cooperation Treaty applications which are counterparts to certain United States patents and patent applications. These patents and patent applications include claims covering various aspects of our product pipeline and research and development strategies, including: arhalofenate crystal forms, methods of use both alone and in combination with other drugs and methods of manufacture, certain PPAR delta agonists, their compositions and uses, certain GPR119 agonist compositions and uses and undisclosed metabolic disease target agonist compositions and uses.

The arhalofenate portfolio consists of approximately 130 issued patents and 78 pending patent applications relating to composition, method of use or methods of manufacture. We believe our issued patents protect Arhalofenate through at least 2019-2029 before accounting for any potential patent term extension. The MBX-8025 portfolio consists of approximately 83 issued patents and 41 pending patent applications related to composition and method of use that we believe protect it through at least 2024-2026 before accounting for any potential patent term extension.

Patent and trade secret protection is critical to our business. Our success will depend in large part on our ability to obtain, maintain, defend and enforce patents and other intellectual property to extend the life of patents covering our product candidates, to preserve trade secrets and proprietary know-how, and to operate without infringing the patents and proprietary rights of third parties we actively seek patent protection in the U.S.

### **Manufacturing**

CymaBay does not currently own or operate manufacturing facilities for the production or testing of arhalofenate or other product candidates that it develops, nor does it have plans to develop its own manufacturing operations in the foreseeable future. CymaBay presently depends on third party contract manufacturers to obtain all of its required raw materials, Active Pharmaceutical Ingredients (APIs) and finished products for its clinical studies for arhalofenate. CymaBay has executed manufacturing agreements for its API and tablet supplies of arhalofenate with established manufacturing firms which are responsible for sourcing and obtaining the raw materials necessary for the finished products. The raw materials necessary to manufacture the API for arhalofenate, MBX-8025 and MBX-2982 are available from more than one source and CymaBay has also executed manufacturing agreements for the production of MBX-8025 and MBX-2982.

### ***Siegfried AG***

On April 30, 2012, CymaBay entered into a Development and Clinical Manufacture Agreement with Siegfried AG for the manufacturing of the API necessary for the tablet form of arhalofenate. Under the agreement, CymaBay shall deliver or Siegfried shall obtain the raw materials necessary for the API. CymaBay owns the rights, title and interest to the deliverables and intellectual property covering the deliverables generated

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## [Table of Contents](#)

## [Index to Financial Statements](#)

under the agreement. Siegfried shall grant a non-exclusive license to CymaBay to use Siegfried intellectual property to exploit any product or service based or derived from the deliverables under the agreement. Both Siegfried and CymaBay have agreed to indemnify the other party with respect to losses due to the breach of a covenant or obligation under the agreement or the gross negligence, recklessness or intentional misconduct of the other party. CymaBay may terminate the agreement at any time with written notice and Siegfried may terminate the agreement in the event CymaBay discontinues its activities related to the development or commercialization of the API for arhalofenate. In addition, either party may terminate the agreement at any time for material breach under the agreement or in the case of insolvency of the other party.

### *Patheon Inc.*

On June 5, 2012, CymaBay entered into a Development and Clinical Manufacture Agreement with Patheon Inc. for the manufacturing of the tablet form of arhalofenate. Under the agreement, CymaBay shall deliver the API or Patheon shall obtain the API from a qualified vendor. CymaBay owns the rights, title and interest to the deliverables and intellectual property generated by Patheon in connection with the performance of the services for CymaBay under the agreement. Both Patheon and CymaBay have agreed to indemnify the other party with respect to losses due to the breach of a covenant or obligation under the agreement or the gross negligence, recklessness or intentional misconduct of the other party. CymaBay may terminate the agreement at any time with written notice provided that CymaBay terminates the agreement within certain times in advance of the start date of certain services. In addition, either party may terminate the agreement at any time for material breach under the agreement.

### *Metrics Inc.*

On October 31, 2006, CymaBay entered into a Standard Development Agreement with Metrics, Inc. Under the agreement, Metrics will provide CymaBay with pharmaceutical development, formulation and analytical services in consideration of which CymaBay will provide appropriate compensation as outlined in the agreement. CymaBay owns the rights, title and interest to the intellectual property relating to all pharmaceutical products developed or manufactured for CymaBay by Metrics, as well as any active pharmaceutical ingredient provided to Metrics by CymaBay. CymaBay has agreed to indemnify Metrics against third party claims that involve the breach by CymaBay of any of its obligations, warranties or representations under the agreement, and Metrics has agreed to indemnify CymaBay against third party claims that involve (i) the negligence, gross negligence, or intentional misconduct on the part of Metrics, (ii) a failure by Metrics to comply with the law in their performance of the agreement, or (iii) a breach of Metrics' obligations, covenants, representations, or warranties under the agreement. Either party may terminate the agreement at any time with advance written notice.

## **Research & Development Costs**

Research and development costs for the years ended December 31, 2013 and 2012 were \$4.5 million and \$9.3 million, respectively, and were \$2.6 million for the three months ended March 31, 2014.

## **Government Regulation and Product Approval**

Government authorities in the United States, at the federal, state and local level, and other countries extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of products such as those CymaBay is developing. The pharmaceutical drug product candidates that CymaBay develops must be approved by the Food and Drug Administration (FDA) before they may be legally marketed in the United States.

## **United States Pharmaceutical Product Development Process**

In the United States, the FDA regulates pharmaceutical products under the Federal Food, Drug and Cosmetic Act, and implementing regulations. Pharmaceutical products are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with



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## [Table of Contents](#)

### [Index to Financial Statements](#)

appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable United States requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative or judicial sanctions. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning letters, product recalls, product seizures, total or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on CymaBay. The process required by the FDA before a non-biological pharmaceutical product may be marketed in the United States generally involves the following:

- Completion of preclinical laboratory tests, animal studies and formulation studies according to Good Laboratory Practices (GLP) or other applicable regulations;
- Submission to the FDA of an Investigational New Drug application (IND), which must become effective before human clinical studies may begin;
- Performance of adequate and well-controlled human clinical studies according to the FDA's current Good Clinical Practices (GCP), to establish the safety and efficacy of the proposed pharmaceutical product for its intended use;
- Submission to the FDA of a New Drug Application (NDA) for a new pharmaceutical product;
- Satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the pharmaceutical product is produced to assess compliance with the FDA's current Good Manufacturing Practice standards (cGMP), to assure that the facilities, methods and controls are adequate to preserve the pharmaceutical product's identity, strength, quality and purity;
- Potential FDA audit of the preclinical and clinical study sites that generated the data in support of the NDA; and
- FDA review and approval of the NDA.

The lengthy process of seeking required approvals and the continuing need for compliance with applicable statutes and regulations require the expenditure of substantial resources and approvals are inherently uncertain.

Before testing any compounds with potential therapeutic value in humans, the pharmaceutical product candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the pharmaceutical product candidate. These early proof-of-principle studies are done using sound scientific procedures and thorough documentation. The conduct of the single and repeat dose toxicology and toxicokinetic studies in animals must comply with federal regulations and requirements including Good Laboratory Practices. The sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA has concerns and notifies the sponsor. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical study can begin. If resolution cannot be reached within the 30-day review period, either the FDA places the IND on clinical hold or the sponsor withdraws the application. The FDA may also impose clinical holds on a pharmaceutical product candidate at any time before or during clinical studies due to safety concerns or non-compliance. Accordingly, CymaBay cannot be sure that submission of an IND will result in the FDA allowing clinical studies to begin, or that, once begun, issues will not arise that suspend or terminate such clinical study.

During the development of a new drug, sponsors are given opportunities to meet with the FDA at certain points. These points may be prior to submission of an IND, at the end of Phase 2, and before an NDA is submitted. Meetings at other times may be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date, for the FDA to provide advice, and for the sponsor and FDA to reach agreement on the next phase of development. Sponsors typically use the End-of-Phase 2 meeting to discuss their Phase 2 clinical results and present their plans for the pivotal Phase 3 clinical trial that they believe will support approval of the new drug.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

Clinical studies involve the administration of the pharmaceutical product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the clinical study sponsor's control. Clinical studies are conducted under protocols detailing, among other things, the objectives of the clinical study, dosing procedures, subject selection and exclusion criteria, how the results will be analyzed and presented and the parameters to be used to monitor subject safety. Each protocol must be submitted to the FDA as part of the IND. Clinical studies must be conducted in accordance with GCP. Further, each clinical study must be reviewed and approved by an independent institutional review board (IRB) at, or servicing, each institution at which the clinical study will be conducted. An IRB is charged with protecting the welfare and rights of study participants and considers such items as whether the risks to individuals participating in the clinical studies are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical study subject or his or her legal representative and must monitor the clinical study until completed.

Human clinical studies are typically conducted in three sequential phases that may overlap or be combined:

- Phase 1. The pharmaceutical product is initially introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion.
- Phase 2. The pharmaceutical product is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases, to determine dosage tolerance, optimal dosage and dosing schedule and to identify patient populations with specific characteristics where the pharmaceutical product may be more effective.
- Phase 3. Clinical studies are undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population at geographically dispersed clinical study sites. These clinical studies are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling. The studies must be well-controlled and usually include a control arm for comparison. One or two Phase 3 studies are required by the FDA for an NDA approval, depending on the disease severity and other available treatment options.
- Post-approval studies, or Phase 4 clinical studies, may be conducted after initial marketing approval. These studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication.
- Progress reports detailing the results of the clinical studies must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA and the investigators for serious and unexpected adverse events or any finding from tests in laboratory animals that suggests a significant risk for human subjects. Phase 1, Phase 2 and Phase 3 clinical studies may not be completed successfully within any specified period, if at all. The FDA or the sponsor or its data safety monitoring board may suspend a clinical study at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the pharmaceutical product has been associated with unexpected serious harm to patients.

Concurrent with clinical studies, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the pharmaceutical product as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the pharmaceutical product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final pharmaceutical product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the pharmaceutical product candidate does not undergo unacceptable deterioration over its shelf life.

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[Table of Contents](#)

[Index to Financial Statements](#)

**United States Review and Approval Processes**

The results of product development, preclinical studies and clinical studies, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the pharmaceutical product, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. The submission of an NDA is subject to the payment of substantial user fees; a waiver of such fees may be obtained under certain limited circumstances.

In addition, under the Pediatric Research Equity Act (PREA), an NDA or supplement to an NDA must contain data to assess the safety and effectiveness of the pharmaceutical product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any pharmaceutical product for an indication for which orphan designation has been granted.

The FDA reviews all NDAs submitted before it accepts them for filing and may request additional information rather than accepting an NDA for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act (PDUFA), the FDA has 10 months from filing in which to complete its initial review of a standard NDA and respond to the applicant, and six months from filing for a priority NDA. The FDA does not always meet its PDUFA goal dates for standard and priority NDAs. The review process and the PDUFA goal date may be extended by three months if the FDA requests or if the NDA sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

After the NDA submission is accepted for filing, the FDA reviews the NDA application to determine, among other things, whether the proposed product is safe and effective for its intended use, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality and purity. The FDA may refer applications for novel pharmaceutical products or pharmaceutical products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the pharmaceutical product approval process, the FDA also will determine whether a risk evaluation and mitigation strategy (REMS) is necessary to assure the safe use of the pharmaceutical product. If the FDA concludes that a REMS is needed, the sponsor of the NDA must submit a proposed REMS; the FDA will not approve the NDA without a REMS, if required.

Before approving an NDA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites as well as the site where the pharmaceutical product is manufactured to assure compliance with GCP and cGMP. If the FDA determines the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. In addition, the FDA will require the review and approval of product labeling.

The NDA review and approval process is lengthy and difficult and the FDA may refuse to approve an NDA if the applicable regulatory criteria are not satisfied or may require additional clinical data or other data and information. Even if such data and information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. Data obtained from clinical studies are not always conclusive and the FDA may interpret data differently than CymaBay interprets the same data. The FDA will issue a complete response letter if the agency decides not to approve the NDA. The complete response letter usually describes all of the specific deficiencies in the NDA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical studies. Additionally, the

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## [Table of Contents](#)

## [Index to Financial Statements](#)

complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the NDA, addressing all of the deficiencies identified in the letter, or withdraw the application.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. In addition, the FDA may require Phase 4 testing which involves clinical studies designed to further assess pharmaceutical product safety and effectiveness and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized.

### ***Post-Approval Requirements***

Any pharmaceutical products for which CymaBay receives FDA approvals are subject to continuing regulation by the FDA, including, among other things, record-keeping requirements, reporting of adverse experiences with the product, providing the FDA with updated safety and efficacy information, product sampling and distribution requirements, complying with certain electronic records and signature requirements and complying with FDA promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, prohibitions on promoting pharmaceutical products for uses or in patient populations that are not described in the pharmaceutical product's approved labeling (known as "off-label use"), industry-sponsored scientific and educational activities and promotional activities involving the internet. Failure to comply with FDA requirements can have negative consequences, including adverse publicity, enforcement letters from the FDA, actions by the United States Department of Justice and/or United States Department of Health and Human Services Office of Inspector General, mandated corrective advertising or communications with doctors, and civil or criminal penalties. Although physicians may prescribe legally available pharmaceutical products for off-label uses, manufacturers may not directly or indirectly market or promote such off-label uses.

CymaBay relies, and expects to continue to rely, on third parties for the production of clinical and commercial quantities of CymaBay's products. Manufacturers of CymaBay's products are required to comply with applicable FDA manufacturing requirements contained in the FDA's cGMP regulations. cGMP regulations require, among other things, quality control and quality assurance, as well as the corresponding maintenance of records and documentation. Pharmaceutical product manufacturers and other entities involved in the manufacture and distribution of approved pharmaceutical products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer or holder of an approved NDA, including withdrawal of the product from the market. In addition, changes to the manufacturing process generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

The FDA also may require post-marketing testing, known as Phase 4 testing, risk minimization action plans and surveillance to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product.

### ***U.S. Foreign Corrupt Practices Act***

The U.S. Foreign Corrupt Practices Act, or FCPA, prohibits certain individuals and entities, including CymaBay, from promising, paying, offering to pay, or authorizing the payment of anything of value to any foreign government official, directly or indirectly, to obtain or retain business or an improper advantage. The U.S. Department of Justice and the U.S. Securities and Exchange Commission, or SEC, have increased their enforcement efforts with respect to the FCPA. Violations of the FCPA may result in large civil and criminal penalties and could result in an adverse effect on a company's reputation, operations, and financial condition. A company may also face collateral consequences such as debarment and the loss of export privileges.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

### *Federal and state fraud and abuse laws*

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain business practices in the biopharmaceutical industry in recent years. These laws include anti-kickback statutes and false claims statutes. The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting, or receiving remuneration to induce or in return for purchasing, leasing, ordering, or arranging for the purchase, lease, or order of any healthcare item or service reimbursable under Medicare, Medicaid, or other federally financed healthcare programs. The term “remuneration” has been broadly interpreted to include anything of value, including for example, gifts, discounts, the furnishing of supplies or equipment, credit arrangements, payments of cash, waivers of payment, ownership interests and providing anything at less than its fair market value. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution, the exemptions and safe harbors are drawn narrowly, and CymaBay’s practices may not in all cases meet all of the criteria for statutory exemptions or safe harbor protection. Practices that involve remuneration that may be alleged to be intended to induce prescribing, purchases, or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Several courts have interpreted the statute’s intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of federal healthcare covered business, the statute has been violated. The reach of the Anti-Kickback Statute was also broadened by the Patient Protection and Affordable Health Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively the PPACA, which, among other things, amends the intent requirement of the federal Anti-Kickback Statute. Pursuant to the statutory amendment, a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act (discussed below) or the civil monetary penalties statute, which imposes penalties against any person who is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

The federal False Claims Act prohibits any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies’ marketing of the product for unapproved, and thus non-reimbursable, uses. Many states also have statutes or regulations similar to the federal Anti-Kickback Statute and False Claims Act, which state laws apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer. Also, the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, created new federal criminal statutes that prohibit knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private third-party payers and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Because of the breadth of these laws and the narrowness of the federal Anti-Kickback Statute’s safe harbors, it is possible that some of our business activities could be subject to challenge under one or more of such laws. Such a challenge could have a material adverse effect on our business, financial condition and results of operations. If CymaBay obtains FDA approval for any of our product candidates and begin commercializing those products in the United States, CymaBay’s operations may be directly, or indirectly through our customers, distributors, or other business partners, subject to various federal and state fraud and abuse laws, including, without limitation, anti-kickback statutes and false claims statutes. These laws may impact, among other things, our proposed sales, marketing and education programs. In addition, we may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology and Clinical Health Act, or HITECH, and its implementing regulations, imposes certain requirements relating to the privacy, security and

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## [Table of Contents](#)

## [Index to Financial Statements](#)

transmission of individually identifiable health information. Among other things, HITECH makes HIPAA's privacy and security standards directly applicable to "business associates"—independent contractors or agents of covered entities that receive or obtain protected health information in connection with providing a service on behalf of a covered entity. HITECH also increased the civil and criminal penalties that may be imposed against covered entities, business associates and possibly other persons, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, state laws govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

If CymaBay's operations are found to be in violation of any of the federal and state laws described above or any other governmental regulations that apply to CymaBay, CymaBay may be subject to penalties, including criminal and significant civil monetary penalties, damages, fines, imprisonment, exclusion from participation in government healthcare programs, and the curtailment or restructuring of CymaBay's operations, any of which could adversely affect CymaBay's ability to operate its business and CymaBay's results of operations. To the extent that any of CymaBay's product candidates are ultimately sold in a foreign country, we may be subject to similar foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws, and implementation of corporate compliance programs and reporting of payments or transfers of value to healthcare professionals.

### ***Patent Term Restoration and Marketing Exclusivity***

Depending upon the timing, duration and specifics of the FDA approval of the use of CymaBay's pharmaceutical product candidates, some of CymaBay's patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permits a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA plus the time between the submission date of an NDA and the approval of that application. Only one patent applicable to an approved pharmaceutical product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The United States Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, CymaBay may intend to apply for restoration of patent term for one of its currently owned or licensed patents to add patent life beyond its current expiration date, depending upon the expected length of the clinical studies and other factors involved in the filing of the relevant NDA.

Market exclusivity provisions under the U.S. Food, Drug, and Cosmetic Act can also delay the submission or the approval of certain applications of other companies seeking to reference another company's NDA. Currently seven years of reference product exclusivity are available to pharmaceutical products designated as Orphan Drugs, during which the FDA may not approve generic products relying upon the reference product's data. Pediatric exclusivity is another type of regulatory market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric clinical study in accordance with an FDA-issued "Written Request" for such a clinical study.

### ***Pharmaceutical Coverage, Pricing and Reimbursement***

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical product candidates for which CymaBay obtains regulatory approval. In the United States and markets in other countries, sales of any products for which CymaBay receives regulatory approval for commercial sale will depend in part upon the availability of reimbursement from third-party payors. Third-party payors include government payors

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## [Table of Contents](#)

## [Index to Financial Statements](#)

such as Medicare and Medicaid, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a pharmaceutical product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the pharmaceutical product. Third-party payors may limit coverage to specific pharmaceutical products on an approved list, or formulary, which might not include all of the FDA-approved pharmaceutical products for a particular indication.

Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. CymaBay may need to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of its products, in addition to the costs required to obtain the FDA approvals. CymaBay's pharmaceutical product candidates may not be considered medically necessary or cost-effective. A payor's decision to provide coverage for a pharmaceutical product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable CymaBay to maintain price levels sufficient to realize an appropriate return on CymaBay's investment in product development. In addition, in the United States there is a growing emphasis on comparative effectiveness research, both by private payors and by government agencies. To the extent other drugs or therapies are found to be more effective than CymaBay's products, payors may elect to cover such therapies in lieu of CymaBay's products and/or reimburse CymaBay's products at a lower rate.

In 2003, the United States government enacted legislation providing a partial prescription drug benefit for Medicare recipients, which became effective at the beginning of 2006. Government payment for some of the costs of prescription drugs may increase demand for any products for which CymaBay receives marketing approval. However, to obtain payments under this program, CymaBay would be required to sell products to Medicare recipients through prescription drug plans operating pursuant to this legislation. As part of their participation in the Medicare prescription drug program, these plans negotiate discounted prices for prescription drugs and will likely do so for CymaBay's products. Federal, state and local governments in the United States continue to consider legislation to limit the growth of health care costs, including the cost of prescription drugs. Future legislation and regulations could limit payments for pharmaceuticals such as the drug candidates that CymaBay is developing.

Different pricing and reimbursement schemes exist in other countries. The downward pressure on health care costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of any pharmaceutical product candidates for which CymaBay receives regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and CymaBay expects this will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which CymaBay receives regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In the United States and foreign jurisdictions, there have been a number of legislative and regulatory changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the United States federal and state levels that seek to reduce healthcare costs. The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, imposed new requirements for the distribution and pricing of prescription drugs for Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities which will provide coverage of outpatient prescription drugs. Part D plans include both stand-alone prescription drug benefit plans and prescription drug coverage as a supplement to Medicare Advantage plans. Unlike Medicare Part A and B, Part D coverage is not standardized. Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase

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## [Table of Contents](#)

## [Index to Financial Statements](#)

demand for CymaBay's products for which CymaBay receives marketing approval. However, any negotiated prices for CymaBay's products covered by a Part D prescription drug plan will likely be lower than the prices CymaBay might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from Medicare Part D may result in a similar reduction in payments from non-governmental payors.

The American Recovery and Reinvestment Act of 2009 provides funding for the federal government to compare the effectiveness of different treatments for the same illness. Although the results of the comparative effectiveness studies are not intended to mandate coverage policies for public or private payors, it is not clear what effect, if any, the research will have on the sales of any product, if any such product or the condition that it is intended to treat is the subject of a study. It is also possible that comparative effectiveness research demonstrating benefits in a competitor's product could adversely affect the sales of our product candidates. If third-party payors do not consider CymaBay's products to be cost-effective compared to other available therapies, they may not cover our products as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow CymaBay to sell its products on a profitable basis.

In March 2010 the PPACA was enacted, which includes measures to significantly change the way healthcare is financed by both governmental and private insurers. Among the provisions of the PPACA of importance to the pharmaceutical and biotechnology industry are the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs, that began in 2011;
- an increase in the rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13% of the average manufacturer price for branded and generic drugs, respectively;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts to negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for certain individuals with income at or below 133% of the Federal Poverty Level beginning in 2014, thereby potentially increasing manufacturers' Medicaid rebate liability;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements under the federal Open Payments program, created under Section 6002 of the PPACA and its implementing regulations, that manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) report annually to the U.S. Department of Health and Human Services, or HHS, information related to "payments or other transfers of value" made or distributed to physicians and teaching hospitals, and that applicable manufacturers and applicable group purchasing organizations report annually to HHS ownership and investment interests held by physicians and their immediate family members, with reporting to the Centers for Medicare & Medicaid Services, or CMS, required by March 31 of each calendar year;
- a requirement to annually report drug samples that manufacturers and distributors provide to physicians, effective April 1, 2012;
- expansion of health care fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;



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## [Table of Contents](#)

## [Index to Financial Statements](#)

- a licensure framework for follow-on biologic products;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research;
- creation of the Independent Payment Advisory Board which, beginning in 2014, will have authority to recommend certain changes to the Medicare program that could result in reduced payments for prescription drugs and those recommendations could have the effect of law even if Congress does not act on the recommendations; and
- establishment of a Center for Medicare Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending that began on January 1, 2011.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. In August 2011, the president signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction, or joint committee, to recommend proposals in spending reductions to Congress. The joint committee did not achieve its targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, triggering automatic reductions to several government programs. These reductions include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. In January 2013, the president signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our financial operations.

### ***International Regulation***

In addition to regulations in the United States, there are a variety of foreign regulations governing clinical studies and commercial sales and distribution of CymaBay's future product candidates. Whether or not FDA approval is obtained for a product, approval of a product must be obtained by the comparable regulatory authorities of foreign countries before clinical studies or marketing of the product can commence in those countries. The approval process varies from country to country, and the time may be longer or shorter than that required for FDA approval. The requirements governing the conduct of clinical studies, product licensing, pricing and reimbursement vary greatly from country to country. In addition, certain regulatory authorities in select countries may require CymaBay to repeat previously conducted preclinical and/or clinical studies under specific criteria for approval in their respective country which may delay and/or greatly increase the cost of approval in certain markets targeted for approval by CymaBay.

### **Employees**

As of June 30, 2014, CymaBay had 16 full-time employees, 8 of whom hold Ph.D.s and one of whom holds a Master's degree in relevant areas of expertise.

### **Properties**

Our corporate office is located in Newark, California. We entered into a lease for our corporate office in November 2013 which commenced on January 1, 2014, and continues for a period of sixty (60) months with an option to extend the lease for an additional three years. We believe that our existing facility arrangements are adequate to meet our current requirements.

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[Table of Contents](#)

[Index to Financial Statements](#)

**MANAGEMENT**

The following table sets forth information regarding CymaBay's executive officers, directors, key employees and consultants, as of June 30, 2014.

**Management Team**

<u>Name</u>	<u>Age</u>	<u>Position</u>
<i>Executive Officers &amp; Significant Employees</i>		
Harold Van Wart, Ph.D.	66	President, Chief Executive Officer & Director
Sujal Shah	40	Chief Financial Officer
Pol Boudes, Ph.D.	57	Chief Medical Officer
Charles A. McWherter, Ph.D.	59	Senior Vice President and Chief Scientific Officer
Robert L. Martin, Ph.D.	51	Vice President, Nonclinical Development and Project Management
Patrick J. O'Mara	53	Vice President, Business Development
<i>Non-Employee Directors</i>		
Louis G. Lange, M.D., Ph.D.	66	Chairman of the Board
Carl Goldfischer, M.D.	55	Director
Hari Kumar, Ph.D.	58	Director
Edward E. Penhoet, Ph.D.	73	Director
Kurt von Emster, CFA	47	Director

**Biographical Information**

*Executive Officers*

**Harold E. Van Wart, Ph.D.** has served as CymaBay's President since April 2001 and Chief Executive Officer and member of its board of directors since 2003. He served as Chief Operating Officer from December 2002 to January 2003 and Senior Vice President, Research and Development from October 2000 to December 2002. From 1999 to 2000, Dr. Van Wart was vice president and therapy area head for arthritis and fibrotic diseases at Roche Biosciences, a biopharmaceutical company. From 1992 to 1999, he was vice president and director of the institute of biochemistry and cell biology at Syntex Corporation, a biopharmaceutical company acquired by Roche Biosciences in 1994. From 1978 to 1992, Dr. Van Wart served on the faculty of Florida State University. Dr. Van Wart holds a Ph.D. from Cornell University and a B.A. from SUNY Binghamton. Dr. Van Wart has been a member of the board of directors of Conatus Pharmaceuticals since 2007. He currently also serves on the Emerging Companies and Health Section Governing Boards of the Biotechnology Industry Organization (BIO), as well as on its board of directors, and on the board of directors and executive committee at BayBio.

**Sujal Shah** joined CymaBay as Chief Financial Officer in December of 2013. Prior to that he served as a consultant and acting Chief Financial Officer since June 2012. From 2010 to 2012, Mr. Shah served as Director, Health Care Investment Banking for Citigroup Inc., where he was responsible for managing client relationships and executing strategic and financing related transactions for clients focused in life sciences. From 2004 to 2010 Mr. Shah was employed with Credit-Suisse, last serving in the capacity as Vice President, Health Care Investment Banking Group. Mr. Shah received a MBA from Carnegie Mellon University—Tepper School of Business in 2004 and a M.S. from Northwestern University in Biomedical Engineering in 1997.

**Pol Boudes, Ph.D.** joined CymaBay in April 2014 as our Chief Medical Officer. Prior to joining CymaBay, Dr. Boudes was Chief Medical Officer at Amicus Therapeutics. From 2004 to 2009, Dr. Boudes was with Berlex Laboratories (which merged with Bayer HealthCare Pharmaceuticals in 2006) where he held the position of Vice President, Global Clinical Development, Women's Health Care US. From 1990 to 2004, he held positions of increasing responsibility with Wyeth-Ayerst Research both in Philadelphia, PA and in Europe, with Hoffmann-

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## [Table of Contents](#)

## [Index to Financial Statements](#)

La Roche, and with Pasteur-Merieux Serums & Vaccines. Dr. Boudes received his M.D. from the University of Aix-Marseilles, France. He completed his internship and residency in Marseilles and in Paris, France and was an Assistant Professor of Medicine at the University of Paris. He is specialized in Endocrinology and Metabolic Diseases, Internal Medicine, and Geriatric diseases.

**Charles A. McWherter, Ph.D.** has served as our Senior Vice President and Chief Scientific Officer since July 2007. From 2003 to 2007, he served as Vice President and head of the cardiovascular therapeutics areas of Pfizer Inc., a biopharmaceutical company. From 2001 to 2003, Dr. McWherter served as Vice President of Drug Discovery at Sugen, Inc., a biopharmaceutical company acquired by Pfizer Inc. in 2003. Dr. McWherter obtained his Ph.D. from Cornell University.

### *Significant Employees*

**Robert L. Martin, Ph.D.** has served as our Vice President of Nonclinical Development and Project Management since 2008. Dr. Martin served as our Sr. Director of Preclinical Development and Project Management from 2006 to 2008 and our Director of Preclinical Development and Project Management from 2004 to 2006. From 1994 to 2004, Dr. Martin served in various positions with Roche Palo Alto, a division of F. Hoffman-La Roche Ltd. Dr. Martin obtained his Ph.D. in Biochemistry from the University of California, Davis.

**Patrick J. O'Mara** joined CymaBay in 1991 and has served CymaBay in a variety of operational and business development positions. He became Vice President for Business Development in August 2006. Before joining CymaBay, Mr. O'Mara worked at Thymax Corporation and Thomas Research Corp. Mr. O'Mara received a B.A. in Biochemistry from the University of California, Berkeley.

### *Directors*

**Louis G. Lange, M.D., Ph.D.** has been a member of our Board of Directors since November 2003 and has been chairman of the board since October 2009. Dr. Lange was elected to the Board of Directors due to his significant drug development experience and leadership roles held in various companies and academic institutions. Dr. Lange has 22 years experience in academic medicine at Harvard and Washington University, where he served as Chief of Cardiology and Professor of Medicine at Jewish Hospital from 1985-1992 and was one of the first academicians in molecular cardiology. He founded CV Therapeutics, Inc. in 1990 and as Chairman, CEO and Chief Scientific Officer, led CV Therapeutics, Inc.'s initial public offering in 1996 and the overall pipeline development and the initiatives for U.S. FDA and European EMEA approval for Ranexa®, a late sodium channel blocker. He also led the approval of Lexiscan®, an adenosine A2a receptor agonist for use in myocardial perfusion imaging studies. Dr. Lange oversaw CV Therapeutics, Inc. and its sale to Gilead Sciences Inc. in 2009 for \$1.4 billion dollars. As a member of the Board of Trustees at the University of Rochester since 1998 and as Chair of the Health Affairs committee that oversaw all of the medical operations, Dr. Lange has been part of the leadership team for strategic re-invigoration of the medical center with construction of two research buildings and recruitment of over 100 faculty members. As a member of the Board of Directors of BIO from 1999 to 2009, Dr. Lange led the largest committee of member companies for two years and was picked as one of two biotech executives to attend the ceremonies at the White House for the signing of the Bioterrorism bill in 2004. Dr. Lange has been a General Partner at Asset Management since 2009; remains a senior advisor to Gilead Sciences Inc. and serves on numerous other public and private Boards in both the non-profit and for-profit arena.

**Carl Goldfischer, M.D.** has been a member of our Board of Directors since August 2003. Dr. Goldfischer was elected to the Board of Directors as a result of Bay City Capital's investment in the company and his in-depth knowledge of the pharmaceutical industry. Dr. Goldfischer is an investment partner and managing director of Bay City Capital, serving as a member of the board of directors and executive committee, and has been with the firm since December 2000. His background includes extensive public and private investment and transaction work, as well as clinical trial development knowledge. Prior to joining Bay City Capital, Dr. Goldfischer was chief financial officer of ImClone Systems Inc. Dr. Goldfischer is a member of the board of directors for Cydan, EnteroMedics Inc, Epizyme, Inc. and PharmAkea. Dr. Goldfischer received a M.D. with honors in scientific research from Albert Einstein College of Medicine and a B.A. from Sarah Lawrence College.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

**Hari Kumar, Ph.D.** has been a member of our Board of Directors since September 2012. Dr. Kumar was elected to the Board of Directors as a result of his in depth knowledge and experience in the pharmaceutical industry. Dr. Kumar has over 25 years of pharmaceutical experience. Dr. Kumar worked at Hoffmann-La Roche Inc. serving in the capacity of research, sales and marketing, lifecycle management and finally to business development. During the period 1996 through 1999, Dr. Kumar moved to Eisai Ltd, as their European Marketing Director before returning to Roche in 1999. While with Roche, Dr. Kumar was involved in guiding cross functional teams at Roche for the Transplantation franchise which resulted in the growth of the products in the franchise to achieve billion dollar sales. Dr. Kumar also identified and partnered valuable products that have enhanced Roche's portfolio, including Isotechnika, Biotie, Biocryst and Actellion. He joined Amira Pharmaceuticals, Inc. in 2007 as its Chief Business Officer and, after Amira's acquisition by Bristol Meyer Squibb in 2011, became Chief Executive Officer of Panmira Pharmaceuticals LLC. As CEO of Panmira, Dr. Kumar has overseen the launch of the immunosuppressive, CellCept®, the Alzheimer's drug, Aricept® and gastric ulcer drug, Aciphex®. In July 2013, he was appointed Chief Executive Officer and Board Director of Adheron Therapeutics, Inc. Having trained as an immunologist at University College London where he completed his Ph.D. under the supervision of Prof N.A. Mitchison, Dr. Kumar completed a postdoctoral fellowship at Tufts New England Medical Center in Boston and another fellowship at the Marie Curie Cancer Research Centre in UK.

**Edward E. Penhoet, Ph.D.** has been a member of our Board of Directors since November 2004. Dr. Penhoet was elected to the Board of Directors as a result of Alta Partners' investment in CymaBay and his because of his in depth knowledge and experience in the pharmaceutical industry. Dr. Penhoet joined Alta Partners in 2000 as a Director and has been employed full time at Alta Partners since 2008. He currently serves on the board of directors of Immune Design Corp. and Scynexis, Inc. A co-founder of Chiron Corporation an international biopharmaceutical company specializing in vaccine and blood testing units, Dr. Penhoet served as Chiron's President and Chief Executive Officer from its formation in 1981 until April 1998. He served as Vice-Chair of the governing board of the Independent Citizens Oversight Committee for the California Institute of Regenerative Medicine (CIRM) from 2005 to 2010, and served as the President of the Gordon and Betty Moore Foundation from 2004 to 2008. Dr. Penhoet was appointed to President Obama's Council of Advisors on Science and Technology (PCAST). PCAST is an advisory group comprised of 20 of the nation's leading scientists and engineers who directly advise the President and the Executive Office of the President. For 10 years prior to founding Chiron, Dr. Penhoet was a faculty member of the Biochemistry Department of the University of California, Berkeley. Dr. Penhoet is the immediate past Dean of the School of Public Health at the University of California, Berkeley. He is a member of both the Institute of Medicine of the National Academies and the American Academy of Arts and Sciences. He has co-authored more than 50 scientific articles and papers.

**Kurt von Emster, CFA** has been a member of our Board of Directors since April 2009. Mr. von Emster was elected to the Board of Directors as a result of MPM BioEquities Master Fund LP's investment in the company and because of his in depth knowledge of the pharmaceutical industry. Mr. von Emster is a co-founder and Managing Partner of venBio LLC. He has been an institutional biotechnology and health care analyst and portfolio manager for 23 years. He is a member of the board of directors of Aurinia Pharmaceuticals Inc. and Cytos Biotechnology AG, a former member of the board of Facet Biotech Corporation (sold to Abbott Laboratories in 2010) and Somaxon Pharmaceuticals, Inc. (sold to Pernix Therapeutics Holdings, Inc. in 2013), and a former board observer of Acceleron Pharma Inc. Mr. von Emster's investment career started in 1989 at Franklin Templeton Investments where he founded and managed several health and biotechnology funds in the 1990s, each achieving a 5-star Morningstar ranking. In 2000, he was managing over \$2B in biotech and health care funds for Franklin Templeton. In 2001, Mr. von Emster became a General Partner at MPM Capital, a leading biotechnology private equity firm, and launched the MPM BioEquities Fund, a cross over public and private biotechnology hedge fund. He was the portfolio manager of this fund from inception in 2001 until his departure in 2009. He also co-founded the MPM Biogen Idec Strategic Fund during his tenure at MPM. Mr. von Emster is based in venBio's San Francisco office.

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[Table of Contents](#)

[Index to Financial Statements](#)

**Director Independence**

CymaBay's business and affairs are organized under the direction of its board of directors, which currently consists of six members. The company considers each director, other than Dr. Van Wart, to be an independent director using the standards under the rules of The NASDAQ Stock Market. The primary responsibilities of the board of directors are to provide oversight, strategic guidance, counseling and direction to the company's management. Each director shall hold office until a successor is elected and qualified or until the director resigns or is removed. Any director may be removed, with cause, by the holders of a majority of shares then entitled to vote at a meeting for the election of directors. Vacancies occurring on the board of directors will be filled by the vote of a majority of the remaining directors and may be removed, without cause, by the holders of sixty-six and two-thirds percent (66 2/3%) of the shares then entitled to vote at a meeting for the election of directors. The board of directors may, by resolution passed by a majority of the whole board of directors, designate one or more committees, each committee to consist of one or more of the directors of the corporation. In 2012, the non-executive members of the company's board of directors did not receive compensation.

The board of directors at CymaBay currently has three committees:

*Compensation Committee:*

Louis G. Lange, M.D., Ph.D.—Chairman  
Carl Goldfischer, M.D.  
Edward E. Penhoet, Ph.D.

*Audit Committee:*

Carl Goldfischer, M.D.—Chairman  
Hari Kumar, Ph.D.  
Kurt von Emster, CFA

*Nominating and Corporate Governance Committee:*

Kurt von Emster, CFA—Chairman  
Hari Kumar, Ph.D.

[Table of Contents](#)

[Index to Financial Statements](#)

**EXECUTIVE AND DIRECTOR COMPENSATION**

**Summary Compensation Table**

The following table shows information regarding the compensation earned during the fiscal years ending December 31, 2013 and 2012, by (i) our Chief Executive Officer, (ii) our Chief Financial Officer, and (iii) our Senior Vice President and Chief Scientific Officer, each of whom were serving as executive officers in 2013. The officers listed below are collectively referred to herein as the “Named Executive Officers.”

<b>Name</b>	<b>Fiscal Year</b>	<b>Salary (\$)</b>	<b>Bonus (\$)</b>	<b>Option Awards (\$)<sup>(1)(2)</sup></b>	<b>All Other Compensation (\$)</b>	<b>Total (\$)</b>
Harold Van Wart, Ph.D. President and Chief Executive Officer	2013	431,469	50,000	1,236,754	14,856 <sup>(3)</sup>	1,733,079
	2012	411,830	—	26,353	12,430 <sup>(3)</sup>	450,613
Sujal Shah <sup>(4)</sup> Chief Financial Officer	2013	13,750	—	497,025	310,900 <sup>(5)</sup>	821,675
	2012	—	—	—	—	—
Charles A. McWherter Senior Vice President and Chief Scientific Officer	2013	330,967	35,000	373,614	17,230 <sup>(3)</sup>	756,811
	2012	327,309	—	11,400	13,755 <sup>(3)</sup>	352,464

- (1) These amounts are not cash compensation, but represent the aggregate fair value of the stock option grants and incentive awards received by our Named Executive Officers. The aggregate fair value is computed in accordance with FASB ASC Topic 718. See Note 11 to our financial statements in this prospectus regarding assumptions underlying valuation of equity awards. The table above includes options granted from the 2003 Equity Incentive Plan which generally vest and are exercisable over forty-eight (48) months from the grant date and are fully vested within four years from the grant date subject to the optionee’s continued employment or service with CymaBay. The options issued under our 2003 equity incentive plan generally have a maximum term of ten years, subject to earlier termination in certain situations related to cessation of employment or service. Certain of the options issued under our 2003 equity incentive plan were amended by our Compensation Committee in December 2014 to provide that they shall have an exercise price per share equal to \$5.00 and shall have an exercisable term through December 22, 2023. The table above also includes options granted from the 2013 Equity Incentive Plan, 1/3 of which are vested and immediately exercisable upon the date of grant and the remainder which vest in equal monthly installments over forty-eight months from the date of grant, subject to optionee’s continued employment or services with CymaBay. The options issued under the 2013 Equity Incentive Plan generally have a maximum term of 10 years, subject to earlier termination in certain situations related to cessation of employment or services.
- (2) The table above also includes incentive awards issued from our 2013 Equity Incentive Plan that may be settled at the sole discretion of CymaBay, by either (1) the holder’s purchase of the number of shares of our common stock at the exercise price per share on the date of grant or (2) the holder’s receipt of a cash payment equal to the excess of the fair market value of one share of our common stock on the date of exercise over the exercise price per share on the date of grant, multiplied by the portion of the award being exercised. 1/48 of the shares subject to the incentive award shall vest and be exercisable each month as measured from the date of grant, subject to the holder’s continuous service as of such date; *provided, however*, 100% of the shares subject to the incentive awards shall accelerate and be fully exercisable immediately prior to the consummation of any change of control.
- (3) Represents health insurance, group term life insurance, accidental death and dismemberment insurance, and disability insurance premiums paid by the company.
- (4) Mr. Shah was appointed as interim Chief Financial Officer of CymaBay in October, 2014 and joined CymaBay as its Chief Financial Officer in December, 2014.
- (5) Represents amounts earned by Mr. Shah from January 2013 to December 2013 in connection with consulting services to CymaBay prior to being appointed as our Chief Financial Officer.

[Table of Contents](#)

[Index to Financial Statements](#)

**Outstanding Equity Awards at Fiscal Year-End**

The following table presents the outstanding equity awards held by each of the Named Executive Officers as of December 31, 2013. The share numbers below give retroactive effect to the reverse stock split that occurred on September 30, 2013. Stock options were granted pursuant to our 2003 Equity Incentive Plan and 2013 Equity Incentive Plan (collectively the “Plans”).

Name	Option Awards			
	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable	Option Exercise Price (\$)	Option Expiration Date
Harold Van Wart, Ph.D.	5,974 <sup>(1)</sup>	0	5.00	12/22/2023
	4,402 <sup>(2)</sup>	0	4.77	01/24/2022
	24,600 <sup>(2)</sup>	0	5.00	12/22/2023
	75,011 <sup>(3)</sup>	132,713	5.00	10/30/2023
	0 <sup>(4)</sup>	95,977	5.00	12/22/2023
Sujal Shah	31,036 <sup>(3)</sup>	62,070	5.00	12/22/2023
	0 <sup>(4)</sup>	38,917	5.00	12/22/2023
Charles A. McWherter, Ph.D.	1,886 <sup>(1)</sup>	0	5.00	12/22/2023
	1,205 <sup>(2)</sup>	1,310	4.77	01/24/2022
	5,660 <sup>(2)</sup>	0	5.00	12/22/2023
	22,544 <sup>(3)</sup>	39,886	5.00	10/30/2023
	0 <sup>(4)</sup>	31,134	5.00	12/22/2023

- (1) These options were granted from the 2003 Equity Incentive Plan. The option vests in equal monthly installments of over forty-eight (48) months, provided however, that initially, the vesting did not commence until achievement of a milestone, such that upon achievement of such milestone, the number of shares that would have vested under the option equal to the number of months between the date of grant and the date of achievement of the milestone vested and thereafter 1/48 of the shares underlying the option vest monthly thereafter subject to the optionee’s continued employment or service with CymaBay. The options generally have a maximum term of 10 years, subject to earlier termination in certain situations related to cessation of employment or service. These options were amended by our Board of Directors on December 23, 2013, to extend the term of the option for an additional 10 years.
- (2) These options were granted from the 2003 Equity Incentive Plan and vest and are exercisable in equal monthly installments over forty-eight (48) months from the grant date and are fully vested within four years from the grant date subject to the optionee’s continued employment or service with CymaBay. The options generally have a maximum term of 10 years, subject to earlier termination in certain situations related to cessation of employment or service. Certain of these options were amended by our Board of Directors on December 23, 2013, to provide that they shall have an exercise price per share equal to \$5.00 and to extend the term of the option for an additional 10 years.
- (3) These options were granted from the 2013 Equity Incentive Plan and 1/3 of the shares underlying these options are fully vested on the date of grant and the remainder vest in equal monthly installments over the following forty-eight (48) months and are fully vested within four years from the grant date subject to the optionee’s continued employment or service with CymaBay. The options generally have a maximum term of 10 years, subject to earlier termination in certain situations related to cessation of employment or service.
- (4) These incentive awards were issued from our 2013 Equity Incentive Plan and may be settled at the sole discretion of CymaBay, by either (1) the holder’s purchase of the number of shares of our common stock at the exercise price per share on the date of grant or (2) the holder’s receipt of a cash payment equal to the excess of the fair market value of one share of our common stock on the date of exercise over the exercise price per share on the date of grant, multiplied by the portion of the award being exercised. 1/48 of the

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[Table of Contents](#)

[Index to Financial Statements](#)

shares subject to the incentive award shall vest and be exercisable each month as measured from the date of grant, subject to the holder's continuous service as of such date; provided, however, 100% of the shares subject to the incentive awards shall accelerate and be fully exercisable immediately prior to the consummation of any change of control.

**Employment Contracts and Termination of Employment and Change of Control Arrangements**

**Chief Executive Officer**

CymaBay entered into an employment letter agreement with Dr. Harold Van Wart on November 21, 2013. Dr. Van Wart serves as Chief Executive Officer of the company.

**Base Salary, Bonus, Benefits:** Pursuant to the terms of his employment agreement, Dr. Van Wart earns an annual base salary of \$500,000. In addition, Dr. Van Wart is eligible to receive a bonus of up to 50% of his base salary pursuant to his participation in the company's annual bonus program. The actual amount of Dr. Van Wart's bonus will be determined by the Board of Directors in its sole discretion based upon its evaluation of Dr. Van Wart's performance, the company's performance and other considerations it deems relevant. In addition, Dr. Van Wart is entitled to participate in any employee benefit plans that the company may from time to time have in effect for its employees. Dr. Van Wart is also eligible to participate in an individual disability income protection plan. The company will reimburse Dr. Van Wart for reasonable business expenses incurred in the discharge of his duties in accordance with the general practices and policies of the company and subject to the company's annual expense budget.

**Stock Option Grant:** Pursuant to the terms of his employment agreement, Dr. Van Wart was granted stock options to purchase 349,014 shares of the company's common stock and an incentive award to purchase 95,997 shares of the company's common stock. 1/3 of the shares subject to Dr. Van Wart's stock option were vested at the time of grant, with the remaining shares vesting in 48 equal monthly installments subject to Dr. Van Wart's continuous service with the company.

**Termination:** Pursuant to the terms of his employment agreement, Dr. Van Wart entered into an at-will employment relationship with the company. Either Dr. Van Wart or the company may terminate the employment relationship at any time, with or without cause and with or without advance notice. If the company terminates Dr. Van Wart without cause and other than as a result of his death or disability, or if Dr. Van Wart resigns for good reason, Dr. Van Wart will be eligible to receive 12 months of his current base salary. In addition, Dr. Van Wart is eligible to receive his annual bonus amount as if all his performance targets have been satisfied. Base salary and bonus severance will be paid in equal installments during the 12 month period following his termination date, provided, however, that no payments will be made to Dr. Van Wart prior to the 60th day following his termination. On the first payroll date following the 60th day following Dr. Van Wart's termination, the company will pay Dr. Van Wart the severance amounts that he would have received on or prior to such date in a lump sum. Such severance amounts will be reduced by any employment or consulting arrangements obtained by Dr. Van Wart following his termination. Additionally, if Dr. Van Wart elects to continue his group health benefits under COBRA, the company will pay his premiums for COBRA coverage until the earlier of (i) the 12 months following his termination date; (ii) when Dr. Van Wart attains full-time employment or (iii) when Dr. Van Wart ceases to be eligible for COBRA. Upon termination, the vesting of Dr. Van Wart's stock options will be accelerated as to the number of shares that would have vested if Dr. Van Wart had been in service for an additional 12 months following his termination date. Dr. Van Wart's benefits are conditioned on his signing a general release of claims against the company and allowing the release to become effective within 60 days after his termination date.

**Termination for Cause, Death or Disability and Resignation for Good Reason:** If Dr. Van Wart's employment is terminated for cause or because of death or disability or in the event Dr. Van Wart resigns for good reason, he will receive only the payment of his accrued salary and vacation and such other benefits as expressly required in such event by applicable law or the terms of any applicable benefit plans.

**Change in Control:** At the close of a change in control, Dr. Van Wart's outstanding stock options will become vested and exercisable with respect to 50% of his then-unvested shares of the company's common stock.



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## [Table of Contents](#)

## [Index to Financial Statements](#)

If within 12 months following a change in control, the company or a successor corporation terminates Dr. Van Wart's employment without cause and other than as a result of his death or disability, or if Dr. Van Wart resigns for good reason, Dr. Van Wart will be eligible to receive 18 months of his current base salary. Such severance amounts will be reduced by any employment or consulting arrangements obtained by Dr. Van Wart following his termination. If Dr. Van Wart elects to continue his group health benefits under COBRA, the company will pay his premiums for COBRA coverage until the earlier of (i) the 18 months following his termination date; (ii) when Dr. Van Wart attains full-time employment or (iii) when Dr. Van Wart ceases to be eligible for COBRA. In addition, Dr. Van Wart is eligible to receive 150% of his annual bonus amount. Upon termination, Dr. Van Wart's outstanding stock options will become fully vested and exercisable with respect to the remaining 50% of his then-unvested shares of the company's common stock. Dr. Van Wart's benefits are conditioned on his signing and making effective a general release of claims against the company on or after his termination date.

### **Chief Financial Officer**

CymaBay entered into an employment letter agreement with Mr. Sujal Shah on December 6, 2013. Mr. Shah serves as Chief Financial Officer.

**Base Salary, Bonus, Benefits:** Pursuant to the terms of his employment agreement, Mr. Shah earns an annual base salary of \$330,000. In addition, Mr. Shah is eligible to receive a bonus of up to 35% of his base salary pursuant to his participation in the company's annual bonus program. The actual amount of Mr. Shah's bonus will be determined by the Board of Directors in its sole discretion based upon its evaluation of Mr. Shah's performance, the company's performance and other considerations it deems relevant. In addition, Mr. Shah is entitled to participate in any employee benefit plans that the company may from time to time have in effect for its employees. Mr. Shah is also eligible to participate in an individual disability income protection plan. The company will reimburse Mr. Shah for reasonable business expenses incurred in the discharge of his duties in accordance with the general practices and policies of the company and subject to the company's annual expense budget.

**Stock Option Grant:** Pursuant to the terms of his employment agreement, Mr. Shah was granted stock options to purchase 155,672 shares of the company's common stock and an incentive award to purchase 38,917 shares of the company's common stock. 1/3 of the shares subject to Mr. Shah's stock option were vested at the time of grant, with the remaining shares vesting in 48 equal monthly installments subject to Mr. Shah's continuous service with the company.

**Termination:** Pursuant to the terms of his employment agreement, Mr. Shah entered into an at-will employment relationship with the company. Either Mr. Shah or the company may terminate the employment relationship at any time, with or without cause and with or without advance notice. If the company terminates Mr. Shah without cause and other than as a result of his death or disability, or if Mr. Shah resigns for good reason, and provided Mr. Shah was continuously employed by the company for the one year following the execution of his employment agreement, Mr. Shah will be eligible to receive 12 months of his current base salary. In addition, Mr. Shah is eligible to receive his annual bonus amount as if all his performance targets have been satisfied, pro-rated for the number of months that have elapsed in the year in which his employment terminates, but in no event will Mr. Shah be paid a bonus pro-rated for less than 9 months. Base salary and bonus severance will be paid in equal installments during the 12 month period following his termination date, provided, however, that no payments will be made to Mr. Shah prior to the 60th day following his termination. On the first payroll date following the 60th day following Mr. Shah's termination, the company will pay Mr. Shah the severance amounts that he would have received on or prior to such date in a lump sum. Such severance amounts will be reduced by any employment or consulting arrangements obtained by Mr. Shah following his termination. Additionally, if Mr. Shah elects to continue his group health benefits under COBRA, the company will pay his premiums for COBRA coverage until the earlier of (i) the 12 months following his termination date; (ii) when Mr. Shah attains full-time employment; or (iii) when Mr. Shah ceases to be eligible for COBRA. Upon termination, the vesting of Mr. Shah's stock options will be accelerated as to the number of shares that would have vested if Mr. Shah had been in service for an additional 12 months following his termination date.

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[Table of Contents](#)

[Index to Financial Statements](#)

Mr. Shah's benefits are conditioned on his signing a general release of claims against the company and allowing the release to become effective within 60 days after his termination date.

**Termination for Cause, Death or Disability and Resignation for Good Reason:** If Mr. Shah's employment is terminated for cause or because of death or disability or in the event Mr. Shah resigns for good reason, he will receive only the payment of his accrued salary and vacation and such other benefits as expressly required in such event by applicable law or the terms of any applicable benefit plans.

**Change in Control:** At the close of a change in control, provided Mr. Shah was continuously employed by the company for the one year following the execution of his employment agreement, Mr. Shah's outstanding stock options will become vested and exercisable with respect to 50% of his then-unvested shares of the company's common stock.

If within 12 months following a change in control, the company or a successor corporation terminates Mr. Shah's employment without cause and other than as a result of his death or disability, or if Mr. Shah resigns for good reason, Mr. Shah will be eligible to receive 12 months of his current base salary. Such severance amounts will be reduced by any employment or consulting arrangements obtained by Mr. Shah following his termination. If Mr. Shah elects to continue his group health benefits under COBRA, the company will pay his premiums for COBRA coverage until the earlier of (i) the 15 months following his termination date; (ii) when Mr. Shah attains full-time employment; or (iii) when Mr. Shah ceases to be eligible for COBRA. In addition, Mr. Shah is eligible to receive 125% of his annual bonus amount. Upon termination, Mr. Shah's outstanding stock options will become fully vested and exercisable with respect to the remaining 50% of his then-unvested shares of the company's common stock. Mr. Shah's benefits are conditioned on his signing and making effective a general release of claims against the company on or after his termination date.

#### **Senior Vice President and Chief Scientific Officer**

CymaBay entered into an employment letter agreement with Dr. Charles A. McWherter on November 21, 2013. Dr. McWherter serves as Senior Vice President and Chief Scientific Officer.

**Base Salary, Bonus, Benefits:** Pursuant to the terms of his employment agreement, Dr. McWherter earns an annual base salary of \$343,000. In addition, Dr. McWherter is eligible to receive a bonus of up to 35% of his base salary pursuant to his participation in the company's annual bonus program. The actual amount of Dr. McWherter's bonus will be determined by the Board of Directors in its sole discretion based upon its evaluation of Dr. McWherter's performance, the company's performance and other considerations it deems relevant. In addition, Dr. McWherter is entitled to participate in any employee benefit plans that the company may from time to time have in effect for its employees. Dr. McWherter is also eligible to participate in an individual disability income protection plan. The company will reimburse Dr. McWherter for reasonable business expenses incurred in the discharge of his duties in accordance with the general practices and policies of the company and subject to the company's annual expense budget.

**Stock Option Grant:** Pursuant to the terms of his employment agreement, Dr. McWherter was granted stock options to purchase 114,476 shares of the company's common stock and an incentive award to purchase 31,134 shares of the company's common stock. 1/3 of the shares subject to Dr. McWherter's stock option were vested at the time of grant, with the remaining shares vesting in 48 equal monthly installments subject to Dr. McWherter's continuous service with the company.

**Termination:** Pursuant to the terms of his employment agreement, Dr. McWherter entered into an at-will employment relationship with the company. Either Dr. McWherter or the company may terminate the employment relationship at any time, with or without cause and with or without advance notice. If the company terminates Dr. McWherter without cause and other than as a result of his death or disability, or if Dr. McWherter resigns for good reason, Dr. McWherter will be eligible to receive 12 months of his current base salary. In addition, Dr. McWherter is eligible to receive his annual bonus amount as if all his performance targets have been satisfied, pro-rated for the number of months that have elapsed in the year in which his employment terminates, but in no event will Dr. McWherter be paid a bonus pro-rated for less than 9 months. Base salary and bonus severance will be paid in equal installments during the 12 month period following his termination date.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

provided, however, that no payments will be made to Dr. McWherter prior to the 60th day following his termination. On the first payroll date following the 60th day following Dr. McWherter's termination, the company will pay him the severance amounts that he would have received on or prior to such date in a lump sum. Such severance amounts will be reduced by any employment or consulting arrangements obtained by Dr. McWherter following his termination. Additionally, if Dr. McWherter elects to continue his group health benefits under COBRA, the company will pay his premiums for COBRA coverage until the earlier of (i) the 12 months following his termination date; (ii) when Dr. McWherter attains full-time employment; or (iii) when Dr. McWherter ceases to be eligible for COBRA. Upon termination, the vesting of Dr. McWherter's stock options will be accelerated as to the number of shares that would have vested if Dr. McWherter had been in service for an additional 12 months following his termination date. Dr. McWherter's benefits are conditioned on his signing a general release of claims against the company and allowing the release to become effective within 60 days after his termination date.

**Termination for Cause, Death or Disability and Resignation for Good Reason:** If Dr. McWherter's employment is terminated for cause or because of death or disability or in the event Dr. McWherter resigns for good reason, he will receive only the payment of his accrued salary and vacation and such other benefits as expressly required in such event by applicable law or the terms of any applicable benefit plans.

**Change in Control:** At the close of a change in control, Dr. McWherter's outstanding stock options will become vested and exercisable with respect to 50% of his then-unvested shares of the company's common stock.

If within 12 months following a change in control, the company or a successor corporation terminates Dr. McWherter's employment without cause and other than as a result of his death or disability, or if Dr. McWherter resigns for good reason, Dr. McWherter will be eligible to receive 12 months of his current base salary. Such severance amounts will be reduced by any employment or consulting arrangements obtained by Dr. McWherter following his termination. If Dr. McWherter elects to continue his group health benefits under COBRA, the company will pay his premiums for COBRA coverage until the earlier of (i) the 15 months following his termination date; (ii) when Dr. McWherter attains full-time employment; or (iii) when Dr. McWherter ceases to be eligible for COBRA. In addition, Dr. McWherter is eligible to receive 125% of his annual bonus amount. Upon termination, Dr. McWherter's outstanding stock options will become fully vested and exercisable with respect to the remaining 50% of his then-unvested shares of the company's common stock. Dr. McWherter's benefits are conditioned on his signing and making effective a general release of claims against the company on or after his termination date.

For the purpose of the employee agreements summarized above:

"Cause" means: (i) conviction of, or plea of no contest, with respect to, any felony or any crime involving fraud, dishonesty or moral turpitude; (ii) participation in a fraud or act of dishonesty that results in material harm to the company; (iii) intentional material violation of any contract or agreement between the executive and the company, including but not limited to the executive's employment agreement or Employee Agreement on Confidential Information and Inventions, or the executive's violation of any statutory duty that he owes to the company, but only if the executive does not correct any such violation within 30 days after written notice has been provided to the executive; or (iv) gross negligence or willful neglect of the executive's job duties, as determined by the Board of Directors in good faith, but only if the executive does not correct such violation within 30 days after written notice has been provided to the executive (if such notice is reasonably practicable).

"Good reason" means: (i) the material reduction in responsibilities, authorities or functions as an employee of the company; (ii) a material reduction in level of compensation; (iii) a relocation material change of the executive's place of employment that results in an increase to his round trip commute of more than 20 miles; or (iv) the company's material breach of this letter agreement. Notwithstanding the foregoing, the executive must provide written notice to the general counsel of the company within 30 days after the date on which such event first occurs, and allow the company 30 days during which the company may attempt to rescind or correct the matter giving rise to good reason. If the company does not rescind or correct the conduct giving rise to good reason to the executive's reasonable satisfaction by the expiration of such period, the executive's employment will then terminate with good reason as of such thirtieth day.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

“Change in control” means an event or a series of related events such as: (i) the direct or indirect sale or exchange in a single or series of related transactions by the stockholders of the company of more than 50% of the voting stock of the company; (ii) a merger or consolidation in which the company is a party; or (iii) the sale, exchange or transfer of all or substantially all of the assets of the Company. A change in control will only occur if the stockholders of the company immediately before the transaction do not retain direct or indirect beneficial ownership of more than 50% of the total combined voting power of the outstanding securities of the company.

In addition, each of the employment agreements contains a “gross up” provision, which provides that if any of the executive officer’s payments constitutes a parachute payment under Section 280G of the Internal Revenue Code of 1986, as amended (the “Code”) and is subject to the excise tax under Code Section 4999, such executive will be entitled to receive from the company an additional payment in an amount equal to (i) all excise taxes (including any interest or penalties imposed with respect to such taxes) imposed on such parachute payments (the “reimbursement payment”) and (ii) all federal, state and local income taxes, employment taxes and any excise taxes that may be imposed on the reimbursement payment.

### *Stock Options*

In August 2003, the company’s stockholders approved the 2003 Equity Incentive Plan (2003 Plan), under which shares of common stock are reserved for the granting of options, stock bonuses, and restricted stock awards by the company. These awards may be granted to employees, members of the Board of Directors, and consultants to the company. The 2003 Plan terminated in accordance with its terms on July 31, 2013 and replaced the 1993 Stock Option Plan, which had similar terms.

The 2003 Plan permits the company to (i) grant incentive stock options to directors and employees at not less than 100% of the fair value of common stock on the date of grant; (ii) grant nonqualified options to employees, directors, and consultants at not less than 85% of fair value; (iii) award stock bonuses; and (iv) grant rights to acquire restricted stock at not less than 85% of fair value. Options generally vest over a four- or five-year period and have a term of ten years. Options granted to 10% stockholders have a maximum term of five years and require an exercise price equal to at least 110% of the fair value on the date of grant. The exercise price of all options granted to date has been at least equal to the fair value of common stock on the date of grant. Restricted stock units granted in 2007 vested over a four- or five-year period, subject to certain performance conditions, and terminated on August 19, 2012.

On September 25, 2013, our stockholders approved the 2013 Equity Incentive Plan, or 2013 Plan, under which shares of our common stock are reserved for issuance pursuant to stock awards, including, but not limited to, incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, performance stock awards, and performance cash awards. An aggregate of 577,294 shares were initially reserved under the 2013 Plan for issuance pursuant to stock awards, including shares which may be returned to the share reserve under options outstanding as of September 25, 2013, under the 2003 Plan. In addition, the share reserve will automatically increase on January 1<sup>st</sup> of each year, for a period of not more than ten years, commencing on January 1, 2014, in an amount equal to 5% of the total number of shares of capital stock outstanding on December 31<sup>st</sup> of the preceding calendar year, unless the Board determines otherwise prior to December 31<sup>st</sup> of such calendar year. As of June 30, 2014, we had issued options outstanding for an aggregate of 975,238 shares of our common stock under the 2013 Plan.

In the past, our Board of Directors has determined the fair market value of our common stock based upon inputs including valuation reports prepared by third party valuation firms. Generally, our stock options granted to new hires have vested as 25% of the total number of option shares granted on the first anniversary of the award and in equal monthly installments over the ensuing 36 months, whereas subsequent grants to employees generally vest in equal monthly installments over 48 months. We have offered our Executive Officers the opportunity to purchase the unvested shares subject to their options, with the company retaining a right to repurchase from the employee any shares that remain unvested if the employee’s services with us terminate prior to the date on which the options are fully vested.

[Table of Contents](#)

[Index to Financial Statements](#)

**Director Compensation**

The following table shows for the fiscal year ended December 31, 2013, certain information with respect to the compensation of all non-employee directors of CymaBay:

<u>Name</u>	<u>Fees Earned or Paid in Cash</u> (\$)	<u>Option Awards (1) (2)(3)</u> (\$)	<u>Total (\$)</u>
Louis G. Lange, M.D., Ph.D.	19,096	153,918	173,014
Carl Goldfischer, M.D.	13,142	33,055	46,197
Hari Kumar, Ph.D.	10,447	33,055	43,501
Edward E. Penhoet, Ph.D.	9,211	33,055	42,266
Kurt von Emster, CFA	9,829	38,451	48,280
Eric Converse <sup>(4)</sup>	—	—	—
Anthony B. Evnin, Ph.D. <sup>(5)</sup>	—	—	—

- (1) These amounts are not cash compensation, but rather the aggregate fair value of the equity compensation paid to our non-employee directors during the fiscal year. The aggregate fair value is computed in accordance with FASB ASC Topic 718. See Note 11 to our financial statements contained in this prospectus regarding assumptions underlying valuation of equity awards.
- (2) Assumptions made in the valuation of stock options granted are discussed in Note 11 to CymaBay's financial statements. Reflects the aggregate grant date fair value computed in accordance with ASC 718. Each director received only one option grant award in 2013, the fair market value of which is reflected in the table.
- (3) Includes incentive awards issued from our 2013 Equity Incentive Plan that may be settled at the sole discretion of CymaBay, by either (1) the holder's purchase of the number of shares of the Company's common stock at the exercise price per share on the date of grant or (2) the holder's receipt of a cash payment equal to the excess of the fair market value of one share of the Company's common stock on the date of exercise over the exercise price per share on the date of grant multiplied by the portion of the award being exercised.
- (4) Mr. Converse resigned from the Board of Directors effective September 24, 2013.
- (5) Dr. Evnin resigned from the Board of Directors effective September 26, 2013.

At December 31, 2013, the following non-employee directors held options and incentive awards to purchase the following number of shares:

<u>Name</u>	<u>Options</u>	<u>Incentive Awards</u>
Louis G. Lange, M.D., Ph.D.	30,631	12,972
Carl Goldfischer, M.D.	6,470	2,335
Edward E. Penhoet, Ph.D.	6,470	2,335
Hari Kumar, Ph.D.	6,470	2,335
Kurt von Emster, CFA	8,243	3,372
Eric Converse	0	0
Anthony B. Evnin, Ph.D.	0	0

**Non-Employee Director Compensation Policy**

In October 2013, our Board adopted a Non-Employee Director Compensation Program intended to compensate our non-employee directors with a combination of cash and equity. Each non-employee director will receive an annual base cash retainer of \$35,000 for such service. The chairman of our board of directors will receive an additional annual base cash retainer of \$5,000 for this service. In addition, we intend to compensate the members of our board of directors for service on our committees as follows:

- The chairperson of our audit committee will receive an annual cash retainer of \$17,500 for this service, and each of the other members of the audit committee will receive an annual cash retainer of \$7,750.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

- The chairperson of our compensation committee will receive an annual cash retainer of \$10,000 for such service, and each of the other members of the compensation committee will receive an annual cash retainer of \$6,000.
- The chairperson of our nominating and corporate governance committee will receive an annual cash retainer of \$8,750 for this service, and each of the other members of the nominating and corporate governance committee will receive an annual cash retainer of \$3,750.

Cash payments described above shall be paid either quarterly or semi-annually at the discretion of the board member. Further, at our first regularly scheduled meeting of the Board in the first quarter each year, our non-employee directors will receive an additional equity award of an option to purchase shares of our common stock equal to 0.035% of our outstanding stock on the date of grant. If a new board member joins our board of directors, the director will receive an initial stock option to purchase shares of our common stock equal to 0.057% of our outstanding stock on the date of grant. Annual option grants and option grants to new board members will vest will be subject to vesting as determined by our Compensation Committee on the date of grant.

## TRANSACTIONS WITH RELATED PERSONS

### Related Party Transactions

There have been no transactions since January 1, 2011, to which we have been a party, in which the amount involved exceeded or will exceed \$120,000, and in which any of our directors, executive officers or beneficial owners of more than 5% of our preferred stock or common stock, or an affiliate or immediate family member thereof, had or will have a direct or indirect material interest, other than compensation, termination and change-in-control arrangements, which are described under “Executive Compensation” and under “2013 Financing” below.

#### *Related Party Transactions Policies and Procedures.*

Our Related Person Transaction Policy, adopted by our Board of Directors on October 9, 2013, requires advance approval of all related person transactions. Our policy requires directors and executive officers and any of their affiliates and members of their immediate families to inform our management prior to consummating or becoming aware of any related party transactions. We conduct a review of all related party transactions for potential conflicts of interest. Any potential conflicts of interest must be reviewed and ratified, if applicable, by the Audit Committee and or another independent body of our Board.

#### *2013 Financing*

On September 30, 2013, CymaBay issued: (a) 374,999 shares of its common stock and warrants exercisable for 74,998 shares of its common stock to entities affiliated with Alta BioPharma for an aggregate purchase price of \$1,874,995 (Ed Penhoet is a director of CymaBay and is affiliated with the Alta BioPharma entities); (b) 10,000 shares of its common stock and warrants exercisable for 2,000 shares of its common stock to The Konrad Hans von Emster III and Elizabeth F. von Emster Revocable Trust dated January 18, 2005 (the “von Emster Trust”) for an aggregate purchase price of \$50,000 (Kurt von Emster is a director of CymaBay and affiliated with the von Emster Trust); (c) 50,000 shares of its common stock and warrants exercisable for 10,000 shares of its common stock to JJDC for an aggregate purchase price of \$250,000 and 624,944 shares of its common stock to JJDC in cancellation of approximately \$16.9 million of debt; (d) 400,000 shares of its common stock and warrants exercisable for 80,000 shares of its common stock to entities affiliated with the Deerfield Funds for an aggregate purchase price of \$2,000,000; and (e) 374,999, shares of its common stock and warrants exercisable for 74,999 shares of its common stock to entities affiliated with Versant Venture Capital for an aggregate purchase price of \$1,874,995.

#### *Indemnification Agreements*

We have entered into indemnification agreements with certain of our officers and directors. The form of agreement provides that we will indemnify our directors against any and all expenses incurred by that director because of his or her status as one of our directors to the fullest extent permitted by Delaware law, our amended and restated certificate of incorporation and our amended and restated bylaws (except under certain circumstances including on account of such officer’s or director’s breach of a duty to CymaBay as determined by a final judgment or in a proceeding initiated by such person without board approval). In addition, the form agreement provides that, to the fullest extent permitted by Delaware law, we will pay for all expenses incurred by our directors, in connection with a legal proceeding.

[Table of Contents](#)

[Index to Financial Statements](#)

**PRINCIPAL STOCKHOLDERS**

The following table sets forth certain information regarding the ownership of our common stock as of June 30, 2014, by:

- each of our directors;
- each of our executive officers named in the Summary Compensation Table above;
- all of our executive officers and directors as a group; and
- all those known by us to be beneficial owners of more than five percent of our common stock.

Beneficial ownership is determined according to the rules of the SEC and generally means that a person has beneficial ownership of a security if he, she or it possesses sole or shared voting or investment power of that security, including options and warrants that are currently exercisable or exercisable within 60 days of June 30, 2014 and excluding any incentive awards held by such person. Shares of our common stock issuable pursuant to stock options and warrants are deemed outstanding for computing the percentage of the person holding such options or warrants and the percentage of any group of which the person is a member but are not deemed outstanding for computing the percentage of any other person. Except as indicated by the footnotes below, we believe, based on the information furnished to us, that the persons named in the table below have sole voting and investment power with respect to all shares of common stock shown that they beneficially own, subject to community property laws where applicable. The information does not necessarily indicate beneficial ownership for any other purpose.

Our calculation of the percentage of beneficial ownership prior to this offering is based on 10,064,826 shares of common stock outstanding as of June 30, 2014. Our calculation of the percentage of beneficial ownership after this offering is based on 14,064,826 shares of common stock outstanding immediately after the closing of this offering (assuming no exercise of the underwriters' over-allotment option to purchase additional shares of our common stock).

Except as otherwise noted below, the address for each person or entity beneficially owning 5% or more of our common stock is c/o CymaBay Therapeutics, 7999 Gateway Blvd., Suite 130, Newark, CA 94560.

Name of Beneficial Owner	Amount and Nature of Beneficial Ownership	Percentage of Shares Beneficially Owned	
		Before Offering	After Offering
Harold Van Wart <sup>(1)</sup>	192,177	1.87%	1.35%
Charles McWherter <sup>(2)</sup>	65,382	*%	*%
Sujal Shah <sup>(3)</sup>	74,806	*%	*%
Louis Lange <sup>(4)</sup>	55,499	*%	*%
Carl Goldfischer M.D. <sup>(5)</sup>	56,458	*%	*%
Hari Kumar Ph.D. <sup>(6)</sup>	9,729	*%	*%
Edward E. Penhoet Ph.D. <sup>(7)</sup>	9,729	*%	*%
Kurt von Emster <sup>(8)</sup>	33,385	*%	*%
Entities Associated With Alta BioPharma <sup>(9)</sup>	1,123,600	11.08%	7.95%
Entities Associated With Deerfield Funds <sup>(10)</sup>	593,206	5.85%	4.19%
Johnson & Johnson Development Corporation <sup>(11)</sup>	860,266	8.54%	6.11%
Entities Associated With Versant Venture Capital <sup>(12)</sup>	1,123,600	11.08%	7.95%
All directors and officers as a group (nine persons) <sup>(13)</sup>	499,331	4.76%	3.44%

\* Less than 1%.

(1) Includes 175,720 shares issuable pursuant to options and 15,999 shares issuable pursuant to incentive awards, exercisable within 60 days of June 30, 2014.



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## [Table of Contents](#)

### [Index to Financial Statements](#)

- (2) Includes 60,193 shares issuable pursuant to options and 5,189 shares issuable pursuant to incentive awards, exercisable within 60 days of June 30, 2014.
- (3) Includes 68,320 shares issuable pursuant to options and 6,486 shares issuable pursuant to incentive awards, exercisable within 60 days of June 30, 2014.
- (4) Includes 51,891 shares issuable pursuant to options and 2,162 shares issuable pursuant to incentive awards, exercisable within 60 days of June 30, 2014.
- (5) Includes 41 shares of common stock held by Bay City Capital LLC, 43,824 shares of common stock held by The Bay City Capital Fund II, L.P. and 2,864 shares of common stock held by The Bay City Capital Fund II Co-Investment Fund, L.P. (collectively the “Bay City Capital Funds”), and 9,340 shares issuable pursuant to options and 389 shares issuable pursuant to incentive awards, exercisable within 60 days of June 30, 2014. Carl Goldfischer is a managing director of Bay City Capital Funds, and has voting and investment control over shares owned by the Bay City Capital Funds, Mr. Goldfischer disclaims beneficial ownership of the shares owned by the Bay City Capital Funds, except to the extent of his pecuniary interest therein.
- (6) Includes 9,340 shares issuable pursuant to options and 389 shares issuable pursuant to incentive awards, exercisable within 60 days of June 30, 2014.
- (7) Includes 9,340 shares issuable pursuant to options and 389 shares issuable pursuant to incentive awards, exercisable within 60 days of June 30, 2014.
- (8) Consists of 17,326 shares held by The Konrad Hans von Emster III and Elizabeth F. von Emster Revocable Trust dated January 18, 2005, 5 shares held by MPM BioEquities Master Fund LP, 2,000 shares issuable upon exercise of warrants, 13,492 shares issuable pursuant to options and 562 shares issuable pursuant to incentive awards, exercisable within 60 days of June 30, 2014.
- (9) Based on a Schedule 13G filed with the SEC on February 14, 2014, Alta BioPharma Partners III, L.P. (“ABPIII”) has sole voting and dispositive control over 960,433 shares of Common Stock and warrants to purchase 68,693 shares of Common Stock, except that Alta BioPharma Management III, LLC (“ABMIII”), the general partner of ABPIII, and Farah Champs (“Champs”), and Edward Hurwitz (“Hurwitz”), and Edward Penhoet (“Penhoet”), directors of ABMIII, may be deemed to share the right to direct the voting and dispositive control over such stock. Alta BioPharma Partners III GmbH & Co. Beteiligungs KG (“ABPIIIKG”) has sole voting and dispositive control over 64,501 shares of Common Stock and warrants to purchase 4,613 shares of Common Stock, except that Alta BioPharma Management III, LLC (“ABMIII”), the managing limited partner of ABPIIIKG, Champs, Penhoet, and Hurwitz, directors of ABMIII, may be deemed to share the right to direct the voting and dispositive control over such stock. Alta Embarcadero BioPharma Partners III, LLC (“AEBPIII”) has sole voting and dispositive control over 23,668 shares of Common Stock and warrants to purchase 1,692 shares of Common Stock, except that Champs, Penhoet, and Hurwitz, managing directors of AEBPIII, may be deemed to share the right to direct the voting and dispositive control over such stock. Alta Partners III, Inc. provides investment advisory services to several venture capital funds including, ABPIII, ABPIIIKG and AEBPIII. Alta Partners III, Inc. is a venture capital firm with an office in San Francisco. Alta Partners III, Inc. is a California Corporation. ABPIII is a Delaware Limited Partnership. ABPIIIKG is a German Limited Partnership, and AEBPIII is a California Limited Liability Company. The address of the Alta BioPharma entities is: One Embarcadero Center, Suite 3700, San Francisco, CA 94111.
- (10) Based on a Schedule 13G filed with the SEC on April 8, 2014, Deerfield Funds consists of 255,071 shares of common stock and warrants exercisable for 35,920 shares of common stock held by Deerfield Special Situations International Master Fund, L.P., and 258,135 shares of common stock and warrants exercisable for 44,080 shares of common stock held by Deerfield Special Situations Fund, LP (collectively, the “Deerfield Funds”). Deerfield MGMT, L.P. (“Deerfield MGMT”) is the general partner, and Deerfield Management Company, L.P. (“Deerfield Management”) is the investment advisor, of the Deerfield Funds, James E. Flynn, president of the general partners of Deerfield MGMT and Deerfield Management, holds voting and dispositive power over the shares held by the Deerfield Funds. The address of the Deerfield Funds is 780 Third Avenue 37th Floor, New York, NY 10017.

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[Table of Contents](#)

[Index to Financial Statements](#)

- (11) Consists of 850,266 shares of common stock and warrants exercisable for 10,000 shares of common stock held by the Johnson & Johnson Development Corporation. Linda M. Vogel, Manager, Operations of Johnson & Johnson Development Corporation (“JJDC”) exercises voting and dispositive power over the shares held by JJDC. The address of JJDC is: 410 George St., New Brunswick, NJ 08901.
- (12) Based on a Schedule 13G filed with the SEC on February 13, 2014, consists of 19,358 shares of common stock and warrants exercisable for 1,384 shares of common stock held by Versant Side Fund II, L.P., 9,116 shares of common stock and warrants exercisable for 652 shares of common stock held by Versant Affiliates Fund II-A, L.P., and 1,020,127 shares of common stock and warrants exercisable for 72,963 shares of common stock held by Versant Venture Capital II, L.P. Versant Ventures II, LLC, the general partner of Versant Venture Capital II, L.P., Versant Side Fund II, L.P. and Versant Affiliates Fund II-A (collectively, the “Versant Funds”), has the authority to vote for or dispose of the CymaBay stock held by the Versant Funds. The managing directors of the general partners are Brian Atwood, Sam Colella, Ross Jaffe, Bill Link, Barbara Lubash, Don Milder, Rebecca Robertson, Charles Warden and Brad Bolzon, who share voting and signing authority with respect to the general partner. The address of The Versant Funds is: 3000 Sand Hill Rd., Building 4, Suite 210, Menlo Park, CA 94025.
- (13) Consists of shares held by each executive officer and director of CymaBay, including the shares described in footnotes 1 through 8 above.

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[Table of Contents](#)

[Index to Financial Statements](#)

## DESCRIPTION OF CAPITAL STOCK

The following description of CymaBay's capital stock does not purport to be complete and is subject in all respects to applicable Delaware law and to the provisions of CymaBay's certificate of incorporation, and bylaws, copies of which have been filed as exhibits to the Registration Statement.

### Common Stock

**Outstanding Shares.** CymaBay's certificate of incorporation provides that an aggregate of 100,000,000 shares of CymaBay common stock, par value \$0.0001 per share, are authorized for issuance. As of June 30, 2014, 10,064,826 shares of common stock and the following options, incentive awards and warrants to purchase common stock were issued and outstanding:

- 975,238 shares of CymaBay's common stock issuable upon the exercise of stock options outstanding at a weighted average exercise price of \$6.09 per share.
- 248,861 shares of CymaBay's common stock issuable upon the exercise of incentive awards outstanding at a weighted average exercise price of \$5.00 per share.
- 1,848,487 shares of CymaBay's common stock issuable upon the exercise of warrants outstanding at a weighted average exercise price of \$5.70 per share.

The following is a summary of the material rights of CymaBay's common stock as set forth in its certificate of incorporation and bylaws.

**Voting Rights.** Each holder of common stock is entitled to one vote for each share of common stock held on all matters submitted to a vote of the stockholders, including the election of directors. The certificate of incorporation and by-laws do not provide for cumulative voting rights in connection with election of directors unless, at the time of such election, CymaBay is subject to Section 2115(b) of the California General Corporation Law. The affirmative vote of holders of 66 2/3% of the voting power of all of the then-outstanding shares of capital stock, voting as a single class, will be required to amend certain provisions of our amended and restated certificate of incorporation, including provisions relating to amending our amended and restated bylaws, and removal of directors.

**Dividends.** Subject to preferences that may be applicable to any then outstanding preferred stock, the holders of outstanding shares of common stock may receive dividends, if any, as may be declared from time to time by the Board of Directors out of legally available funds. CymaBay has never issued a dividend on shares of its common stock and has no intention to do so in the future.

**Liquidation.** In the event of liquidation, dissolution or winding up of CymaBay, the assets legally available for distribution shall be distributed ratably to the holders of shares of common stock and preferred stock, subject to the satisfaction of any liquidation preference granted to the holders of any outstanding shares of preferred stock.

**Rights and Preferences.** Holders of common stock have no preemptive, conversion or subscription rights, and there are no redemption or sinking fund provisions applicable to the common stock. The rights, preferences and privileges of the holders of common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of preferred stock that CymaBay may designate and issue in the future.

**Fully Paid and Nonassessable.** All outstanding shares of common stock are fully paid and nonassessable.

### Warrants

As of June 30, 2014 we had warrants exercisable for 1,311,958 shares of our common stock (the "Financing Warrants"). The Financing Warrants are exercisable for a period of five (5) years from September 30, 2013, at an exercise price of \$5.75 per share. The exercise prices for such Financing Warrants may be adjusted in the event of any recapitalization, reclassification, exchange, or subdivision of our outstanding shares of Common Stock. In

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## [Table of Contents](#)

## [Index to Financial Statements](#)

the event we declare and pay a dividend or other distribution on the shares of our common stock, then the holder of the Financing Warrants shall be entitled to receive such dividends or distributions to the same extent as if the holder had exercised the Financing Warrant and held common stock. In the event of an acquisition or change (a "Major Transaction") of control of CymaBay, the proceeds payable to the holder of a Financing Warrant shall be determined as more completely described in Note 10 to our financial statements contained in this prospectus. Furthermore, we may be subject to liquidated damages in the event of certain "Events of Failure" including failure to deliver shares upon exercise of the Financing Warrants, failure to remove a restrictive legend from a Financing Warrant or the underlying shares, or failure to affect a transfer of a Financing Warrant. We may be subject to liquidated damages in connection with any Event of Failure in the form of cash payments or issuance of shares of common stock in connection with any such Event of Failure, each as determined by the Black-Scholes Option Pricing Model. We may be subject to additional liquidated damages in the event of certain "Events of Default" including Events of Failure that are not cured within the requisite periods or in the event we fail to provide for appropriate payments to the holders of Financing Warrants in connection with a Major Transaction. We may be subject to liquidated damages or early mandatory termination of the Financing Warrant in connection with any Event of Default in the form of cash payments or issuance of shares of common stock in full satisfaction of the Financing Warrants, each as determined by the Black-Scholes Option Pricing Model. CymaBay further issued warrants exercisable for 414,790 shares of its common stock to NSC in its capacity as placement agent in the 2013 financing under the same terms and conditions as the Financing Warrants.

On September 30, 2013, we issued warrants to purchase an aggregate of 121,739 shares of common stock to SVB and Oxford, as partial consideration for SVB and Oxford entering into a \$10,000,000 credit facility with CymaBay (the "Bank Warrants"). The Bank Warrants are exercisable for a period of ten (10) years from September 30, 2013, at an exercise price of \$5.00 per share. The exercise prices for such Bank Warrants may be adjusted in the event of any recapitalization, reclassification, exchange, or subdivision of our outstanding shares of common stock. In the event CymaBay was to declare and pay a dividend or other distribution on the shares of its common stock, then upon exercise of the Bank Warrants, the holder shall be entitled to receive, without additional cost to the holder, the total number and kind of securities and property which the holder would have received had holder owned the shares of record as of the date the dividend or distribution occurred. In the event of any merger or acquisition of CymaBay, the holder of any Bank Warrant is obligated to exercise the Bank Warrant prior to the consummation of such merger or acquisition and the Bank Warrant shall expire immediately prior to the consummation of such merger or acquisition, unless the consideration to be paid to the holders of our common stock is something other than cash or marketable securities, in which case any successor entity to CymaBay shall be obligated to assume the Bank Warrants.

### **Preferred Stock**

CymaBay's board of directors is authorized, subject to limitations prescribed by Delaware law, to issue up to 10,000,000 shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each series and to fix the designation, powers, preferences and rights of the shares of each series and any of its qualifications, limitations or restrictions. CymaBay's board of directors can also increase or decrease the number of shares of any series, but not below the number of shares of that series then outstanding, without any further vote or action by the company's stockholders. CymaBay's board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of the common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring, discouraging or preventing a change in control of CymaBay and may adversely affect the market price of CymaBay's common stock and the voting and other rights of the holders of common stock.

### **Registration Rights**

As of June 30, 2014, holders or persons who hold 9,830,095 shares of CymaBay's common stock, and holders or persons who hold warrants to purchase 1,311,958 shares of CymaBay's common stock, have the right to require CymaBay to register with the SEC the shares of common stock and the shares of common stock issuable

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## [Table of Contents](#)

## [Index to Financial Statements](#)

upon exercise of such warrants so that those shares of common stock may be publicly resold, or, in the event any such registration statement is effective, to include those shares in any registration statement CymaBay files.

*Resale Registration Statement.* Pursuant to CymaBay's Registration Rights Agreement, dated September 30, 2013, as amended, entered into in connection with the 2013 financing (the "Registration Agreement"), CymaBay was obligated to file a resale registration statement (the "Resale Registration Statement") with the SEC to register the Shares, Warrant Shares and Conversion Shares (each as defined in the Registration Agreement). CymaBay filed and caused to become effective its Resale Registration Statement on December 24, 2013. In the event CymaBay fails to keep such Resale Registration Statement effective during the period required for such registration statement, then CymaBay shall pay to each holder of such affected registrable securities liquidated damages in an amount in cash equal to 1.5% of the aggregate purchase price paid by such holder for such registrable securities required to be included in such registration statement per month, provided that the amount of such liquidated damages paid to each holder may not exceed more than 25% of the aggregate purchase price paid by such holder for such registrable securities.

*"Piggyback" Registration Rights.* If CymaBay registers any securities for public sale (other than any registration statement relating to any employee benefit plan, any corporate reorganization or stock issued upon conversion of debt securities), holders of registrable securities under the Registration Agreement shall have the right to include their shares in the registration statement in the event the Resale Registration Statement is not effective at the time of such public sale by CymaBay. The underwriters of any underwritten offering will have the right to limit the number of shares having registration rights to be included in the registration statement.

*Expenses of Registration.* CymaBay will pay all expenses relating to all registrations and piggyback registrations provided for under the terms of the Registration Agreement.

*Termination of Registration Rights.* All registration rights described above shall terminate and be of no further force and effect at such time that all holders can sell their registrable securities under Rule 144 (1) without limitations as to volume of sales, method of sale requirements or notice requirements and (2) without the requirement for us to be in compliance with the current public information requirement under Rule 144(c)(1).

### **Anti-Takeover Provisions**

Our amended and restated certificate of incorporation and amended and restated bylaws, include a number of provisions that may deter or impede hostile takeovers or changes of control or management. These provisions include:

*Issuance of undesignated preferred stock.* Our board of directors has the authority, without further action by the stockholders, to issue up to 10,000,000 shares of undesignated preferred stock with rights and preferences, including voting rights, designated from time to time by our board of directors. The existence of authorized but unissued shares of preferred stock enables our board of directors to make it more difficult or to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise.

*Board of directors vacancies.* Our amended and restated certificate of incorporation and amended and restated bylaws authorize only our board of directors to fill vacant directorships. In addition, the number of directors constituting our board of directors may be set only by resolution adopted by a majority vote of our entire board of directors. These provisions prevent a stockholder from increasing the size of our board of directors and gaining control of our board of directors by filling the resulting vacancies with its own nominees.

*Stockholder action; special meetings of stockholders.* Our amended and restated certificate of incorporation provides that our stockholders may not take action by written consent, but may only take action at annual or special meetings of our stockholders. Stockholders will not be permitted to cumulate their votes for the election of directors unless required by applicable law. Our amended and restated bylaws provide that only the chairman of our board of directors, chief executive officer or a majority of our board of directors may call special meetings of our stockholders.

*Advance notice requirements for stockholder proposals and director nominations.* Our amended and restated bylaws provide advance notice procedures for stockholders seeking to bring business before our annual

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## [Table of Contents](#)

## [Index to Financial Statements](#)

meeting of stockholders, or to nominate candidates for election as directors at our annual meeting of stockholders. Our amended and restated bylaws also specify certain requirements as to the form and content of a stockholder's notice. These provisions may make it more difficult for our stockholders to bring matters before our annual meeting of stockholders or to nominate directors at annual meetings of stockholders.

CymaBay designed these provisions to enhance the likelihood of continued stability in the composition of our board of directors and its policies, to discourage certain types of transactions that may involve an actual or threatened acquisition of us, and to reduce our vulnerability to an unsolicited acquisition proposal. We also designed these provisions to discourage certain tactics that may be used in proxy fights. However, these provisions could have the effect of discouraging others from making tender offers for our shares and, as a consequence, they may also reduce fluctuations in the market price of our shares that could result from actual or rumored takeover attempts.

### **Section 203 of the Delaware General Corporation Law**

We are subject to Section 203 of the DGCL, which prohibits a Delaware corporation from engaging in a business combination with any interested stockholder for a period of three years following the date the person became an interested stockholder, with the following exceptions:

- before such date, the board of directors of the corporation approved either the business combination or the transaction that resulted in the stockholder becoming an interested holder;
- upon completion of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction began, excluding for purposes of determining the voting stock outstanding (but not the outstanding voting stock owned by the interested stockholder) those shares owned (a) by persons who are directors and also officers and (b) pursuant to employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; and
- on or after such date, the business combination is approved by the board of directors and authorized at an annual or special meeting of the stockholders, and not by written consent, by the affirmative vote of at least 66 2/3% of the outstanding voting stock that is not owned by the interested stockholder.

In general, Section 203 of the DGCL defines business combination to include the following:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, pledge or other disposition of 10% or more of the assets of the corporation involving the interested stockholder;
- subject to certain exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- any transaction involving the corporation that has the effect of increasing the proportionate share of the stock or any class or series of the corporation beneficially owned by the interested stockholder; and
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits by or through the corporation.

Section 203 of the DGCL defines an "interested stockholder" as an entity or person who, together with the entity's or person's affiliates and associates, beneficially owns, or is an affiliate of the corporation and within three years prior to the time of determination of interested stockholder status did own, 15% or more of the outstanding voting stock of the corporation. A Delaware corporation may "opt out" of these provisions with an express provision in its certificate of incorporation. We have not opted out of these provisions, which may as a result, discourage or prevent mergers or other takeover or change of control attempts of us.

### **Transfer Agent and Registrar**

Our transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC.

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[Table of Contents](#)

[Index to Financial Statements](#)

**MATERIAL U.S. FEDERAL INCOME AND ESTATE TAX CONSIDERATIONS FOR NON-U.S. HOLDERS**

The following summary describes the material U.S. federal income and estate tax consequences of the acquisition, ownership and disposition of our common stock acquired in this offering by Non-U.S. Holders (as defined below). This discussion does not address all aspects of U.S. federal income and estate taxes and does not deal with foreign, state and local consequences that may be relevant to Non-U.S. Holders in light of their particular circumstances, nor does it address U.S. federal tax consequences other than income and estate taxes. Special rules different from those described below may apply to certain Non-U.S. Holders that are subject to special treatment under the Internal Revenue Code of 1986, as amended, or the Code, such as financial institutions, insurance companies, tax-exempt organizations, broker-dealers and traders in securities, U.S. expatriates, “controlled foreign corporations,” “passive foreign investment companies,” corporations that accumulate earnings to avoid U.S. federal income tax, persons that hold our common stock as part of a “straddle,” “hedge,” “conversion transaction,” “synthetic security” or integrated investment or other risk reduction strategy, persons subject to the alternative minimum tax or Medicare contribution tax, partnerships and other pass-through entities, and investors in such pass-through entities. Such Non-U.S. Holders are urged to consult their own tax advisors to determine the U.S. federal, state, local and other tax consequences that may be relevant to them. Furthermore, the discussion below is based upon the provisions of the Code, and Treasury regulations, rulings and judicial decisions thereunder as of the date hereof, and such authorities may be repealed, revoked or modified, perhaps retroactively, so as to result in U.S. federal income and estate tax consequences different from those discussed below. We have not requested a ruling from the U.S. Internal Revenue Service, or IRS, with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS will agree with such statements and conclusions. This discussion assumes that the Non-U.S. Holder holds our common stock as a “capital asset” within the meaning of Section 1221 of the Code (generally, property held for investment).

Persons considering the purchase of our common stock pursuant to this offering should consult their own tax advisors concerning the U.S. federal income and estate tax consequences of acquiring, owning and disposing of our common stock in light of their particular situations as well as any consequences arising under the laws of any other taxing jurisdiction, including any state, local or foreign tax consequences.

For the purposes of this discussion, a “Non-U.S. Holder” is, for U.S. federal income tax purposes, a beneficial owner of common stock that is neither a U.S. Holder, nor a partnership (or other entity treated as a partnership for U.S. federal income tax purposes regardless of its place of organization or formation). A “U.S. Holder” means a beneficial owner of our common stock that is for U.S. federal income tax purposes (a) an individual who is a citizen or resident of the U.S., (b) a corporation or other entity treated as a corporation created or organized in or under the laws of the U.S., any state thereof or the District of Columbia, (c) an estate the income of which is subject to U.S. federal income taxation regardless of its source or (d) a trust if it (1) is subject to the primary supervision of a court within the U.S. and one or more U.S. persons have the authority to control all substantial decisions of the trust or (2) has a valid election in effect under applicable U.S. Treasury regulations to be treated as a U.S. person.

**Distributions**

Subject to the discussion below, distributions, if any, made on our common stock to a Non-U.S. Holder of our common stock to the extent made out of our current or accumulated earnings and profits (as determined under U.S. federal income tax principles) generally will constitute dividends for U.S. tax purposes and will be subject to withholding tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. To obtain a reduced rate of withholding under a treaty, a Non-U.S. Holder generally will be required to provide us with a properly executed IRS Form W-8BEN, or other appropriate form, certifying the Non-U.S. Holder’s entitlement to benefits under that treaty. In the case of a Non-U.S. Holder that is an entity, Treasury Regulations and the relevant tax treaty provide rules to determine whether, for purposes of determining the applicability of a tax treaty, dividends will be treated as paid to the entity or to those holding an interest in that

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## [Table of Contents](#)

## [Index to Financial Statements](#)

entity. If a Non-U.S. Holder holds stock through a financial institution or other agent acting on the holder's behalf, the holder will be required to provide appropriate documentation to such agent. The holder's agent will then be required to provide certification to us or our paying agent, either directly or through other intermediaries. If you are eligible for a reduced rate of U.S. federal withholding tax under an income tax treaty, you may be able to obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim for a refund with the IRS.

We generally are not required to withhold tax on dividends paid to a Non-U.S. Holder that are effectively connected with the Non-U.S. Holder's conduct of a trade or business within the U.S. (and, if required by an applicable income tax treaty, are attributable to a permanent establishment that such holder maintains in the U.S.) if a properly executed IRS Form W-8ECI, stating that the dividends are so connected, is furnished to us (or, if stock is held through a financial institution or other agent, to such agent). In general, such effectively connected dividends will be subject to U.S. federal income tax, on a net income basis at the regular graduated rates. A corporate Non-U.S. Holder receiving effectively connected dividends may also be subject to an additional "branch profits tax," which is imposed, under certain circumstances, at a rate of 30% (or such lower rate as may be specified by an applicable treaty) on the corporate Non-U.S. Holder's effectively connected earnings and profits, subject to certain adjustments.

To the extent distributions on our common stock, if any, exceed our current and accumulated earnings and profits, they will first reduce the Non-U.S. Holder's adjusted basis in our common stock, but not below zero, and then will be treated as gain to the extent of any excess, and taxed in the same manner as gain realized from a sale or other disposition of common stock as described in the next section.

### **Gain on Disposition of Our Common Stock**

Subject to the discussion below regarding backup withholding and foreign accounts, a Non-U.S. Holder generally will not be subject to U.S. federal income tax with respect to gain realized on a sale or other disposition of our common stock unless (a) the gain is effectively connected with a trade or business of such holder in the U.S. (and, if required by an applicable income tax treaty, is attributable to a permanent establishment that such holder maintains in the U.S.), (b) the Non-U.S. Holder is a nonresident alien individual and is present in the U.S. for 183 or more days in the taxable year of the disposition and certain other conditions are met or (c) we are or have been a "United States real property holding corporation" within the meaning of Code Section 897(c)(2) at any time within the shorter of the five-year period preceding such disposition or such holder's holding period. In general, we would be a U.S. real property holding corporation if interests in U.S. real estate comprised (by fair market value) at least half of our business assets. We believe that we are not, and do not anticipate becoming, a U.S. real property holding corporation. Even if we are treated as a U.S. real property holding corporation, gain realized by a Non-U.S. Holder on a disposition of our common stock will not be subject to U.S. federal income tax so long as (1) the Non-U.S. Holder owned, directly, indirectly and constructively, no more than five percent of our common stock at all times within the shorter of (i) the five-year period preceding the disposition or (ii) the holder's holding period and (2) our common stock is regularly traded on an established securities market. There can be no assurance that our common stock will continue to qualify as regularly traded on an established securities market.

If you are a Non-U.S. Holder described in (a) above, you will be required to pay tax on the net gain derived from the sale at regular graduated U.S. federal income tax rates, and corporate Non-U.S. Holders described in (a) above may be subject to the additional branch profits tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. If you are an individual Non-U.S. Holder described in (b) above, you will be required to pay a flat 30% tax on the gain derived from the sale, which gain may be offset by U.S. source capital losses (even though you are not considered a resident of the U.S.).

### **Information Reporting Requirements and Backup Withholding**

Generally, we must report information to the IRS with respect to any dividends we pay on our common stock including the amount of any such dividends, the name and address of the recipient, and the amount, if any,



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## [Table of Contents](#)

## [Index to Financial Statements](#)

of tax withheld. A similar report is sent to the holder to whom any such dividends are paid. Pursuant to tax treaties or certain other agreements, the IRS may make its reports available to tax authorities in the recipient's country of residence.

Dividends paid by us (or our paying agents) to a Non-U.S. Holder may also be subject to U.S. backup withholding. U.S. backup withholding generally will not apply to a Non-U.S. Holder who provides a properly executed IRS Form W-8BEN or otherwise establishes an exemption.

Under current U.S. federal income tax law, U.S. information reporting and backup withholding requirements generally will apply to the proceeds of a disposition of our common stock effected by or through a U.S. office of any broker, U.S. or foreign, except that information reporting and such requirements may be avoided if the holder provides a properly executed IRS Form W-8BEN or otherwise meets documentary evidence requirements for establishing Non-U.S. Holder status or otherwise establishes an exemption. Generally, U.S. information reporting and backup withholding requirements will not apply to a payment of disposition proceeds to a Non-U.S. Holder where the transaction is effected outside the U.S. through a non-U.S. office of a non-U.S. broker. Information reporting and backup withholding requirements may, however, apply to a payment of disposition proceeds if the broker has actual knowledge, or reason to know, that the holder is, in fact, a U.S. person. For information reporting purposes, certain brokers with substantial U.S. ownership or operations will generally be treated in a manner similar to U.S. brokers.

Any amounts of tax withheld under the backup withholding rules may be credited against the tax liability of persons subject to backup withholding, provided that the required information is timely furnished to the IRS.

### **Foreign Accounts**

A U.S. federal withholding tax of 30% may apply on dividends on and the gross proceeds of a disposition of our common stock paid to a foreign financial institution (as specifically defined by applicable rules) unless such institution enters into an agreement with the U.S. government to withhold on certain payments and to collect and provide to the U.S. tax authorities substantial information regarding U.S. account holders of such institution (which includes certain equity holders of such institution, as well as certain account holders that are foreign entities with U.S. owners). This U.S. federal withholding tax of 30% will also apply on dividends on and the gross proceeds of a disposition of our common stock to a non-financial foreign entity unless such entity provides the withholding agent with either a certification that it does not have any substantial direct or indirect U.S. owners or provides information regarding substantial direct and indirect U.S. owners of the entity. The withholding tax described above will not apply if the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from the rules. Under certain circumstances, a Non-U.S. Holder might be eligible for refunds or credits of such taxes. Holders are encouraged to consult with their own tax advisors regarding the possible implications of these rules for their investment in our common stock.

The IRS has issued guidance providing that the withholding provisions described above will generally apply to payments of dividends made on or after July 1, 2014 and to payments of gross proceeds from a sale or other disposition of common stock on or after January 1, 2017.

### **Federal Estate Tax**

An individual Non-U.S. Holder who is treated as the owner of, or has made certain lifetime transfers of, an interest in our common stock will be required to include the value thereof in his or her gross estate for U.S. federal estate tax purposes, and may be subject to U.S. federal estate tax unless an applicable estate tax treaty provides otherwise, even though such individual was not a citizen or resident of the U.S. at the time of his or her death.

**EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS OWN TAX ADVISOR REGARDING THE TAX CONSEQUENCES OF PURCHASING, HOLDING AND DISPOSING OF OUR COMMON STOCK, INCLUDING THE CONSEQUENCES OF ANY PROPOSED CHANGE IN APPLICABLE LAW.**

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[Table of Contents](#)

[Index to Financial Statements](#)

**UNDERWRITING**

We and the underwriters for the offering named below have entered into an underwriting agreement with respect to the shares of common stock being offered. Subject to the terms and conditions of the underwriting agreement, each underwriter has severally agreed to purchase from us the number of shares of common stock set forth opposite its name below. Cowen and Company, LLC and Stifel, Nicolaus & Company, Incorporated, are the representatives of the underwriters.

<b>Underwriter</b>	<b>Number of Shares</b>
Cowen and Company, LLC	1,600,000
Stifel, Nicolaus & Company, Incorporated	1,600,000
Roth Capital Partners, LLC	400,000
National Securities Corporation	400,000
Total	4,000,000

The underwriting agreement provides that the obligations of the underwriters are subject to certain conditions precedent and that the underwriters have agreed, severally and not jointly, to purchase all of the shares of common stock sold under the underwriting agreement if any of these shares are purchased, other than those shares covered by the over-allotment option described below. If an underwriter defaults, the underwriting agreement provides that the purchase commitments of the non-defaulting underwriters may be increased or the underwriting agreement may be terminated.

We have agreed to indemnify the underwriters against specified liabilities, including liabilities under the Securities Act, and to contribute to payments the underwriters may be required to make in respect thereof.

The underwriters are offering the shares of common stock, subject to prior sale, when, as and if issued to and accepted by them, subject to approval of legal matters by their counsel and other conditions specified in the underwriting agreement. The underwriters reserve the right to withdraw, cancel or modify offers to the public and to reject orders in whole or in part.

**Over-allotment Option to Purchase Additional Shares**

We have granted to the underwriters an option to purchase up to 600,000 additional shares of common stock at the public offering price, less the underwriting discount. This option is exercisable for a period of 30 days. The underwriters may exercise this option solely for the purpose of covering over-allotments, if any, made in connection with the sale of shares offered hereby. To the extent that the underwriters exercise this option, the underwriters will purchase additional shares from us in approximately the same proportion as shown in the table following the first paragraph of this section.

**Discount and Commissions**

The following table shows the public offering price, underwriting discount and proceeds, before expenses, to us. These amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase additional shares of common stock.

We estimate that our total expenses of the offering, excluding the underwriting discount and the advisory fees, will be approximately \$725,000 and are payable by us, which includes \$50,000 for reasonable and documented out-of-pocket FINRA related expenses incurred by the underwriters in connection with the offering.

We have agreed to pay National Securities Corporation, one of the underwriters in this offering, a financial advisory fee of \$66,000 and Trout Capital LLC a financial advisory fee of \$132,000. On October 31, 2013, we issued warrants to purchase 66,430 shares of our common stock at an exercise price of \$5.75 per share to National Securities Corporation as part of the commission paid for its services as placement agent in a private placement of our common stock and warrants to purchase common stock. These warrants that were issued to National Securities Corporation are deemed to be an item of value in connection with this offering pursuant to

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## [Table of Contents](#)

## [Index to Financial Statements](#)

FINRA Rule 5110(c)(2)(C). In compliance with and subject to FINRA Rule 5110(g), National Securities Corporation has agreed that such warrants shall not be sold during this offering or sold, transferred, assigned, or hypothecated, or be the subject of any hedging, short sale, derivative, put, or call transaction that would result in the effective economic disposition of the warrants for 180 days after the effective date of the registration statement on Form S-1 of which this prospectus forms a part. In addition, we have also agreed to reimburse Trout Capital LLC up to \$10,000 for all reasonable out-of-pocket expenses incurred by it in connection with serving as our financial advisor in connection with this offering.

	Per Share	Total	
		Without Over-allotment	With Over-allotment
Public offering price	\$5.50	\$ 22,000,000	\$ 25,300,000
Underwriting discount	\$0.33	\$ 1,320,000	\$ 1,518,000
Proceeds, before expenses, to us	\$5.17	\$ 20,680,000	\$ 23,782,000

Until the earlier of the day (i) that persons or entities, placed by National Securities Corporation, who purchased securities in CymaBay's private placements on September 30, 2013 and October 31, 2013 collectively beneficially own less than 15% of the outstanding shares of common stock or (ii) that is three years from the commencement of sales in this offering, National Securities Corporation has a right of first refusal to act as a placement agent or underwriter in certain public offerings of CymaBay's securities. Pursuant to the requirements for FINRA, CymaBay has the right to terminate the engagement of National Securities Corporation for cause if National Securities Corporation materially fails to provide certain underwriting services agreed to with CymaBay, in which case the right of first refusal would also be terminated.

The underwriters propose to offer the shares of common stock to the public at the public offering price set forth on the cover of this prospectus. The underwriters may offer the shares to securities dealers at the public offering price less a concession not in excess of \$0.198 per share. If all of the shares are not sold at the public offering price, the underwriters may change the offering price and other selling terms.

### **Discretionary Accounts**

The underwriters do not intend to confirm sales of the shares of common stock to any accounts over which they have discretionary authority.

### **Market Information**

Our common stock is currently listed on the NASDAQ Capital Market under the symbol "CBAY".

### **Price Stabilization, Short Positions and Penalty Bids**

*Stabilization.* In connection with this offering, the underwriters may engage in stabilizing transactions, over-allotment transactions, syndicate covering transactions, penalty bids and purchases to cover positions created by short sales.

- Stabilizing transactions permit bids to purchase shares of our common stock so long as the stabilizing bids do not exceed a specified maximum, and are engaged in for the purpose of preventing or retarding a decline in the market price of our common stock while the offering is in progress.
- Over-allotment transactions involve sales by the underwriters of shares of our common stock in excess of the number of shares the underwriters are obligated to purchase. This creates a syndicate short position which may be either a covered short position or a naked short position. In a covered short position, the number of shares over-allotted by the underwriters is not greater than the number of shares that it may purchase in the over-allotment option. In a naked short position, the number of shares involved is greater than the number of shares in the over-allotment option. The underwriters may close out any short position by exercising the over-allotment option and/or purchasing shares of common stock in the open market.
- Syndicate covering transactions involve purchases of shares of our common stock in the open market after the distribution has been completed in order to cover syndicate short positions. In determining the

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## [Table of Contents](#)

## [Index to Financial Statements](#)

source of shares of common stock to close out the short position, the underwriter will consider, among other things, the price of shares available for purchase in the open market as compared with the price at which they may purchase shares through exercise of the over-allotment option. If the underwriter sells more shares than could be covered by exercise of the over-allotment option and, therefore, have a naked short position, the position can be closed out only by buying securities in the open market. A naked short position is more likely to be created if the underwriter is concerned that after pricing there could be downward pressure on the price of our common stock in the open market that could adversely affect investors who purchase in the offering.

- Penalty bids permit the underwriters to reclaim a selling concession from a syndicate member when the shares of common stock originally sold by that syndicate member are purchased in stabilizing or syndicate covering transactions to cover syndicate short positions.

These stabilizing transactions, syndicate covering transactions and penalty bids may have the effect of raising or maintaining the market price of our common stock or preventing or retarding a decline in the market price of our common stock. As a result, the price of our common stock in the open market may be higher than it would otherwise be in the absence of these transactions. Neither we nor the underwriters make any representation or prediction as to the effect that the transactions described above may have on the price of shares of our common stock. These transactions may be effected on the NASDAQ Capital Market, in the over-the-counter market or otherwise and, if commenced, may be discontinued at any time.

### **Passive Market Making**

In connection with this offering, the underwriters may engage in passive market making transactions in our common stock on the NASDAQ Capital Market in accordance with Rule 103 of Regulation M under the Securities Exchange Act of 1934, as amended, or the Exchange Act, during a period before the commencement of offers or sales of shares and extending through the completion of the distribution. A passive market maker must display its bid at a price not in excess of the highest independent bid of that security. However, if all independent bids are lowered below the passive market maker's bid, that bid must then be lowered when specified purchase limits are exceeded.

### **Lock-Up Agreements**

Pursuant to certain "lock-up" agreements, we and our executive officers and directors and certain stockholders have agreed, subject to certain exceptions, not to offer, sell, assign, transfer, pledge, contract to sell, or otherwise dispose of or announce the intention to otherwise dispose of, or enter into any swap, hedge or similar agreement or arrangement that transfers, in whole or in part, the economic consequence of ownership of, directly or indirectly, or make any demand or request or exercise any right with respect to the registration of, or file with the SEC a registration statement under the Securities Act relating to, any common stock or securities convertible into or exchangeable or exercisable for any common stock without the prior written consent of both of the representatives of the underwriters, for a period of 90 days after the date of the pricing of the offering.

This lock-up provision applies to common stock and to securities convertible into or exchangeable or exercisable for common stock. It also applies to common stock owned now or acquired later by the person executing the agreement or for which the person executing the agreement later acquires the power of disposition. The exceptions to the lock-up for executive officers, directors and stockholders subject to the lock-up include: (a) transfers made as a bona fide gift to an immediate family member, to a trust the beneficiaries of which are exclusively the executive officer, director or stockholder or immediate family member, or to a charity or educational institution; (b) transfers made by will or intestate succession; (c) transfers not for value to a shareholder, partner, member or similar equity owner of, or business entity that is an affiliate of, a similar equity interest in, a stockholder that is an entity or to any trustor or beneficiary of a stockholder that is a trust; (d) the exercise of any stock options held by officers or directors issued pursuant to our existing equity incentive plans or the exercise of any warrant issued by the Company and held by such officer, director or stockholder prior to the date of this offering; and (e) the execution of a written plan meeting the requirements of Rule 10b5-1 under the Securities Exchange Act of 1934.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

### **Electronic Offer, Sale and Distribution of Shares**

A prospectus in electronic format may be made available on the websites maintained by one or more of the underwriters or selling group members, if any, participating in this offering and one or more of the underwriters participating in this offering may distribute prospectuses electronically. The representatives may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the underwriters and selling group members that will make Internet distributions on the same basis as other allocations. Other than the prospectus in electronic format, the information on these websites is not part of this prospectus or the registration statement of which this prospectus forms a part, has not been approved or endorsed by us or any underwriter in its capacity as underwriter, and should not be relied upon by investors.

### **Other Relationships**

Certain of the underwriters and their affiliates have provided, and may in the future provide, various investment banking, commercial banking and other financial services for us and our affiliates for which they have received, and may in the future receive, customary fees.

### **Selling Restrictions**

No action has been taken in any jurisdiction except the United States that would permit a public offering of our common stock, or the possession, circulation or distribution of this prospectus or any other material relating to us or our common stock in any jurisdiction where action for that purpose is required. Accordingly, the shares may not be offered or sold, directly or indirectly, and neither this prospectus nor any other offering material or advertisements in connection with the shares may be distributed or published, in or from any country or jurisdiction except in compliance with any applicable rules and regulations of any such country or jurisdiction.

*United Kingdom.* The underwriters have represented and agreed that:

- it has not made or will not make an offer of the securities to the public in the United Kingdom within the meaning of section 102B of the Financial Services and Markets Act 2000 (as amended), or the FSMA, except to legal entities which are authorized or regulated to operate in the financial markets or, if not so authorized or regulated, whose corporate purpose is solely to invest in securities or otherwise in circumstances which do not require the publication by us of a prospectus pursuant to the Prospectus Rules of the Financial Services Authority, or FSA;
- it has only communicated or caused to be communicated and will only communicate or cause to be communicated an invitation or inducement to engage in investment activity (within the meaning of section 21 of FSMA) to persons who have professional experience in matters relating to investments falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 or in circumstances in which section 21 of FSMA does not apply to us; and
- it has complied with and will comply with all applicable provisions of FSMA with respect to anything done by it in relation to the securities in, from or otherwise involving the United Kingdom.

*Switzerland.* The securities will not be offered, directly or indirectly, to the public in Switzerland and this prospectus does not constitute a public offering prospectus as that term is understood pursuant to article 652a or 1156 of the Swiss Federal Code of Obligations.

*European Economic Area.* In relation to each Member State of the European Economic Area (Iceland, Norway and Lichtenstein in addition to the member states of the European Union) that has implemented the Prospectus Directive (each, a Relevant Member State), the underwriter has represented and agreed that with effect from and including the date on which the Prospectus Directive is implemented in that Relevant Member State, or the Relevant Implementation Date, it has not made and will not make an offer of the securities to the public in that Relevant Member State prior to the publication of a prospectus in relation to the securities that has been approved by the competent authority in that Relevant Member State or, where appropriate, approved in another Relevant Member State and notified to the competent authority in that Relevant Member State, all in

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## [Table of Contents](#)

### [Index to Financial Statements](#)

accordance with the Prospectus Directive, except that it may, with effect from and including the Relevant Implementation Date, make an offer of the securities to the public in that Relevant Member State at any time:

- to legal entities which are authorized or regulated to operate in the financial markets or, if not so authorized or regulated, whose corporate purpose is solely to invest in securities;
- to any legal entity which has two or more of (1) an average of at least 250 employees during the last financial year; (2) a total balance sheet of more than €43,000,000 and (3) an annual net turnover of more than €50,000,000, as shown in its last annual or consolidated accounts; and
- in any other circumstances which do not require the publication by the issuer of a prospectus pursuant to Article 3 of the Prospectus Directive.

Each person in a Relevant Member State who receives any communication in respect of, or who acquires any securities under, the offer contemplated in this prospectus will be deemed to have represented, warranted and agreed to and with us and the underwriter that:

- it is a qualified investor within the meaning of the law in that Relevant Member State implementing Article 2(1)(e) of the Prospectus Directive; and
- in the case of any securities acquired by it as a financial intermediary, as that term is used in Article 3(2) of the Prospectus Directive, (1) the securities acquired by it in the offer have not been acquired on behalf of, nor have they been acquired with a view to their offer or resale to, persons in any Relevant Member State other than qualified investors, as that term is defined in the Prospectus Directive, or in circumstances in which the prior consent of the underwriter has been given to the offer or resale; or (2) where securities have been acquired by it on behalf of persons in any Relevant Member State other than qualified investors, the offer of those securities to it is not treated under the Prospectus Directive as having been made to such persons.

For the purposes of the provisions in the two immediately preceding paragraphs, the expression an “offer of the securities to the public” in relation to the securities in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the securities to be offered so as to enable an investor to decide to purchase or subscribe for the securities, as the same may be varied in that Relevant Member State by any measure implementing the Prospectus Directive in that Relevant Member State, and the expression “Prospectus Directive” means Directive 2003/71/EC and includes any relevant implementing measure in each Relevant Member State.

*United Arab Emirates.* This document has not been reviewed, approved or licensed by the Central Bank of the United Arab Emirates, or UAE, Emirates Securities and Commodities Authority or any other relevant licensing authority in the UAE including any licensing authority incorporated under the laws and regulations of any of the free zones established and operating in the territory of the UAE, in particular the Dubai International Financial Services Authority, or DFSA, a regulatory authority of the Dubai International Financial Centre, or DIFC. The issue of shares of common stock does not constitute a public offer of securities in the UAE, DIFC and/or any other free zone in accordance with the Commercial Companies law, Federal Law No. 8 of 1984 (as amended), DFSA Offered Securities Rules and the Dubai International Financial Exchange Listing Rules, accordingly or otherwise.

The shares may not be offered to the public in the UAE and/or any of the free zones including, in particular, the DIFC. The shares may be offered and this document may be issued, only to a limited number of investors in the UAE or any of its free zones (including, in particular, the DIFC) who qualify as sophisticated investors under the relevant laws and regulations of the UAE or the free zone concerned. Management of the company and the representatives of the underwriters represent and warrant the shares will not be offered, sold, transferred or delivered to the public in the UAE or any of its free zones.

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[Table of Contents](#)

[Index to Financial Statements](#)

**LEGAL MATTERS**

Cooley LLP, Palo Alto, California, will pass upon the validity of the shares of common stock offered hereby. The underwriters are being represented by Goodwin Procter LLP, New York, New York, in connection with the offering.

**EXPERTS**

The financial statements of CymaBay Therapeutics, Inc. at December 31, 2013 and 2012, and for each of the years then ended, appearing in this prospectus and registration statement have been audited by Ernst & Young LLP, an independent registered public accounting firm, as set forth in their report thereon appearing elsewhere herein, and are included in reliance upon such report given on the authority of such firm as experts in accounting and auditing.

**WHERE YOU CAN FIND ADDITIONAL INFORMATION**

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of common stock being offered by this prospectus. This prospectus does not contain all of the information in the registration statement and its exhibits. For further information about us and the common stock offered by this prospectus, we refer you to the registration statement and its exhibits. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You can read our SEC filings, including the registration statement, over the Internet at the SEC's website at <http://www.sec.gov>. You may also read and copy any document we file with the SEC at its public reference facilities at 100 F Street, NE, Washington, D.C. 20549. You may also obtain copies of these documents at prescribed rates by writing to the Public Reference Section of the SEC at 100 F Street, NE, Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the operation of the public reference facilities.

We are subject to the information reporting requirements of the Exchange Act, and are required to file reports, proxy statements and other information with the SEC. These reports, proxy statements and other information are available for inspection and copying at the public reference room and web site of the SEC referred to above. We also maintain a website at [www.cymabay.com](http://www.cymabay.com), at which you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. The information contained on, or that can be accessed through, our website is not a part of this prospectus. Investors should not rely on any such information in deciding whether to purchase our common stock. We have included our website address in this prospectus solely as an inactive textual reference.

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[Table of Contents](#)

[Index to Financial Statements](#)

**CYMABAY THERAPEUTICS, INC.  
INDEX TO FINANCIAL STATEMENTS**

	<u>Page</u>
<a href="#">Report of Independent Registered Public Accounting Firm</a>	F-2
<a href="#">Balance Sheets as of December 31, 2013 and 2012 (audited) and March 31, 2014 (unaudited)</a>	F-3
<a href="#">Statements of Operations and Comprehensive Loss for the years ended December 31, 2013 and 2012 (audited) and the three-month periods ended March 31, 2014 and 2013 (unaudited)</a>	F-4
<a href="#">Statements of Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit) for the years ended December 31, 2013 and 2012 (audited) and the three-month periods ended March 31, 2014 and 2013 (unaudited)</a>	F-5
<a href="#">Statements of Cash Flows for the years ended December 31, 2013 and 2012 (audited) and the three-month periods ended March 31, 2014 and 2013 (unaudited)</a>	F-6
<a href="#">Notes to Financial Statements</a>	F-8



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[Table of Contents](#)

[Index to Financial Statements](#)

**REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

The Board of Directors and Stockholders  
CymaBay Therapeutics, Inc.

We have audited the accompanying balance sheets of CymaBay Therapeutics, Inc. as of December 31, 2013 and 2012, and the related statements of operations and comprehensive loss, redeemable convertible preferred stock and stockholders' equity (deficit), and cash flows for each of the two years in the period ended December 31, 2013. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of CymaBay Therapeutics, Inc. at December 31, 2013 and 2012, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2012 in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

Redwood City, CA  
March 31, 2014

[Table of Contents](#)[Index to Financial Statements](#)

**CYMABAY THERAPEUTICS, INC.**  
**BALANCE SHEETS**  
(In Thousands, except share and per share amounts)

	March 31, 2014 <u>(unaudited)</u>	<u>December 31</u>	
		2013	2012
<b>Assets</b>			
Current assets:			
Cash and cash equivalents	\$ 22,342	\$ 24,401	\$ 7,726
Marketable securities	6,191	6,843	—
Contract receivables	168	110	108
Accrued interest receivable	90	68	9
Prepaid expenses	1,876	364	147
Other current assets	266	453	—
Total current assets	<u>30,933</u>	<u>32,239</u>	<u>7,990</u>
Property and equipment, net	60	3	84
Other assets	203	258	42
Total assets	<u>\$ 31,196</u>	<u>\$ 32,500</u>	<u>\$ 8,116</u>
<b>Liabilities and redeemable convertible preferred stock and stockholders' equity (deficit)</b>			
Current liabilities:			
Accounts payable	\$ 1,069	\$ 697	\$ 657
Accrued liabilities	2,368	2,251	990
Warrant liability	11,638	6,466	—
Facility loan	410	38	—
Convertible notes	—	—	13,737
Accrued interest payable	36	36	2,566
Total current liabilities	<u>15,521</u>	<u>9,488</u>	<u>17,950</u>
Facility loan, less current portion	4,099	4,407	—
Other liabilities	200	9	36
Total liabilities	<u>19,820</u>	<u>13,904</u>	<u>17,986</u>
Commitments and contingencies			
Redeemable convertible preferred stock, \$0.0001 par value: no shares authorized, issued or outstanding at March 31, 2014 and December 31, 2013; 55,258,608 shares authorized and 661,059 shares issued and outstanding at December 31, 2012; aggregate liquidation preference \$256,750 as of December 31, 2012			
	—	—	318,697
Stockholders' equity (deficit):			
Preferred stock, \$0.0001 par value: 10,000,000 shares authorized at March 31, 2014 and December 31, 2013; no shares authorized at December 31, 2012; no shares issued and outstanding			
	—	—	—
Common stock, \$0.0001 par value: 100,000,000 shares authorized; 10,064,495, 9,455,064 and 5,792 shares issued and outstanding as of March 31, 2014, December 31, 2013 and 2012, respectively			
	1	1	—
Additional paid-in capital	370,276	367,435	913
Accumulated other comprehensive income	3	2	—
Accumulated deficit	<u>(358,904)</u>	<u>(348,842)</u>	<u>(329,480)</u>
Total stockholders' equity (deficit)	<u>11,376</u>	<u>18,596</u>	<u>(328,567)</u>
Total liabilities and redeemable convertible preferred stock and stockholders' equity (deficit)	<u>\$ 31,196</u>	<u>\$ 32,500</u>	<u>\$ 8,116</u>

[Table of Contents](#)

[Index to Financial Statements](#)

**CYMABAY THERAPEUTICS, INC.**  
**STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**

(In Thousands, except share and per share information)

	Three Months Ended		Year Ended December 31,	
	March 31,		2013	2012
	2014	2013	2013	2012
	(unaudited)			
Contract revenue	\$ —	\$ —	\$ —	\$ 3,050
Operating expenses:				
Research and development	2,615	1,490	4,525	9,280
General and administrative	2,500	925	4,871	4,208
Total operating expenses	<u>5,115</u>	<u>2,415</u>	<u>9,396</u>	<u>13,488</u>
Loss from operations	(5,115)	(2,415)	(9,396)	(10,438)
Other income (expense):				
Interest income	12	1	10	22
Interest expense	(184)	(212)	(822)	(841)
Other income (expense), net	(4,775)	(2)	135	2
Net loss	<u>\$ (10,062)</u>	<u>\$ (2,628)</u>	<u>\$ (10,073)</u>	<u>\$ (11,255)</u>
Net income (loss) attributable to common stockholders	<u>\$ (10,062)</u>	<u>\$ (5,737)</u>	<u>\$ 243,994</u>	<u>\$ (23,899)</u>
Net loss	(10,062)	(2,628)	(10,073)	(11,255)
Other comprehensive loss/income:				
Unrealized gains (losses) on marketable securities	1	—	2	(2)
Other comprehensive income (loss)	1	—	2	(2)
Comprehensive loss	<u>\$ (10,061)</u>	<u>\$ (2,628)</u>	<u>\$ (10,071)</u>	<u>\$ (11,257)</u>
Basic net income (loss) per common share	<u>\$ (1.02)</u>	<u>\$ (985.06)</u>	<u>\$ 103.52</u>	<u>\$ (4,128.71)</u>
Weighted average common shares outstanding used to calculate basic net income (loss) per common share	<u>9,873,687</u>	<u>5,824</u>	<u>2,357,036</u>	<u>5,788</u>
Diluted net loss per common share	<u>\$ (1.02)</u>	<u>\$ (985.06)</u>	<u>\$ (3.54)</u>	<u>\$ (4,128.71)</u>
Weighted average common shares outstanding used to calculate diluted net loss per common share	<u>9,873,687</u>	<u>5,824</u>	<u>2,845,609</u>	<u>5,788</u>

*See accompanying notes.*

[Table of Contents](#)

[Index to Financial Statements](#)

**CYMABAY THERAPEUTICS, INC.**  
**STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)**  
(In Thousands, except share and per share information)

	Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
Balances as of December 31, 2011	661,059	\$ 306,053	5,773	\$ —	\$ 762	2	\$ (305,581)	\$ (304,817)
Discount conversion feature associated with convertible notes	—	—	—	—	70	—	—	70
Issuance of common stock upon exercise of options	—	—	19	—	—	—	—	—
Non-employee stock-based compensation expense	—	—	—	—	1	—	—	1
Employee and director stock-based compensation expense	—	—	—	—	80	—	—	80
Accretion to redemption value of redeemable convertible preferred stock	—	12,644	—	—	—	—	(12,644)	(12,644)
Net loss	—	—	—	—	—	—	(11,255)	(11,255)
Net unrealized gain on marketable securities	—	—	—	—	—	(2)	—	(2)
Balances as of December 31, 2012	661,059	\$ 318,697	5,792	\$ —	\$ 913	\$ —	\$ (329,480)	\$ (328,567)
Issuance of common stock upon exercise of options	—	—	78	—	—	—	—	—
Non-employee stock-based compensation expense	—	—	—	—	17	—	—	17
Employee and director stock-based compensation expense	—	—	—	—	866	—	—	866
Accretion to redemption value of redeemable convertible preferred stock	—	9,289	—	—	—	—	(9,289)	(9,289)
Repurchase of convertible preferred stock	(39,606)	(8,250)	—	—	8,247	—	—	8,247
Conversion of preferred stock to common stock	(621,453)	(319,736)	2,793,281	—	319,736	—	—	319,736
Issuance of common stock, net of \$5,356 issuance costs	—	—	6,030,969	1	20,711	—	—	20,712
Extinguishment of debt through issuance of common stock	—	—	624,944	—	16,945	—	—	16,945
Net loss	—	—	—	—	—	—	(10,073)	(10,073)
Net unrealized gain on marketable securities	—	—	—	—	—	2	—	2
Balances as of December 31, 2013	—	\$ —	9,455,064	\$ 1	\$ 367,435	\$ 2	\$ (348,842)	\$ 18,596
Issuance of common stock upon cashless net exercise of warrant	—	—	5,431	—	55	—	—	55
Non-employee stock-based compensation expense	—	—	—	—	2	—	—	2
Employee and director stock-based compensation expense	—	—	—	—	526	—	—	526
Issuance of common stock, net of \$762 issuance costs	—	—	604,000	—	2,258	—	—	2,258
Net loss	—	—	—	—	—	—	(10,062)	(10,062)
Net unrealized gain on marketable securities	—	—	—	—	—	1	—	1
Balances as of March 31, 2014	—	\$ —	10,064,495	\$ 1	\$ 370,276	\$ 3	\$ (358,904)	\$ 11,376

[Table of Contents](#)

[Index to Financial Statements](#)

**CYMABAY THERAPEUTICS, INC.**  
**STATEMENTS OF CASH FLOWS**  
*(In Thousands)*

	Three Months Ended		Year Ended December	
	March 31,		2013	2012
	2014	2013	2013	2012
	(unaudited)			
<b>Operating activities</b>				
Net loss	\$(10,062)	\$(2,628)	\$(10,073)	\$(11,255)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	3	26	55	119
Amortization of notes payable conversion option	—	—	10	—
Non-employee stock-based compensation expense	2	—	17	1
Employee and director stock-based compensation expense	714	18	875	80
Amortization of premium on marketable securities	71	—	48	—
Non-cash interest associated with debt discount accretion	49	10	47	60
Change in fair value of warrant liability	4,784	—	494	—
Gain on sale of property and equipment	—	—	(632)	—
Changes in assets and liabilities:				
Contract receivables	(58)	(11)	(2)	16
Accrued interest receivable	(22)	9	(59)	91
Prepaid expenses	(1,512)	20	(217)	87
Other assets	(211)	(55)	(216)	51
Accounts payable	372	20	40	(951)
Accrued liabilities	830	228	499	(291)
Accrued interest payable	26	204	692	781
Other liabilities	3	—	(36)	(82)
Net cash used in operating activities	(5,011)	(2,159)	(8,458)	(11,293)
<b>Investing activities</b>				
Purchases of property and equipment	(32)	—	—	—
Proceeds from the sale of property and equipment	—	—	658	—
Purchases of marketable securities	—	—	(6,933)	(2,881)
Proceeds from sales of marketable securities	582	—	44	13,891
Net cash (used in) provided by investing activities	550	—	(6,231)	11,010
<b>Financing activities</b>				
Proceeds from facility loan	—	—	4,853	—
Proceeds from issuance of common stock and warrants, net of issuance costs	2,402	—	26,514	—
Repurchase of preferred stock	—	—	(3)	—
Principal payments on equipment loans	—	—	—	(12)
Net cash provided by (used in) financing activities	2,402	—	31,364	(12)
Net increase(decrease) in cash and cash equivalents	(2,059)	(2,159)	16,675	(295)
Cash and cash equivalents at beginning of period	24,401	7,726	7,726	8,021
Cash and cash equivalents at end of period	<u>\$ 22,342</u>	<u>\$ 5,567</u>	<u>\$ 24,401</u>	<u>\$ 7,726</u>
<b>Supplemental disclosure of cash flow information</b>				
Interest paid	\$ 109	\$ —	\$ 74	\$ —
Financing costs in accrued expenses	—	—	309	—
Issuance of common stock for debt extinguishment	—	—	16,945	—
Issuance of common stock warrants to lenders	—	—	479	—
Issuance of common stock warrants	443	—	5,493	—

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[Table of Contents](#)

[Index to Financial Statements](#)

	Three Months Ended March 31,		Year Ended December	
	2014	2013	2013	2012
	(unaudited)			
Fair value of forward contract	—	—	453	—
Conversion of preferred stock into common stock	—	—	323,155	—
Fixed assets in accrued expenses	28	—	—	—
Issuance of common stock upon cashless warrant exercise	55	—	—	—
Noncash issuance costs incurred in common stock financing	453	—	—	—

## NOTES TO FINANCIAL STATEMENTS

(Information as of March 31, 2014 and for the three months ended March 31, 2013 and 2014 is 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 and rare diseases with high unmet need. 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.

On September 30, 2013, the Company sold shares of its common stock and warrants to purchase shares of its 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 it entered into simultaneously with the private placement on September 30, 2013, resulting in aggregate net proceeds to the Company of \$28.8 million after deducting placement agent fees and estimated offering expenses. At the same time the Company issued shares of its common stock in cancellation of approximately \$16.9 million of debt owed to the holder of that debt. On October 31, 2013, the Company sold additional shares of its common stock and warrants to purchase shares of its common stock, which sales are also part of the private placement, for net proceeds of \$2.2 million after deducting placement agent fees and estimated offering expenses. Further, on November 22, 2013, the Company entered into an agreement with investors to purchase shares of its common stock and warrants to purchase shares of its common stock as part of the private placement for net proceeds of \$2.7 million, which sales occurred on January 29, 2014, after the listing of the Company’s common stock on the OTCQB Marketplace. The Company refers to the private placement, the venture debt financing and the issuance of the Company’s common stock in cancellation of the \$16.9 million of debt as the 2013 financing.

The Company has incurred net operating losses and negative cash flows from operations since its inception. During the three months ended March 31, 2014, the Company incurred a net loss of \$10.1 million and used \$5.0 million of cash in operations. At March 31, 2014, the Company had an accumulated deficit of \$358.9 million. The Company expects to incur increased research and development expenses as it continues to study its product candidates in clinical trials.

### 2. Summary of Significant Accounting Policies

#### Basis of Presentation and Use of Estimates

The financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America (U.S. GAAP), which requires management to make informed estimates and assumptions that impact 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 determining revenue recognition and in estimating stock-based compensation, clinical trial accruals, and equity instrument valuations.

#### Unaudited Interim Financial Information

The accompanying interim consolidated financial statements are unaudited. The financial data and other information disclosed in these notes to the financial statements related to March 31, 2014 and the three month periods ended March 31, 2014 and 2013, are also unaudited. These unaudited interim financial statements have been prepared in accordance U.S. 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

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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 income (loss) and its cash flows for periods presented. The results for the three months ended March 31, 2014, are not necessarily indicative of results to be expected for the year ending December 31, 2014, or for any other interim period or for any future year.

### Reverse Stock Split

On September 30, 2013, the Company filed amended and restated certificates of incorporation under which the Company's preferred stock and common stock was reverse split on a 1-for-79.5 basis. The accompanying financial statements and notes to the financial statements, other than with respect to the authorized number of shares, give retroactive effect to the reverse split for all periods presented.

### Reclassification of Prior Period Balances

Certain reclassifications have been made to prior period amounts to conform to current-year presentation.

### Fair Value of Financial Instruments

The Company's financial instruments consist of cash and cash equivalents, short-term marketable securities, accounts payable, accrued expenses, warrant liabilities, forward contracts and convertible notes. 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 cash and cash equivalents, accounts payable, convertible notes and accrued expenses are generally considered to be representative of their respective fair values because of the short-term nature of those instruments. The following table presents the fair value of the Company's other financial assets and liabilities using the above input categories (in thousands):

Description	As of December 31, 2013			Fair Value
	Level 1	Level 2	Level 3	
Money market funds	\$21,097	\$ —	\$ —	\$ 21,097
Corporate debt and asset backed securities	—	6,843	—	6,843
Total assets measured at fair value	\$21,097	\$6,843	\$ —	\$ 27,940
Forward contract	—	—	453	453
Warrant liability	—	—	6,466	6,466
Total liabilities measured at fair value	\$ —	\$ —	\$6,919	\$ 6,919



## [Table of Contents](#)

## [Index to Financial Statements](#)

Description	As of March 31, 2014 (unaudited)			
	Level 1	Level 2	Level 3	Fair Value
Money market funds	\$21,737	\$ —	\$ —	\$ 21,737
Corporate debt and asset backed securities	—	6,191	—	6,191
Total assets measured at fair value	\$21,737	\$6,191	\$ —	\$ 27,928
Warrant liability	\$ —	\$ —	\$11,638	\$ 11,638
Total liabilities measured at fair value	\$ —	\$ —	\$11,638	\$ 11,638

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), a component of stockholders' equity (deficit). 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.

As of December 31, 2013 and March 31, 2014, the Company held a Level 3 liability associated with warrants, issued in connection with the Company's equity offerings. The warrants are considered liabilities and are valued using an option-pricing model, the significant inputs for which include 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. As of December 31, 2013, the Company also held a Level 3 liability associated with a forward contract which arose in connection with the Company's November 22, 2013 execution of an equity purchase agreement with certain investors. The agreement required the Company to issue a fixed number of shares of common stock and warrants to purchase common stock at a predetermined price of \$3.0 million provided the Company completes the listing of its common stock on a public stock exchange. The forward contract's fair value was determined upon execution as the difference between the present value of the equity proceeds to be received under the agreement less the fair value of the underlying securities. The forward contract liability is presented in the balance sheet as a component of accrued liabilities and is revalued at each reporting period until the contract is settled which occurred on January 29, 2014. The fair value of the underlying common stock and warrants were valued using an option-pricing model, the inputs of which are similar to those used in the valuation of the Company's liability classified 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 and forward contract liabilities. As of December 31, 2012, the Company had no assets or liabilities measured at fair value on a recurring basis within the Level 3 hierarchy.

The following table sets forth a summary of the changes in the fair value of our Level 3 financial instruments (in thousands):

	Warrant Liability	Forward Contract
Balance as of December 31, 2012	\$ —	\$ —
Issuance of financial instrument	5,972	453
Change in fair value	494	—
Balance as of December 31, 2013	\$ 6,466	\$ 453
Issuance of financial instrument (unaudited)	443	—
Change in fair value (unaudited)	4,784	(10)
Settlement of financial instrument (unaudited)	(55)	(443)
Balance as of March 31, 2014 (unaudited)	\$11,638	\$ —

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

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## [Table of Contents](#)

## [Index to Financial Statements](#)

### **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 which are classified in Level 1 and Level 2 of the fair value hierarchy. 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 accompanying 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 and comprehensive loss. Unrealized holding gains and losses are reported in accumulated other comprehensive 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.

### **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, 2014 and December 31, 2013 and 2012, cash restricted under these arrangements was \$100,000, \$155,000 and none, respectively. These amounts are presented in other assets on the accompanying 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 carried 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 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, 2014, December 31, 2013 and 2012.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

### **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 accompanying balance sheets.

### **Revenue Recognition**

The Company recognizes revenue when (i) persuasive evidence of an arrangement exists, (ii) delivery has occurred or services have been rendered, (iii) the price is fixed and determinable, and (iv) collectability is reasonably assured. Payments received in advance of work performed are recorded as deferred revenue and recognized when earned. All revenue recognized to date under collaboration agreements has been nonrefundable.

In 2012, contract revenue was from two strategic partners. There was no contract revenue recorded for the year ended December 31, 2013 and the three months ended March 31, 2014.

### ***Multiple Element Arrangements***

The Company evaluates revenue from agreements that have multiple elements to determine whether the components of the arrangement represent separate units of accounting. Management considers whether components of an arrangement represent separate units of accounting based upon whether certain criteria are met, including whether the delivered element has stand-alone value to the customer. To date, all of the Company's collaboration agreements have been assessed to have one unit of accounting. Up-front and license fees received for a combined unit of accounting have been deferred and recognized ratably over the projected performance period. Non-refundable fees where the Company has no continuing performance obligations have been recognized as revenue when collection is reasonably assured and all other revenue recognition criteria have been met.

### ***Milestones and Contingent Payments***

Contingent consideration received from the achievement of a substantive milestone will be recognized in its entirety in the period in which the milestone is achieved. A milestone is defined as an event having all of the following characteristics: (i) there is substantive uncertainty at the date the arrangement is entered into that the event will be achieved, (ii) the event can only be achieved based in whole or in part on either the company's performance or a specific outcome resulting from the company's performance and (iii) if achieved, the event would result in additional payments being due to the Company.

The Company's future research and development and license agreements may provide for success fees or payments to be paid to the Company upon the achievement of certain development milestones. Given the challenges inherent in developing biologic products, there may be substantial uncertainty as to whether any such milestones would be achieved at the time the agreements are executed. In addition, the Company will evaluate whether the development milestones meet all of the conditions to be considered substantive. The conditions include: (1) the consideration is commensurate with either of the following: (a) the Company's performance to achieve the milestone or (b) the enhancement of the value of the delivered item or items as a result of a specific outcome resulting from the Company's performance to achieve the milestone; (2) the consideration relates solely to past performance; and (3) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. If the Company considers the development milestones to be substantive, revenue related to such future milestone payments will be recognized as the Company achieves each milestone. Research and development funding internal and external research and development costs reimbursed in connection with research and development funding or collaboration agreements are recognized as revenue in the same period as the costs are incurred, and are presented on a gross basis because the Company acts as a principal, has the discretion to choose suppliers, bears credit risk, and performs part of the services.

### **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

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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. Research and development expenses under collaboration agreements approximate the revenue recognized under such agreements.

The expenses related to clinical trials are based upon estimates of the services received and efforts expended pursuant to contracts with research institutions and clinical research organizations (CROs) that conduct and manage clinical trials on behalf of the Company. The Company's objective is to reflect the appropriate trial expenses in its financial statements by matching those expenses with the period in which services and efforts are incurred. 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.

### **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-based measurements of such instruments. The fair value-based measurements of options and warrants granted to non-employees are re-measured over the related vesting period and amortized to expense as earned.

### **Common Stock Warrants**

The Company's outstanding common stock warrants issued in with the 2013 financing are classified as liabilities in the accompanying balance sheets as they contain provisions that could require the Company to settle the warrants in cash. 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 with any changes in fair value being recognized as a component of other income (expense) in the accompanying statements of operations and comprehensive loss.

### **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.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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 operations and comprehensive loss. There was no interest or penalties related to income taxes recorded during the years ended December 31, 2013 and 2012.

### **Comprehensive Loss**

Comprehensive loss includes net loss and net unrealized gains and losses on marketable securities, which are presented in a single continuous statement. Comprehensive loss is disclosed in the statements of convertible preferred stock and stockholders' deficit, and is stated net of related tax effects, if any.

### **Net Income (Loss) Per Common Share**

Basic net income (loss) per share of common stock is based on the weighted average number of shares of common stock outstanding equivalents during the period. Prior to the 2013 financing, in addition to common stock, the Company had redeemable convertible preferred stock outstanding that contractually entitled the holder to participate in dividends and earnings of the Company. Accordingly, the Company applied the two-class method for calculating net income (loss) per share. Under this method, all undistributed earnings were allocated first to the preferred stockholders based on their contractual right to dividends. This right was calculated on a pro rated basis for the portion of the period the preferred shares were outstanding. In addition, in connection with the 2013 financing, during the year ended December 31, 2013, the Company converted all outstanding redeemable convertible preferred stock into common stock. The excess of the carrying amount of such redeemable convertible preferred stock over the fair value of the consideration paid to the holders was treated as an adjustment that reduced preferred stockholders' dividend or distribution entitlement. The amount of earnings that resulted from adjusting net loss for the period as described above was allocated between weighted average number of participating preferred and common stock shares based on their entitlement to such distributions as if all of the earnings of the period had been distributed.

Diluted net loss per share of common stock is calculated using the more dilutive of the two approaches: one, "as-converted" method, under which the weighted average number of common stock shares outstanding during the period is adjusted to include the assumed conversion of redeemable convertible preferred stock at the beginning of the period, and the other, the "two-class" method as described above. Under either approach, the weighted average number of shares outstanding is also adjusted to include the assumed exercises of stock options and warrants, if dilutive. For periods in which the Company has basic net loss per share of common stock, such as for the years ended December 31, 2013 and 2012, diluted net loss per share is the same as basic, as any adjustments would have been anti-dilutive. For the year ending December 31, 2013, the Company's diluted net loss per common share was calculated using the "as-converted" method, as it resulted in a net loss per share of common stock and accordingly, was more dilutive than the "two-class" method.

In all periods presented, the Company's outstanding stock options and warrants were excluded from the calculation of earnings (loss) per share because the effect would be antidilutive.

[Table of Contents](#)

[Index to Financial Statements](#)

The following table sets forth the computation of basic and diluted net income (loss) per share (in thousands, except share and per share amounts):

	Three Months Ended		Year Ended December 31,	
	March 31,			
	2014	2013	2013	2012
<b>Basic:</b>				
Numerator:				
Net loss	\$ (10,062)	\$ (2,628)	\$ (10,073)	\$ (11,255)
Accretion to redemption value of redeemable convertible preferred stock	—	(3,109)	(9,289)	(12,644)
Reduction in redeemable convertible preferred stock distribution entitlement upon extinguishment	—	—	313,933	—
Amounts allocated to participating redeemable convertible preferred stock	—	—	(50,577)	—
Net income (loss) allocated to common stock—basic	\$ (10,062)	\$ (5,737)	\$ 243,994	\$ (23,899)
Denominator:				
Weighted average number of common stock shares outstanding	9,873,687	5,824	2,357,036	5,788
Net income (loss) per share—basic:	\$ (1.02)	\$ (985.06)	\$ 103.52	\$ (4,128.71)
<b>Diluted:</b>				
Numerator:				
Net income (loss) allocated to common stock	\$ (10,062)	\$ (5,737)	\$ 243,994	\$ (23,899)
Adjustments from assumed conversion of redeemable convertible preferred stock	—	—	(254,067)	—
Net loss allocated to common stock—diluted	\$ (10,062)	\$ (5,737)	\$ (10,073)	\$ (23,899)
Denominator:				
Weighted average number of common stock shares outstanding	9,873,687	5,824	2,357,036	5,788
Weighted average number of preferred stock shares outstanding	—	—	488,573	—
Total common stock shares equivalents	9,873,687	5,824	2,845,609	5,788
Net loss per share—diluted:	\$ (1.02)	\$ (985.06)	\$ (3.54)	\$ (4,128.71)

The following table shows the total outstanding securities considered anti-dilutive and therefore excluded from the computation of diluted net income (loss) per share (in thousands):

	Three Months Ended		Year ended	
	March 31,		December 31,	
	2014	2013	2013	2012
	(unaudited)			
Warrants for common stock	1,848	28	1,743	28
Common stock options	907	97	577	104
Redeemable convertible preferred stock	—	661	—	661

[Table of Contents](#)

[Index to Financial Statements](#)

### 3. Marketable Securities

Marketable available-for-sale securities as of December 31, 2013 and March 31, 2014 consist of the following (in thousands):

	<u>Amortized Cost</u>	<u>Gross Unrealized Gains</u>	<u>Gross Unrealized Losses</u>	<u>Estimated Fair Value</u>
As of December 31, 2013:				
Corporate debt securities	\$ 6,355	\$ 3	\$ (2)	\$ 6,356
Asset-backed securities	486	1	—	487
	<u>\$ 6,841</u>	<u>\$ 4</u>	<u>\$ (2)</u>	<u>\$ 6,843</u>
As of March 31, 2014:				
Corporate debt securities	\$ 5,832	\$ 2	\$ —	\$ 5,834
Asset-backed securities	356	1	—	357
	<u>\$ 6,188</u>	<u>\$ 3</u>	<u>\$ —</u>	<u>\$ 6,191</u>

As of December 31, 2013, and March 31, 2014, the Company's corporate debt marketable securities had contractual maturities of less than one year and asset-backed securities had contractual maturities between 2-5 years. Realized gains and losses were immaterial for the years ended December 31, 2013 and 2012 and the three months ended March 31, 2014 and 2013. None of these investments have been in a continuous unrealized loss position for more than 12 months as of December 31, 2013. The company did not hold any marketable securities as of December 31, 2012.

### 4. Certain Balance Sheet Items

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

	<u>March 31, 2014</u>	<u>December 31,</u>	
	(unaudited)	<u>2013</u>	<u>2012</u>
Laboratory equipment	\$ —	\$ —	\$ 3,778
Office and computer equipment	556	556	983
Purchased software	166	166	166
Furniture and fixtures	42	42	174
Leasehold improvements	349	2,534	2,534
Total	1,114	3,298	7,635
Less accumulated depreciation and amortization	(1,054)	(3,295)	(7,551)
Property and equipment, net	<u>\$ 60</u>	<u>\$ 3</u>	<u>\$ 84</u>

Property and equipment includes assets financed through equipment loans, which were fully paid in January 2012.

Accrued liabilities consist of the following (in thousands):

	<u>March 31, 2014</u>	<u>December 31,</u>	
	(unaudited)	<u>2013</u>	<u>2012</u>
Accrued compensation	\$ 419	\$ 518	\$291
Accrued pre-clinical and clinical trial expenses	1,233	418	304
Accrued professional fees	501	782	285
Forward contract	—	453	—
Other accruals	215	80	110
Total accrued liabilities	<u>\$ 2,368</u>	<u>\$2,251</u>	<u>\$990</u>

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[Table of Contents](#)

[Index to Financial Statements](#)

**5. Collaboration Agreements**

**Sanofi-Aventis Deutschland GMBH**

In June 2010, the Company entered into a development and license agreement effective July 21, 2010, with Sanofi-Aventis Deutschland GMBH (Sanofi-Aventis), whereby Sanofi-Aventis received an exclusive worldwide license for the research, development, manufacture and commercialization of small molecules that modulate the G-protein coupled receptor 119 (GPR119). The agreement includes rights to MBX-2982, a potent selective orally active GPR119 agonist discovered by the Company. Upon the effective date of this agreement, the Company received a one-time nonrefundable up-front license payment of \$25.0 million. The Company was eligible to receive milestones if certain development and commercial events were achieved, as well as royalties on worldwide product sales, if any. The one-time nonrefundable up-front license payment was being recognized as revenue ratably over the period that the Company expected to complete certain research and development activities that represent the Company's substantive performance obligations under the agreement. Of this up-front license fee, none was recognized for the years ended December 31, 2013 or December 31, 2012.

On June 15, 2011, the arrangement was terminated by Sanofi-Aventis. Following termination, the Company retained rights to the current programs under this agreement and may continue to develop the programs and commercialize any products resulting from the programs, or the Company may elect to cease progressing the programs and/or seek other partners for further development and commercialization of the programs.

In 2012, the Company recognized a final payment from Sanofi-Aventis of \$2.9 million as contract revenue.

**Takeda San Francisco, Inc.**

In March 2010, the Company entered into a research collaboration agreement with Takeda San Francisco, Inc. (TSF), a wholly owned subsidiary of Takeda Pharmaceutical Company Limited. The Company collaborated with TSF on the evaluation and validation of protein targets for the development of biological products. In March 2010, the Company received \$1.5 million, representing \$0.9 million of one-time nonrefundable technology access fees and \$0.6 million of specified research and development funding for the research term of the collaboration. The technology access fee and the research and development funding were deferred and were being recognized ratably over the funded research term, which was scheduled from March 2010 to August 2011. Approximately \$0.1 was recognized as specific research and development funding under this agreement in the year ended December 31, 2012. Takeda terminated this agreement on March 16, 2013 with no further payments being made as of the year ended December 31, 2013.

In August 2006, CymaBay entered into a strategic alliance with Ortho-McNeil, Inc., a subsidiary of Johnson and Johnson. As part of the alliance, Janssen Pharmaceutical NV, an affiliate of Ortho-McNeil, granted to CymaBay an exclusive worldwide, royalty-bearing license to MBX-8025 and certain other PPARd compounds (the "PPARd Products") with the right to grant sublicenses to third parties to make, use and sell such PPARd Products. Under the terms of the agreement, CymaBay 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 CymaBay in the event that CymaBay 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, 2014 and 2013.

In June 2010, CymaBay 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. CymaBay is also eligible to receive up to \$228 million in contingent payments if certain development and commercial events are achieved as well 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



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## [Table of Contents](#)

## [Index to Financial Statements](#)

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.

### **6. License Agreements**

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 years ended December 31, 2013 and 2012 or the three months ended March 31, 2014 and no royalties have been paid to date.

### **7. Debt**

#### **JJDC Convertible Note**

On June 20, 2006 the Company entered into an equity and loan facility with the Johnson and Johnson Development Corporation ("JJDC") pursuant to which the Company could draw down up to an aggregate of \$30 million in loans in the form of convertible preferred stock promissory notes. In March and September 2008, the Company issued notes in the aggregate amount of \$3.5 million and \$10.5 million, respectively. The notes were due on March 17 and September 17, 2011, including interest that accrued at 7.57% per annum. In December 2010, the aggregate principal amount and all accrued interest under the notes issued in March and September 2008 were converted into the Company's Series E-3 convertible preferred stock (Series E-3 Preferred) at 232.93 per share.

In February and July 2009, the Company issued notes in the aggregate amount of \$7.0 million and \$6.7 million, respectively, in accordance with the terms of the equity and loan facility with JJDC. The notes were due in February 2012 and July 2012, including interest that accrued at 4.42% per annum and 4.960% per annum, respectively. In January 2012, the Company amended the maturity dates of the outstanding \$7.0 million and \$6.7 million convertible promissory notes to extend the maturity date to March 1, 2013, and interest rates were increased to 4.919% and 5.46% per annum, respectively. In addition, the conversion price of the notes to convert into shares of the Company's Series C-1 Preferred Stock was decreased from \$438.84 per share to \$292.56 per share. All of these notes were further amended in March 2013, to extend the maturity date on the notes to August 1, 2013, and to make the notes subordinate to repayment of the Company's severance obligations to all employees until January 1, 2014. On July 31, 2013, the maturity date was extended to December 31, 2013. For the years ended December 31, 2013 and 2012, the Company recognized \$0.6 million and \$0.7 million respectively, of interest expense related to the convertible promissory notes. On September 30, 2013, the outstanding principal and accrued interest of \$16.9 million under the equity and loan facility with JJDC was extinguished in exchange for the issuance of 624,944 shares of common stock as an integral part of the 2013 finance restructuring.

#### **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 and bears interest at a rate equal 8.75% per annum. The second tranche of \$5.0 million will be made available to the Company only upon achievement of positive Phase 2b data (the second draw

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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.

For each tranche borrowed, the Company is required to make 12 monthly interest only payments after the funding date followed by a repayment schedule equal to 36 equal monthly payments of interest and principal. 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. The final payment equal to 6.50% of the original principal is being accreted over the life of the loan.

Future principal payments due under the loan facility are as follows (in thousands):

	<b>Principal Payments</b>
<b>Year ending December 31:</b>	
2014	\$ 245
2015	1,546
2016	1,687
2017	1,522
Total future principal payments due under loan agreement	<u>\$ 5,000</u>

During the loan term, the term loan facility provides that the Company must maintain compliance with one of two financial covenants at all times: (1) maintain 1.3 times cash to outstanding debt or (2) maintain sufficient cash on hand to support eight months of operations based on a trailing average monthly cash burn. The term loan facility also contains a series of performance covenants however failure to comply with these performance covenants shall not be an event of default under the term loan facility so long as the Company deposits an amount equal to 100% of the aggregate outstanding term loans in a segregated, blocked deposit account at Silicon Valley Bank. As of December 31, 2013 and March 31, 2014, the Company was in compliance with its loan covenants.

The Company is permitted to make voluntary prepayments of the term loans with a prepayment fee equal to 3% of the term loans prepaid. The Company is 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 that are due and payable at the time of the prepayment.

The Company was required to pay a facility fee of 1.00% on the term loan facility commitment. In addition, at the time of the facility loan drawdown, 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 a warrant liability of \$0.5 million was recorded in the accompanying balance sheet as of September 30, 2013. The facility fee, the warrant value on its issuance date, and other debt issuance costs were reflected as a debt discount and are being amortized to interest expense over the term of the outstanding loan using the effective interest rate method. The liability classified warrants must be remeasured at fair value on each reporting date and changes in fair value are recorded as other income, net in the accompanying statement of operations (see Note 11 for more details).

## **8. Commitments and Contingencies**

### **Operating Lease Commitments**

For the years ended December 31, 2013 and 2012, 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, unless terminated earlier by the Company, 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 \$0.5 million for the years ended December 31, 2013 and 2012, and \$0.2 million and \$0.1 million for the three months ended March 31, 2014 and 2013, respectively.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

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

	<b>Lease Payments</b>
Year ending December 31,	
2014	\$ 337
2015	209
2016	216
2017	222
2018	228
Total future minimum payments	<u>\$ 1,212</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.

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, 2014 and December 31, 2013 and 2012. 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.

### **9. Redeemable Convertible Preferred Stock**

Upon the closing of the 2013 financing on September 30, 2013, all the outstanding shares of the Company's redeemable convertible preferred stock were converted into 2,793,281 shares of common stock, and the related carrying value of \$320.0 million was reclassified to additional paid-in capital. As of March 31, 2014 and December 31, 2013, no shares of redeemable convertible preferred stock were issued or outstanding.

Prior to the September 30, 2013 conversion, the Company had the following series of outstanding convertible preferred stock (collectively, the Preferred Stock): Series A-1 Preferred, Series B-1 Preferred, Series C-1 Preferred, Series D-1 Preferred, Series E-1 Preferred and Series E-3 Preferred. Series E-1 Preferred and Series E-3 Preferred are collectively referred to as the Series E Preferred. The Preferred Stock was initially recorded at its original purchase price, which represented fair value on the date of issuance, net of issuance costs, if any. The original purchase price per share of Series A-1 Preferred, Series B-1 Preferred, Series C-1 Preferred, Series D-1 Preferred, and Series E Preferred was equal to \$232.93, \$232.93, \$365.70, \$232.94, and \$232.93 per share, respectively. The preferred stock balances were recorded at the original fair value and the accreted dividends based on the per share terms at issuance of Series A-1 Preferred, Series B-1 Preferred, Series C-1 Preferred, Series D-1 Preferred, and Series E Preferred, which were equal to \$18.64, \$18.64, \$29.26, \$18.64, and \$18.64 per share per annum, respectively.

The shares of Series B-1 Preferred, Series D-1 Preferred, and Series E Preferred were redeemable upon the request of the holders of at least 66 2/3% of outstanding shares of Series B-1 Preferred, voting as a separate class,

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## [Table of Contents](#)

## [Index to Financial Statements](#)

and 51% of outstanding shares of Series D-1 Preferred and Series E Preferred, voting together as a separate class. In this event, the Company would have been required to redeem the shares in three equal annual installments, beginning in September 2021, at the applicable original purchase price per share. All shares of Preferred Stock were redeemable in the event of a change of control at their liquidation preferences.

As all Preferred Stock was redeemable either at the option of the holder or upon an event outside the control of the Company (i.e., a change in control), the related amounts have been presented outside of stockholders' equity (deficit). In August and December 2003, the Company completed two closings of a private placement of Series B-1 Preferred, in which the Company issued a total of 136,520 shares at a price of \$232.93 per share for gross proceeds of \$31.8 million. In November and December 2004, the Company completed two further closings of Series B-1 Preferred, in which the Company issued a total of 188,894 shares at a price of \$232.93 per share for gross proceeds of \$44.0 million. The Series B-1 Preferred investors in these two final closings also purchased warrants for 29,245 shares of common stock at an exercise price of \$30.21 per share, with an exercise period of five years from the date of purchase, for \$1.51 cents per share of common stock covered by the warrants. In November 2009, the exercise period of these warrants was extended to December 31, 2011. In December 2012, the Company's Board of Directors reduced the number of shares exercisable under these warrant by 45% of the original shares and approved the extension of the exercise period until April 1, 2013. As of December 31, 2012, warrants to purchase 13,160 shares of common stock were outstanding. In April 2013, these warrants expired in accordance with their terms.

In August 2006, the Company issued 27,345 shares of Series C-1 Preferred to JJDC at a price of \$365.70 per share, for gross proceeds of \$10.0 million.

In April 2007, the Company issued 137,592 shares of Series D-1 Preferred at a price of \$232.94 per share, for gross proceeds of \$32.0 million. In connection with the issuance, the Series D-1 Preferred investors also purchased warrants for an aggregate of 20,639 shares of common stock at an exercise price of \$22.13 per share, with an exercise period of five years from the date of purchase, for \$0.79 cents per share of common stock covered by the warrants.

In August 2008, the Company repurchased 646, 1,610 and 472 shares of Series A-1 Preferred, Series B-1 Preferred and Series D-1 Preferred, respectively, and a warrant for 71 shares of common stock, for an aggregate purchase price of \$82,000. The Company allocated the purchase price among the preferred shares and warrant based upon their respective fair values.

In November 2009, the Company issued 1,288 shares of Series E-1 Preferred upon the conversion of debt issued under a loan agreement. In June and December 2010, the Company issued 859 and 37,119 shares of Series E-1 Preferred, respectively, upon conversion of debt issued under a loan agreement.

In December 2010, the Company issued 71,543 shares of Series E-3 Preferred upon conversion of the JJDC convertible notes that were due in 2011 (Note 7).

As of December 31, 2012, convertible preferred stock balances were as follows (in thousands, except share amounts):

	<b>Shares Authorized</b>	<b>Shares Issued and Outstanding</b>	<b>Aggregate Liquidation Preference</b>	<b>Carrying Value</b>
Series A-1	12,734	12,734	\$ 5,187	\$ 75,454
Series B-1	373,223	373,223	146,549	145,408
Series C-1	75,472	27,345	15,122	15,074
Series D-1	136,948	136,949	46,520	43,271
Series E-1	40,252	39,265	19,820	10,674
Series E-3	93,082	71,543	23,552	28,816
Total	<u>731,711</u>	<u>661,059</u>	<u>\$ 256,750</u>	<u>\$318,697</u>

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## [Table of Contents](#)

## [Index to Financial Statements](#)

The significant rights, privileges, and preferences of the Preferred Stock were as follows:

### **Election of Directors**

Prior to the September 30, 2013 conversion, the holders of Series B-1 Preferred were entitled to elect five members of the Company's Board of Directors, the holders of Series D-1 Preferred were entitled to elect one member of the Company's Board of Directors, and the holders of common stock were entitled to elect one member of the Company's Board of Directors, subject to certain restrictions. All remaining members of the Company's Board of Directors were elected by all of the stockholders voting on an as-if-converted basis.

### **Voting Rights**

Prior to the September 30, 2013 conversion, the Preferred Stock carried voting rights equal to the number of shares of common stock into which it could be converted. Additionally, certain corporate actions could only be exercised upon the approval of holders of 66 2/3% of the outstanding shares of Series B-1 Preferred and Series C-1 Preferred, voting together as a single class, and 51% of the outstanding shares of Series D-1 Preferred and Series E Preferred, voting together as a single class.

### **Dividends**

All dividends were payable when and if declared by the Company's Board of Directors. The holders of Series E Preferred were entitled to cumulative dividends in preference to the holders of Series A-1 Preferred, Series B-1 Preferred, Series C-1 Preferred, Series D-1 Preferred, and common stock. The holders of Series D-1 Preferred were entitled to cumulative dividends in preference to the holders of Series A-1 Preferred, Series B-1 Preferred, Series C-1 Preferred, and common stock. The holders of Series B-1 Preferred and Series C-1 Preferred were entitled to cumulative dividends in preference to the holders of Series A-1 Preferred and common stock. The holders of Series A-1 Preferred were entitled to cumulative dividends in preference to the holders of common stock. The dividend rate was \$18.64, \$18.64, \$29.26, \$18.64, and \$18.64 per annum for each outstanding share of Series E Preferred, Series D-1 Preferred, Series C-1 Preferred, Series B-1 Preferred, and Series A-1 Preferred, respectively. Additionally, if dividends were paid to any holder of common stock, the holders of Preferred Stock would receive a dividend of a per share amount (on an as-if-converted to common stock basis) equal to the amount paid to the holders of common stock.

No dividends were declared as of December 31, 2013 and 2012. Prior to the conversion of the Preferred Stock in connection with the 2013 financing, the aggregate cumulative dividends as of September 30, 2013, were \$3.4 million (\$47.28 per share), \$1.9 million (\$48.14 per share), \$15.9 million (\$116.00 per share), \$5.6 million (\$201.83 per share), \$63.1 million (\$168.96 per share), and \$2.3 million (\$183.64 per share) for Series E-3 Preferred, Series E-1 Preferred, Series D-1 Preferred, Series C-1 Preferred, Series B-1 Preferred, and Series A-1 Preferred, respectively. The aggregate cumulative dividends as of December 31, 2012, were \$2.7 million (\$38.04 per share), \$1.5 million (\$38.90 per share), \$14.6 million (\$106.75 per share), \$5.1 million (\$187.32 per share), \$59.6 million (\$159.72 per share), and \$2.2 million (\$174.40 per share) for Series E-3 Preferred, Series E-1 Preferred, Series D-1 Preferred, Series C-1 Preferred, Series B-1 Preferred, and Series A-1 Preferred, respectively.

### **Liquidation Preference**

While the Preferred Stock was outstanding, in the event of a liquidation, dissolution, winding up, or change in control of the Company, the liquidation preference of each stockholder class was to be paid in the following order, from available funds: first to the holders of Series E-1 Preferred and Series E-3 Preferred, second to the holders of Series D-1 Preferred, third to the holders of Series B-1 Preferred and Series C-1 Preferred, and fourth to the holders of Series A-1 Preferred. After payment of the Preferred Stock liquidation preferences, the remaining assets of the Company were to be distributed ratably to all holders of common stock and Preferred Stock on an as-if-converted basis. The liquidation preference of Series E-1 Preferred, Series E-3 Preferred, Series D-1 Preferred, Series C-1 Preferred, Series B-1 Preferred, and Series A-1 Preferred was equal to \$465.87,

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## [Table of Contents](#)

## [Index to Financial Statements](#)

\$290.97, \$232.93, \$365.70, \$232.93, and \$232.93 per share, respectively, plus any cumulative unpaid dividends. If there were insufficient funds available to satisfy each liquidation preference in its entirety, the holders of Preferred Stock were to be paid a pro rata amount based on their liquidation preference.

### **Conversion Rights**

Each share of Preferred Stock was convertible at any time, at the option of the holder, into shares of the Company's common stock at then applicable conversion rate. The conversion rate for each of the series of Preferred Stock was 1:1, except for the Series D-1 Preferred, which had a conversion rate of 1.365:1. With respect to the Series E Preferred, Series D-1 Preferred, Series B-1 Preferred, and Series A-1 Preferred, if the Company issued common stock or securities convertible into or exercisable for shares of common stock at a price less than the respective original purchase price per share, the conversion rate of such stock was to be adjusted to the lowest price per share paid in such issuance. The conversion rate for Preferred Stock would not be adjusted for common stock issuances on the exercise of options or warrants issued to employees, directors, or consultants of the Company and in certain other circumstances.

Each share of Preferred Stock automatically converted into common stock upon the approval of holders of 66 2/3% of the outstanding shares of Series B-1 Preferred, voting as a separate class, and 51% of the outstanding shares of Series D-1 Preferred and Series E Preferred, voting together as a separate class, or upon the closing of an underwritten public offering of the Company's common stock pursuant to an effective registration statement under the Securities Act of 1933, as amended, at a per share price of at least \$8.00, and raising aggregate gross proceeds of at least \$30.0 million. In connection with the 2013 financing each holder of the Company's preferred stock that participated in the 2013 financing for between 1% and up to 99% of such holders "*Pro Rata Share*" (as defined in the Company's then effective certificate of incorporation) had each share of preferred stock represented by such participation amount converted into four shares of common stock and the balance of any shares of preferred stock converted at the then applicable conversion rate. Any holder that participated in the 2013 financing for between 100% and 300% of such holder's Pro Rata Share (the "*Participation Multiple*") had each share of preferred stock convert into shares of common stock by multiplying the product of (y) the aggregate number of shares of preferred stock held by such holder multiplied by the applicable Participation Multiple and (z) four (4).

### **10. Common Stock**

The Company was authorized to issue 100,000,000 shares of common stock as of March 31, 2014 and December 31, 2013 and 74,000,000 shares of common stock were authorized as of December 31, 2012.

#### **Common Stock Issuances in the 2013 Financing**

On September 30, 2013, all the outstanding shares of the Company's redeemable convertible preferred stock were converted into 2,793,281 shares of common stock and the related carrying value of \$320.0 million was reclassified to additional paid-in capital.

Commencing on September 30, 2013, the Company entered into a series of financing transactions (collectively referred to as the 2013 financing) which resulted in the issuance of common stock and warrants to purchase shares of common stock. Specifically, on September 30, 2013, the Company sold 5,366,669 shares of common stock and 1,073,338 warrants to purchase shares of common stock in a private placement for net proceeds to CymaBay of \$22.8 million after deducting placement agent fees and estimated offering expenses. Also on that date, the Company issued 624,944 shares of common stock in cancellation of approximately \$16.9 million of debt owed to JJDC, the holder of that debt (Note 7).

On October 31, 2013, the Company sold an additional 664,300 shares of common stock and warrants to purchase 132,860 shares of common stock, which sales were also part of the private placement, for net proceeds to CymaBay of \$2.2 million after deducting placement agent fees and estimated offering expenses.

On November 22, 2013, the Company entered into an agreement with investors to purchase 604,000 shares of common stock and 120,800 warrants to purchase shares of common stock as part of the private placement for

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[Table of Contents](#)

[Index to Financial Statements](#)

net proceeds of \$2.7 million, which sales were set to occur shortly after the listing of the Company's common stock on the OTCQB Marketplace. Cymabay began trading on the OTCQB Marketplace on January 24, 2014 enabling this portion of the financing to be completed in late January 2014.

**Common Stock Warrants**

In connection with the 2013 financing and the Company's private placement of common stock and warrants, in September 2013, October 2013, and January 2014, the Company issued five-year warrants to purchase an aggregate of 1,741,788 shares of CymaBay's common stock at an exercise price of \$5.75 per share which the Company refers to here as the 2013 financing warrants. Also included in the 2013 financing warrants are five-year warrants the Company issued to purchase 121,739 shares of CymaBay's common stock to its lenders at an exercise price of \$5.00 per share. Certain of the 2013 financing warrants contain provisions that are contingent on the occurrence of a change in control, which would conditionally obligate the Company to repurchase the warrants for cash in an amount equal to their fair value using the Black-Scholes Option Pricing Model (the "Black-Scholes Model") on the date of such change in control. Due to these provisions, the Company is required to account for all the 2013 financing warrants as a liability at fair value. In addition, the estimated liability related to the 2013 financing warrants is required to be revalued at each reporting period until the earlier of the exercise or expiration of the warrants, at which time the liability will be reclassified to stockholders' equity. Upon issuance, the fair value of the 2013 financing warrants was estimated to be \$6.4 million. These warrants were revalued at fair value as of December 31, 2013 and March 31, 2014 using a binomial lattice model and the resulting increase in fair value of \$0.5 million and \$4.8 million, respectively, was recorded as an increase to the warrant liability and as a loss in other income, net in the Company's Statement of Operations and Comprehensive Loss.

In November 2009, the Company's Board of Directors approved the extension of the time period in which the holders of warrants to purchase 29,245 shares of common stock are able to exercise their warrants that were issued in connection with the issuance of Series B-1 Preferred. The exercise periods of the warrants that originally ended in November 2009 were extended to December 31, 2010. In December 2010, the Company's Board of Directors further modified these warrants. The number of common shares exercisable under the warrants was reduced by 50% to 14,623, and the exercise period was extended to December 31, 2012. In December 2012, the Company's Board of Directors again modified these warrants to purchase common stock. The number of shares exercisable under the warrants issued with the issuance of the Series B-1 Preferred was reduced by 45% of the original shares to 13,163, and the exercise period was extended to April 1, 2013. The extension of the agreement did not cause a material change in value. In April 2013, these warrants expired.

In December 2010, the Company's Board of Directors modified the warrants to purchase common stock that were issued in connection with the issuance of Series D-1 Preferred. The exercise period of the warrants issued in connection with the Series D-1 Preferred issuance was extended to April 13, 2013. The charge related to the modifications to these warrants of \$0.1 million was recorded to accumulated deficit and was determined using the Black-Scholes valuation model, with the following inputs used to determine the charge related to the modification: fair value of the Company's common stock of \$15.90 per share, expected life of the modified warrants of one to two years, risk-free interest rate of 0.50%, and expected common stock price volatility of 83%. In April 2013, these warrants expired.

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[Table of Contents](#)

[Index to Financial Statements](#)

**Shares of Common Stock Authorized for Issuance**

As of March 31, 2014, December 31, 2013 and December 31, 2012, the Company had reserved shares of authorized but unissued common stock as follows:

	<u>March 31,</u> <u>2014</u>	<u>Shares Reserved</u> <u>December 31, 2013</u>	<u>Shares Reserved</u> <u>December 31, 2012</u>
	(unaudited)		
Outstanding common stock warrants	1,848,487	1,742,727	28,208
Equity incentive plans	1,050,047	577,294	140,474
Convertible preferred stock	—	—	661,059
Total reserved shares of common stock	<u>2,898,534</u>	<u>2,320,021</u>	<u>829,741</u>

**11. Stock Plans and Stock-Based Compensation**

**Stock Plans**

In September 2013, the Company's stockholders approved the 2013 Equity Incentive Plan (2013 Plan), under which shares of common stock are reserved for the granting of options, stock bonuses, and restricted stock awards by the Company. These awards may be granted to employees, members of the Board of Directors, and consultants to the Company. The 2013 Plan has a term of ten years and replaced the 2003 Equity Incentive Plan, which had similar terms. The 2013 Plan permits the Company to (i) grant incentive stock options to directors and employees at not less than 100% of the fair value of common stock on the date of grant; (ii) grant nonqualified options to employees, directors, and consultants at not less than 85% of fair value; (iii) award stock bonuses; and (iv) grant rights to acquire restricted stock at not less than 85% of fair value. Options generally vest over a four- or five-year period and have a term of ten years. Options granted to 10% stockholders have a maximum term of five years and require an exercise price equal to at least 110% of the fair value on the date of grant. The exercise price of all options granted to date has been at least equal to the fair value of common stock on the date of grant.

Restricted stock units, which had been previously granted in 2007 pursuant to the Company's 2003 Equity Incentive Plan, vested over a four- or five-year period, subject to certain performance conditions, and terminated on August 19, 2012.

**Stock Plan Activity**

In December 2013, the Company's Board of Directors modified the terms of 60,847 stock options held by employees, directors, and scientific advisory board members. Specifically, the exercise price for such options was reduced to \$5, the fair market value of the Company's common stock on the date of modification, and the term of each option was extended to 10 years from the date of the modification. The Company will account for this stock option modification by recognizing any unamortized expense related to the original unmodified options as of the modification date over the remaining vesting periods of those awards. The incremental expense resulting from this modification of \$0.2 million will also be recognized over the remaining vesting period. As substantially all of the modified awards were fully vested on the modification date, the Company recognized \$0.2 million of noncash stock-based compensation expense related to this stock option modification in December 2013.

As of March 31, 2014 and December 31, 2013, 143,251 and 41 shares were available for issuance under the 2013 plan, respectively. In accordance with the provisions of the Company's 2013 Equity Plan, the number of shares available for issuance under the plan automatically increased by 472,753 shares on January 1, 2014.



[Table of Contents](#)

[Index to Financial Statements](#)

The following table summarizes stock option activity:

	Shares Subject to Outstanding Options	Weighted- Average Exercise Price of Options	Weighted- Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of December 31, 2012	103,760	\$ 34.19	4.43	\$ —
Options granted	487,697	5.00		
Options exercised	(77)	4.77		
Options forfeited	(3,490)	10.36		
Options expired	(10,637)	31.31		
Outstanding as of December 31, 2013	577,253	\$ 7.00	9.57	\$ 3
Options granted (unaudited)	342,950	5.02		
Options exercised (unaudited)				
Options forfeited (unaudited)	(8,059)	4.98		
Options expired (unaudited)	(5,348)	30.21		
Outstanding as of March 31, 2014 (unaudited)	906,796	\$ 6.13	9.47	\$ 2,682
Vested and expected to vest as of December 31, 2013	557,995	\$ 7.07	9.56	\$ 3
Exercisable as of December 31, 2013	289,308	\$ 9.55	9.25	\$ 1
Vested and expected to vest as of March 31, 2014 (unaudited)	877,457	\$ 6.17	9.47	\$ 2,595
Exercisable as of March 31, 2014 (unaudited)	380,568	\$ 7.68	9.20	\$ 1,110

The following table summarizes information about stock options outstanding as of March 31, 2014 (unaudited):

Exercise Price	Options Outstanding		Options Exercisable
	Number of Shares	Weighted- Average Remaining Contractual Term (Years)	Number of Shares
\$4.77	11,646	7.42	9,173
\$5.00	881,546	9.61	416,030
\$7.99	2,500	9.96	—
\$15.90	839	0.03	839
\$30.21	3,290	0.77	3,290
\$39.75	3,520	0.03	3,520
\$238.50	3,455	2.49	3,455
	906,796	9.47	436,307

[Table of Contents](#)

[Index to Financial Statements](#)

The following table summarizes information about stock options outstanding as of December 31, 2013:

<u>Exercise Price</u>	<u>Options Outstanding</u>		<u>Options Exercisable</u>
	<u>Number of Shares</u>	<u>Weighted-Average Remaining Contractual Term (Years)</u>	<u>Number of Shares</u>
\$4.77	12,257	7.62	8,815
\$5.00	548,544	9.88	264,041
\$15.90	839	0.27	839
\$30.21	8,638	0.41	8,638
\$39.75	3,520	0.27	3,520
\$238.50	3,455	2.73	3,455
	<u>577,253</u>	<u>9.57</u>	<u>289,308</u>

**Grant Date Fair Value**

The following table presents the weighted-average assumptions the Company used with the Black-Scholes valuation model to derive the grant date fair value-based measurements of employee and director stock options and the resulting estimated weighted-average grant date fair-value-based measurements per share:

	<u>Three months Ended</u>		<u>Year Ended December 31,</u>	
	<u>2014</u>	<u>2013</u>	<u>2013</u>	<u>2012</u>
Weighted-average assumptions:				
Expected term	6.02 yrs	—	6 yrs	6.25 yrs
Expected volatility	91%	—	92%	100%
Risk-free interest rate	2.04%	—	1.76%	1.01%
Expected dividend yield	0%	—	0%	0%
Weighted-average grant date fair value per share	\$ 3.76	—	\$ 3.76	\$ 3.97

***Expected Term***

The Company does not believe it can currently place reliance on its historical exercise and post-vesting termination activity to provide accurate data for estimating the expected term. Therefore, for stock option grants made during the three months ended March 31, 2014 and years ended December 31, 2013 and 2012, the Company has opted to use the simplified method for estimating the expected term which is an average of the contractual term of the options and its ordinary vesting period. The expected term represents the period of time that options are expected to be outstanding.

***Expected Volatility***

As the Company has limited trading history for its common stock, the expected stock price volatility for the Company's common stock was estimated by considering the volatility rates of similar publicly traded peer entities within the life sciences industry.

***Risk-Free Interest Rate***

The risk-free interest rate assumption was based on U.S. Treasury instruments with constant maturities whose term was consistent with the expected term of stock options granted by the Company.

***Expected Dividend Yield***

The Company has never declared or paid cash dividends and does not plan to pay cash dividends in the foreseeable future. Consequently, the Company uses an expected dividend yield of zero.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

### *Common Stock Fair Value*

Prior to January 24, 2014, there was no public market for the Company's common stock, and accordingly, the Company's Board of Directors historically determined the fair value of the Company's common stock for the purpose of pricing the Company's equity awards to employees, directors, and consultants. In making such fair value determinations, the Board of Directors considered a number of factors, including the price at which Preferred Stock was issued to outside investors in arm's-length transactions, the rights, preferences, and privileges of the Preferred Stock relative to the common stock, important developments relating to advancement of the Company's technology and clinical programs, the Company's stage of development and business strategy, the likelihood of achieving a liquidity event for the shares of common stock, such as an initial public offering or sale of the Company, prevailing market conditions, and the market prices of various publicly held life sciences companies. Additionally, the Board of Directors considered contemporaneous valuations provided by third-party valuation specialists.

### *Forfeitures*

The Company estimates forfeitures at the time of grant and revises these estimates in subsequent periods if actual forfeitures differ from those estimates. Changes in forfeiture estimates impact compensation in the period in which the change occurs.

The total intrinsic value of options exercised was none for the three months ended March 31, 2014 and 2013 and the years ended December 31, 2013 and 2012.

### **Vested and Unvested Awards**

The total fair value of options vested for the three months ended March 31, 2014 and 2013 and the years ended December 31, 2013 and 2012, was \$0.5 million, \$0.0 million, \$0.9 million and \$0.1 million, respectively.

As of March 31, 2014 and December 31, 2013, the total compensation expense related to unvested employee stock options to be recognized in future periods, excluding estimated forfeitures, was \$1.9 million and \$1.2 million, respectively. The weighted-average periods over which this compensation expense is expected to be recognized are 3.6 years and 3.9 years as of March 31, 2014 and December 31, 2013, respectively.

### **Incentive Awards**

In December 2013, as permitted by the 2013 Equity Plan, the Company issued certain incentive awards to directors, employees and a consultant which are indexed to 220,266 shares of the Company's common stock and are exercisable at \$5 per share when vested. Furthermore, in January 2014, the Company issued additional incentive awards to employees which are indexed to 6,486 shares of the Company's common stock and are exercisable at \$5 per share when vested. The Company may determine at its option whether to settle exercised awards in shares of common stock or in cash. Each recipient's incentive award defines the number of common shares that may be acquired upon exercise provided the Company chooses to settle in shares. For awards settled in cash, the Company must pay the recipient the excess of the fair market value of the Company's common stock on the date of exercise over the \$5 exercise price paid by the recipient multiplied by the number of shares the recipient would be entitled to receive had the award been settled in shares of the Company's common stock.

The incentive awards vest 100% on the second anniversary of their grant date and have a term of 10 years. If before this vest date the Company's shareholders approve an increase to the 2013 plan's shares available for issuance by an amount sufficient enough to cover the number of shares underlying the incentive awards, then the awards shall automatically be modified to vest monthly over four years effective from their grant date.

The incentive award is a stock based compensation arrangement. As of March 31, 2014 and December 31, 2013, the Company did not have sufficient shares available for issuance to settle the incentive awards in stock. Accordingly, settlement in cash is deemed more likely as of the balance sheet date. The Company accounted for these cash settled awards as a liability and will remeasure the awards at fair value at each reporting date until settled.

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## [Table of Contents](#)

## [Index to Financial Statements](#)

Compensation expense and the related incentive award liability will be recognized over the vesting period of the incentive awards.

As of December 31, 2013, the Company determined the fair value of the incentive awards using the Black-Scholes option pricing model with a stock price input of \$5.00, an exercise price of \$5.00, an expected term of six years, a volatility of 92%, and a dividend yield of 0% which resulted in a fair value of \$3.76 per share underlying the incentive awards. As of March 31, 2014, the Company determined the fair value of the incentive awards using the Black-Scholes option pricing model with a stock price input of \$8.00, an exercise price of \$5.00, an expected term of 5.75 years, a volatility of 89%, and a dividend yield of 0% which resulted in a fair value of \$6.31 per share underlying the incentive awards. The Company recorded \$188,000 and \$9,000 of compensation expense pertaining to incentive awards for the three months ended March 31, 2014 and the year ended December 31, 2013, respectively. The corresponding incentive award liability is presented in other liabilities in the accompanying balance sheet.

### Restricted Stock Units

No restricted stock units were granted or were vested in the three months ended March 31, 2014 and years ended December 31, 2013 and 2012. No restricted stock units were outstanding as of December 31, 2013 and March 31, 2014. Nine restricted stock units were outstanding as of December 31, 2012, and had a weighted-average grant date fair value of \$238.50 per share and a weighted-average remaining contractual term of 0.64 years. No expense has been recorded to date related to the Company's restricted stock units, as no restricted stock units have vested. Vesting of the restricted stock units was contingent upon either an initial public offering of the Company's common stock or a change in control.

### Stock-Based Compensation Expense

#### *Employee and Director Expense*

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

	Three Months Ended		Year Ended December 31	
	March 31,		2013	2012
	2014	2013		
	(unaudited)			
Research and development	\$ 146	\$ 6	\$ 184	\$ 26
General and administrative	568	12	691	54
Total	\$ 714	\$ 18	\$ 875	\$ 80

In January 2004, the Company's Board of Directors canceled outstanding employee options under the 1993 Stock Option Plan and replaced them with new options to purchase 1,230 shares of common stock under the 2003 Plan at an exercise price of \$30.21 per share. These replacement options were fully vested on the grant date and are exercisable for ten years, or 18 months after an initial public offering, if earlier. All replacement options are being accounted for as variable from the date of issuance to the date the options are exercised, forfeited or expire. During the years ended December 31, 2013 and 2012, as a result of decreases in the fair market value of its common stock, the Company did not record any compensation expense related to these options. As of March 31, 2014, all replacement options were either exercised or cancelled, therefore the Company is no longer required to assess and record variable compensation expense related to these options.

#### *Non-Employee Expense*

The Company has issued options to purchase shares of common stock to members of its Scientific Advisory Board (SAB) and certain consultants. The stock options have various exercise prices, a term of ten years, and vest over periods up to sixty months. In 2013 and 2012, the Company granted to its SAB members and consultants options to purchase 6,833 and 3,145 shares of common stock, respectively. As of December 31, 2013, options to purchase 4,555 shares of common stock remained unvested, and compensation related to these

[Table of Contents](#)

[Index to Financial Statements](#)

stock options is subject to periodic adjustment as the shares vest. In 2013, the Company also issued an incentive award for 2,335 shares to an SAB member which remained unvested as of December 31, 2013. The Company recorded \$2,000, \$17,000 and \$6,000 of expense in the three months ended March 31, 2014 and years ended December 31, 2013 and 2012, respectively, related to these options and awards.

The Company has not recognized, and does not expect to recognize in the near future, any tax benefit related to employee stock-based compensation costs.

### 12. 401(k) Plan

The Company provides a qualified 401(k) savings plan for its employees. All employees are eligible to participate, provided they meet the requirements of the plan. While the Company may elect to match employee contributions, no such matching contributions have been made through March 31, 2014, December 31, 2013 and 2012.

### 13. Income Taxes

No provision for U.S. income taxes exists due to tax losses incurred in all periods presented. Deferred income taxes reflect the tax effects of net operating loss and tax credit carryforwards and the net temporary differences between the carrying amounts of assets and liabilities for financial reporting and the amounts used for income tax purposes. Significant components of the Company's deferred tax assets are as follows (in thousands):

	December 31	
	2013	2012
Deferred tax assets:		
Federal and state net operating loss carryforwards	\$ 60,569	\$ 62,745
Capitalized research and development	22,349	22,490
Federal and state tax credit carryforwards	6,600	6,153
Other	1,313	1,200
Total deferred tax assets	90,831	92,588
Valuation allowance	(90,831)	(92,588)
Net deferred tax assets	\$ —	\$ —

Realization of the net deferred tax assets is dependent upon future taxable income, if any, the amount and timing of which is uncertain. Based on the weight of available positive and negative objective evidence, management believes it more likely than not that the Company's deferred tax assets are not realizable. Accordingly, the net deferred tax assets have been fully offset by a valuation allowance. The net valuation allowance decreased by \$1.8 million during the year ended December 31, 2013 and increased \$4.7 million during the year ended December 31, 2012.

The following is a reconciliation of the expected statutory federal income tax provision to the actual income tax provision (in thousands):

	December 31	
	2013	2012
Expected income tax benefit at federal statutory tax rate	\$(3,424)	\$(3,826)
Net operating loss reduction	4,441	—
Change in valuation allowance	(1,757)	4,668
State income taxes, net of federal benefit	583	(763)
Permanent items	555	54
Research credits	(396)	—
Other, net	(2)	(133)
Income tax (benefit) expense	\$ —	\$ —

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## [Table of Contents](#)

## [Index to Financial Statements](#)

Pursuant to Internal Revenue Code (“IRC”), Section 382 and 383, use of the Company’s U.S. federal and state net operating loss and research and development income tax credit carryforwards may be limited in the event of a cumulative change in ownership of more than 50.0% within a three-year period. The Company completed an analysis under IRC Sections 382 and 383 through December 31, 2007 and determined that the Company’s net operating losses and research and development credits were subject to limitations due to changes in ownership through December 31, 2007. The net operating loss carryforwards reflected in the deferred tax assets at December 31, 2013 have been adjusted to reflect Section 382 limitations resulting from the ownership change. As the Company was in a net operating loss position for the year 2013 and 2012, the Company has not performed any additional analysis for IRC Sections 382 and 383 for the years ended December 31, 2013 and 2012. There is a risk that additional changes in ownership could have occurred since December 31, 2007. If a change in ownership were to have occurred, additional net operating loss and tax credit carryforwards could be eliminated or restricted. If eliminated, the related asset would be removed from the deferred tax asset schedule with a corresponding reduction in the valuation allowance.

As of December 31, 2013, we had federal net operating loss carryforwards of \$152.1 million and state net operating loss carryforwards of \$152.2 million to offset future taxable income, if any. In addition, we had federal research and development tax credit carryforwards of \$6.2 million and state research and development tax credit carryforwards of \$3.2 million. If not utilized, the federal net operating loss and tax credit carryforwards will expire beginning in 2024 through 2033 and the state net operating loss carryforwards will expire beginning in 2014 through 2033. The state tax credit will carry forward indefinitely.

The following table summarizes activity related to the Company’s gross unrecognized tax benefits (in thousands):

	<b>Total</b>
Balance as of December 31, 2011	\$1,711
Increases related to 2012 tax positions	36
Balance as of December 31, 2012	\$1,747
Increases related to prior year tax positions	65
Increases related to 2013 tax positions	53
Balance as of December 31, 2013	<u>\$1,865</u>

The unrecognized tax benefits, if recognized, would not have an impact on the Company’s effective tax rate. The Company does not expect a significant change to its unrecognized tax benefits over the next twelve months. The unrecognized tax benefits may increase or change during the next year for items that arise in the ordinary course of business.

The Company files income tax returns in the U.S. federal and California jurisdiction and is not currently under examination by federal, state, or local taxing authorities for any open tax years. The tax years 1998 through 2013 remain open to examination by the major taxing authorities.

### **14. 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 \$45,000 and \$60,000 in the years ended December 31, 2013 and 2012, respectively, and \$15,000 for the three months ended March 31, 2014, 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 (Note 11).

### **15. Subsequent Events**

On June 3, 2014, the Company obtained stockholder approval to increase by 500,000 the shares reserved for future issuance under its 2013 equity incentive plan.

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[Table of Contents](#)

[Index to Financial Statements](#)

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**4,000,000 Shares**



**Common Stock**

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**PROSPECTUS**

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**Cowen and Company**

**Stifel**

**Roth Capital Partners**

**National Securities Corporation**

July 21, 2014

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