

Mirati Therapeutics, Inc.  
Form 10-Q  
November 10, 2014  
Table of Contents

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

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FORM 10-Q

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

For the quarterly period ended September 30, 2014

or

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

Commission File Number: 001-35921

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MIRATI THERAPEUTICS, INC.  
(Exact Name of Registrant as Specified in Its Charter)

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Delaware	46-2693615
(State of Incorporation)	(I.R.S. Employer Identification No.)
9363 Towne Centre Drive, Suite 200	
San Diego, California	92121
(Address of Principal Executive Offices)	(Zip Code)
(858) 332-3410	
(Registrant's Telephone Number, Including Area Code)	

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer", "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Securities Exchange Act of 1934.

Large accelerated filer  Accelerated filer

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

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

Total shares of common stock outstanding as of the close of business on November 3, 2014:

Class	Number of Shares Outstanding
Common Stock, \$0.001 par value	13,536,929

Table of Contents

MIRATI THERAPEUTICS, INC.

FORM 10-Q

TABLE OF CONTENTS

PART I. FINANCIAL INFORMATION	3
Item 1. Condensed Consolidated Financial Statements (Unaudited)	3
Condensed Consolidated Balance Sheets as of September 30, 2014 and December 31, 2013	3
Condensed Consolidated Statements of Operations and Comprehensive Loss for the Three and Nine Months Ended September 30, 2014 and 2013	4
Condensed Consolidated Statements of Cash Flows for the Nine Months Ended September 30, 2014 and 2013	5
Notes to Condensed Consolidated Financial Statements	6
Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations	<u>15</u>
Item 3. Quantitative and Qualitative Disclosure about Market Risk	<u>20</u>
Item 4. Controls and Procedures	<u>20</u>
PART II. OTHER INFORMATION	<u>21</u>
Item 1A. Risk Factors	<u>21</u>
Item 2. Unregistered Sale of Securities and Use of Proceeds	<u>41</u>
Item 3. Defaults Upon Senior Securities	<u>41</u>
Item 4. Mine Safety Disclosures	41
Item 5. Other Information	41
Item 6. Exhibits	<u>43</u>
SIGNATURES	<u>44</u>

Table of Contents

## PART I. FINANCIAL INFORMATION

## ITEM 1. Financial Statements

## MIRATI THERAPEUTICS, INC.

## CONDENSED CONSOLIDATED BALANCE SHEETS

(In thousands except for share and per share amounts)

	September 30, 2014 (Unaudited)	December 31, 2013
<b>ASSETS</b>		
Current assets		
Cash and cash equivalents	\$4,915	\$14,235
Short-term investments	32,792	47,835
Other current assets	2,638	2,145
Total current assets	40,345	64,215
Property and equipment, net	524	322
Other long-term assets	440	—
Total assets	\$41,309	\$64,537
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities		
Accounts payable and accrued liabilities	\$5,404	\$5,245
Warrant liability	167	33,407
Total liabilities	5,571	38,652
Stockholders' equity		
Preferred stock, \$0.001 par value, 10,000,000 shares authorized; none issued and outstanding at both September 30, 2014 and December 31, 2013	—	—
Common stock, \$0.001 par value; 100,000,000 authorized; 13,536,929 and 13,446,976 issued and outstanding at September 30, 2014 and December 31, 2013, respectively	14	13
Additional paid-in capital	257,898	214,756
Accumulated other comprehensive income	9,528	9,507
Accumulated deficit	(231,702)	(198,391)
Total stockholders' equity	35,738	25,885
Total liabilities and stockholders' equity	\$41,309	\$64,537
See accompanying notes		

Table of Contents

## MIRATI THERAPEUTICS, INC.

## CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands except for share and per share amounts)

(Unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2014	2013	2014	2013
Expenses				
Research and development	\$7,029	\$5,492	\$19,472	\$15,477
General and administrative	3,519	3,717	8,855	8,636
Restructuring costs	—	—	334	—
Total operating expenses	10,548	9,209	28,661	24,113
Loss from operations	(10,548 )	(9,209 )	(28,661 )	(24,113 )
Other income (expense), net	24	462	(106 )	(789 )
Change in fair value of warrant liability	1,907	(20,596 )	(4,544 )	(16,609 )
Loss before income taxes	(8,617 )	(29,343 )	(33,311 )	(41,511 )
Income tax expense	—	55	—	115
Net loss	\$(8,617 )	\$(29,398 )	\$(33,311 )	\$(41,626 )
Unrealized gain (loss) on available-for-sale investments	(12 )	—	22	—
Comprehensive loss	\$(8,629 )	\$(29,398 )	\$(33,289 )	\$(41,626 )
Basic net loss per share	\$(0.64 )	\$(2.95 )	\$(2.47 )	\$(4.18 )
Diluted net loss per share	\$(0.72 )	\$(2.95 )	\$(2.47 )	\$(4.18 )
Weighted average number of shares used in computing net loss per share, basic	13,527,113	9,957,896	13,478,735	9,957,792
Weighted average number of shares used in computing net loss per share, diluted	14,717,253	9,957,896	13,478,735	9,957,792

See accompanying notes

Table of Contents

MIRATI THERAPEUTICS, INC.  
 CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS  
 (In thousands)  
 (Unaudited)

	Nine months ended September 30,	
	2014	2013
Operating activities:		
Net loss	\$(33,311	) \$(41,626
Non-cash adjustments reconciling net loss to operating cash flows:		
Depreciation of property and equipment	154	105
Amortization of premium on investments	439	—
Share-based compensation expense	4,822	1,097
Change in fair value of warrant liability	4,544	16,609
Change in fair value of share-based compensation liability	—	899
Restructuring costs	13	—
Lease incentive liability	—	(53
Changes in operating assets and liabilities:		
Other current assets	(493	) (57
Other long-term assets	(440	) —
Accounts payable and accrued liabilities	111	1,283
Cash flows used for operating activities	(24,161	) (21,743
Investing activities:		
Purchases of property and equipment	(320	) (196
Purchases of short-term investments	(8,968	) (25,725
Security deposit, restricted cash equivalents and marketable securities	—	(32
Disposal and maturities of short-term investments	23,593	36,887
Cash flows provided by investing activities	14,305	10,934
Financing activities:		
Proceeds from exercise of common stock options	536	22
Cash flows provided by financing activities	536	22
Decrease in cash and cash equivalents	(9,320	) (10,787
Cash and cash equivalents, beginning of period	14,235	18,403
Cash and cash equivalents, end of period	\$4,915	\$7,616
See accompanying notes		

Table of Contents

MIRATI THERAPEUTICS, INC.

Notes to Condensed Consolidated Financial Statements

September 30, 2014

(Unaudited)

1. Description of Business

Mirati Therapeutics, Inc. ("Mirati" or the "Company") is a clinical-stage biopharmaceutical company focused on developing a pipeline of targeted oncology products. The Company focuses its development programs on drugs intended to treat specific subsets of cancer patients with unmet needs.

The Company's common stock has been listed on the NASDAQ Capital Market since July 15, 2013 under the ticker symbol "MRTX." The Company has a wholly owned subsidiary in Canada, MethylGene, Inc. ("MethylGene"). MethylGene's common stock was listed on the Toronto Stock Exchange from June 29, 2004 until July 26, 2013 under the ticker symbol "MYG". The Company also has an indirect, wholly-owned subsidiary, MethylGene US Inc., which was incorporated in Princeton, New Jersey on December 20, 2011 and started business activity in 2012. MethylGene US Inc. has no operations effective January 1, 2014. During the first half of 2013, the Company conducted the majority of its operations through MethylGene and MethylGene US Inc. As a result of the arrangement agreement discussed in Note 2 under the heading "Basis of Presentation," Mirati became the parent company in June 2013 and primary operating company during the last half of 2013. Refer to Note 2 for further discussion of the Company's corporate structure.

2. Summary of Significant Accounting Policies

Basis of Presentation

The unaudited consolidated financial statements contained in this Quarterly Report on Form 10-Q have been prepared in accordance with the rules and regulations of the Securities and Exchange Commission ("SEC") and, therefore, certain information and disclosures normally included in annual financial statements prepared in accordance with U.S. generally accepted accounting principles ("GAAP") have been omitted.

In the opinion of management, the information reflects all adjustments necessary to make the results of operations for the interim periods a fair statement of such operations. All such adjustments are of a normal recurring nature. Interim results are not necessarily indicative of results for the full year. The consolidated balance sheet at December 31, 2013 has been derived from the audited consolidated financial statements at that date, but does not include all information and footnotes required by U.S. generally accepted accounting principles for complete financial statements. These unaudited consolidated financial statements should be read in conjunction with the audited financial statements included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2013 filed with the SEC on March 17, 2014.

Mirati was incorporated under the laws of the State of Delaware on April 29, 2013. The Company was created to enter into an arrangement agreement described below.

On May 8, 2013, the Company's Board of Directors approved and the Company entered into an arrangement agreement with MethylGene. Subject to the terms and conditions of the arrangement agreement, which was consummated on June 28, 2013, the shareholders of MethylGene received one share of the Company's common stock in exchange for every 50 common shares of MethylGene, which had the effect of a 50 for 1 reverse split of MethylGene's common shares pursuant to a court-approved plan of arrangement under Section 192 of the Canada Business Corporations Act. Such transaction is referred to herein as the "Arrangement". In addition, all outstanding options and warrants to purchase common shares of MethylGene became exercisable on a 50-for-1 basis for shares of the Company's common stock, and a proportionate adjustment was made to the exercise price, as applicable. The accompanying financial statements and notes to the financial statements give retroactive effect to the reverse split of our common stock for all periods presented. Upon completion of the Arrangement, MethylGene became the Company's wholly-owned subsidiary. The shares of the Company's common stock issued at the closing of the Arrangement were issued in reliance upon the exemption from registration under Section 3(A)(10) of the Securities

Act of 1933, as amended.

These condensed interim consolidated financial statements are presented in U.S. dollars, which effective January 1, 2013, is also the functional currency of the Company.



## Table of Contents

### Use of Estimates

The preparation of the Company's unaudited consolidated financial statements requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Reported amounts and note disclosures reflect the overall economic conditions that are most likely to occur and anticipated measures management intends to take. Actual results could differ materially from those estimates.

Estimates and assumptions are reviewed quarterly. All revisions to accounting estimates are recognized in the period in which the estimates are revised and in any future periods affected.

### Cash, Cash Equivalents and Short-term Investments

Cash and cash equivalents consist of cash and highly liquid securities with original maturities at the date of acquisition of ninety days or less. Investments with an original maturity of more than ninety days are considered short-term investments and have been classified by management as available-for-sale. These investments are classified as current assets, even though the stated maturity date may be one year or more beyond the current balance sheet date, which reflects management's intention to use the proceeds from sales of these securities to fund its operations, as necessary. Such investments are carried at fair value, with unrealized gains and losses included as a separate component of stockholders' equity. Realized gains and losses from the sale of available-for-sale securities or the amounts, net of tax, reclassified out of accumulated other comprehensive income, if any, are determined on a specific identification basis.

### Foreign Currency Transactions

Foreign currency transactions are initially recorded by the Company using the exchange rates prevailing at the date of the transaction. At the balance sheet date, monetary assets and liabilities denominated in foreign currencies are translated at the period-end rates of exchange. Non-monetary assets and liabilities are translated at the historical exchange rates. Exchange gains and losses arising from the translation of foreign currency items are included in other income (expense), net in the consolidated statements of operations and comprehensive loss. The Company recognized net foreign exchange losses of \$0.2 million for the nine months ended September 30, 2014 which includes an immaterial amount for the three months ended September 30, 2014, and a gain of \$0.4 million and a loss of \$1.0 million for the three and nine months ended September 30, 2013 in other income (expense), net in the consolidated statement of operations and comprehensive loss.

### Reclassification of Warrants

In 2011 and 2012, MethylGene issued common stock warrants in connection with the issuance of common stock through private placements (referred to as the 2011 Warrants and the 2012 Warrants). The exercise prices of the 2011 and 2012 Warrants were denominated in Canadian dollars. Upon the issuance of the 2011 and 2012 Warrants, the net proceeds were allocated to common stock and warrants based on their relative fair values, and the fair value of the issued common stock warrants was calculated utilizing the Black-Scholes option-pricing model. The allocated fair value was then recorded as warrants within stockholders' equity on the consolidated balance sheet.

Effective January 1, 2013, the Company changed its functional currency which changed how the 2011 and 2012 warrants are accounted for as they continued to have exercise prices denominated in Canadian dollars. Upon the change in functional currency, the warrants were classified as a current liability and a warrant liability of \$16.2 million was recorded which represented the fair market value of the warrants at that date in accordance with accounting standards. The initial fair value recorded as warrants within stockholders' equity of \$11.2 million was reversed. The change in fair value related to periods prior to January 1, 2013 of \$5.0 million was recorded as an adjustment to accumulated deficit. At each reporting period subsequent to January 1, 2013, the fair value of the warrant liability is recalculated and any corresponding increase or decrease to the warrant liability is recorded as change in fair value of warrant liability on the consolidated statement of operations and comprehensive loss. The estimated fair value is determined using the Black-Scholes option-pricing model based on the estimated value of the underlying common stock at the valuation measurement date, the remaining contractual term of the warrants, risk-free interest rates, expected dividends and expected volatility of the price of the underlying common stock.

During the third quarter 2014, the Company amended the majority of its outstanding warrant agreements to allow for the warrants to be denominated in U.S. Dollars. As a result of this amendment, the amended warrants qualified for equity classification and were reclassified into stockholders' equity at their fair value as of the amendment date, or \$36.8 million. Consequently due to the equity classification, revaluations of the fair value of the amended warrants are no longer required. The warrants that did not participate in the amendment will continue to be classified as a current liability with quarterly revaluations.

Table of Contents

The fair value of the warrant liability associated with the warrants that did not participate in the amendment was \$0.2 million at September 30, 2014. For all of the warrants classified as liabilities during the relevant periods, the Company recorded a gain of \$1.9 million and a loss of \$4.5 million for the three and nine months ended September 30, 2014, respectively, and a loss of \$20.6 million and a loss of \$16.6 million for the three and nine months ended September 30, 2013, respectively, which are recorded as change in fair value of warrant liability in the consolidated statement of operations and comprehensive loss.

**Segment Reporting**

Operating segments are components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision-maker for purposes of making decisions regarding resource allocation and assessing performance. To date, the Company has viewed its operations and managed its business as one segment operating primarily in the United States.

**Fair value measurements**

The Company has certain financial assets and liabilities recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements.

The authoritative guidance for fair value measurements defines fair value as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or the most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Market participants are buyers and sellers in the principal market that are (i) independent, (ii) knowledgeable, (iii) able to transact, and (iv) willing to transact. The guidance prioritizes the inputs used in measuring fair value into the following hierarchy:

Level 1- Quoted prices (unadjusted) in active markets for identical assets or liabilities;

Level 2- Inputs other than quoted prices included within Level 1 that are either directly or indirectly observable; and

Level 3- Unobservable inputs in which little or no market activity exists, therefore requiring an entity to develop its own assumptions about the assumptions that market participants would use in pricing.

The following table summarizes the assets and liabilities measured at fair value on a recurring basis (in thousands):

	September 30, 2014	Level 1	Level 2	Level 3
<b>Assets</b>				
Cash and cash equivalents	\$4,915	\$2,362	\$2,553	\$—
Short-term investments	32,792	—	32,792	—
	\$37,707	\$2,362	\$35,345	\$—
<b>Liabilities</b>				
Warrant liability	\$167	\$—	\$—	\$167
	\$167	\$—	\$—	\$167

Table of Contents

	December 31, 2013	Level 1	Level 2	Level 3
<b>Assets</b>				
Cash and cash equivalents	\$ 14,235	\$ 12,431	\$ 1,804	\$—
Short-term investments	47,835	—	47,835	—
	\$62,070	\$12,431	\$49,639	\$—
<b>Liabilities</b>				
Warrant liability	\$ 33,407	\$—	\$—	\$ 33,407
	\$33,407	\$—	\$—	\$33,407

The Company's investments in Level 1 assets are valued based on publicly available quoted market prices for identical securities as of September 30, 2014 and December 31, 2013. The Company determines the fair value of Level 2 related securities with the aid of valuations provided by third parties using proprietary valuation models and analytical tools. These valuation models and analytical tools use market pricing or prices for similar instruments that are both objective and publicly available, including matrix pricing or reported trades, benchmark yields, broker/dealer quotes, issuer spreads, two-sided markets, benchmark securities, bids and/or offers. There were no transfers between fair value measurement levels during the three and nine months ended September 30, 2014.

The following table presents a rollforward of the fair value of the warrant liability which includes Level 3 measurements (in thousands):

	Fair Value Measurements at Reporting Date Using Significant Unobservable Inputs (Level 3)
Warrant liability:	
Balance at January 1, 2013	\$—
Fair value upon reclassification of balance as of January 1, 2013	16,194
Change in fair value of warrant liability included in net loss	19,799
Fair value of warrants exercised	(2,586 )
Balance at December 31, 2013	33,407
Change in fair value of warrant liability included in net loss	4,544
Fair value of warrants exercised	(993 )
Reclassification of warrants to stockholders' equity	(36,791 )
Balance at September 30, 2014	167

The Company estimates the fair value of warrants at the time of issuance and subsequent remeasurement using the Black-Scholes option-pricing model at each reporting date, using the following inputs: the risk-free interest rates; the expected dividend rates; the remaining expected life of the warrants; and the expected volatility of the price of the underlying common stock. The estimates are based, in part, on subjective assumptions and could differ materially in the future. Changes to these assumptions can have a significant impact on the fair value of the warrants.

Table of Contents

The following assumptions were used in the Black-Scholes option-pricing model as of the warrant amendments date to determine the fair value of the warrants to reclassify into equity:

	September 12, 2014			
	2011 Warrants		2012 Warrants	
Risk-free interest rate	1.2	%	1.2	%
Volatility	108.6	%	100.8	%
Dividend yield	—		—	
Expected life in years	1.6		3.2	

The following assumptions were used in the Black-Scholes option-pricing model to determine the fair value of the warrant liability for all remaining 2011 warrants. All 2012 warrants were amended and did not require a valuation as of September 30, 2014.

	September 30, 2014		December 31, 2013			
	2011 Warrants		2011 Warrants		2012 Warrants	
Risk-free interest rate	1.1	%	1.2	%	1.6	%
Volatility	108.0	%	112.0	%	115.9	%
Dividend yield	—		—		—	
Expected life in years	1.5		2.3		3.9	

Table of Contents

## Net loss per share

Net loss per share is calculated by dividing the net loss of the Company by the weighted average number of shares of common stock outstanding during the period. The calculation of diluted loss per share requires that, to the extent the average market price of the underlying shares for the reporting period exceeds the exercise price of the warrants and the presumed exercise of such securities are dilutive to loss per share for the period, an adjustment to net loss used in the calculation is required to remove the change in fair value of the warrants from the numerator for the period.

Likewise, an adjustment to the denominator is required to reflect the related dilutive shares, if any, under the treasury stock method. The gain on fair value of warrant liability for the three months ended September 30, 2014 resulted in a dilutive impact as detailed further below:

	Three Months Ended September 30, 2014	
Numerator:		
Net loss	\$(8,617	)
Minus: Gain on fair value of warrant liabilities	1,907	
Adjusted net loss	\$(10,524	)
Denominator:		
Weighted average number of shares used in computing net loss per share, basic	13,527,113	
Plus: Effect of dilutive potential common shares from warrants	1,190,140	
Weighted average number of shares used in computing net loss per share, diluted	14,717,253	
Diluted net loss per share	\$(0.72	)

The following table presents the weighted average number of potentially dilutive securities not included in the calculation of diluted net loss per share due to the anti-dilutive effect of the securities:

	Three Months Ended September 30, 2014		Nine Months Ended September 30, 2014	
	2014	2013	2014	2013
Common stock options	252,678	51,393	264,723	1,436
Common stock warrants	280,720	481,765	1,552,441	92,192
Total	533,398	533,158	1,817,164	93,628

Table of Contents

## 3. Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standard Update ("ASU") 2014-09, Revenue from Contracts with Customers (Topic 606), which will replace numerous requirements in U.S. GAAP, including industry-specific requirements, and provide companies with a single revenue recognition model for recognizing revenue from contracts with customers. The core principle of the new standard is that a company should recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. The new standard will be effective for annual reporting periods beginning after December 15, 2016, including interim periods within that reporting period. This ASU is not expected to have an impact on the Company's financial statements or disclosures.

In August 2014, the FASB issued ASU 2014-15, Presentation of Financial Statements—Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern. Under the new guidance, management will be required to assess an entity's ability to continue as a going concern, and to provide related footnote disclosures in certain circumstances. The provisions of this ASU are effective for annual periods beginning after December 15, 2016, and for annual and interim periods thereafter. The Company is currently evaluating the potential changes from this ASU to its future financial reporting and disclosures.

## 4. Investments

The following tables summarize our short-term investments (dollars in thousands):

	Maturity (in years)	As of September 30, 2014			
		Amortized cost	Gross unrealized gains	Gross unrealized losses	Estimated fair value
Corporate debt securities <sup>(1)</sup>	2 years or less	32,784	11	(3	) 32,792
		32,784	11	(3	) 32,792
As of December 31, 2013					
	Maturity (in years)	Amortized cost	Gross unrealized gains	Gross unrealized losses	Estimated fair value
Government sponsored enterprise <sup>(1)</sup>	2 years or less	3,001	—	(3	) 2,998
Corporate debt securities <sup>(1)</sup>	2 years or less	31,319	—	(22	) 31,297
Commercial paper <sup>(1)</sup>	1 year or less	8,485	12	—	8,497
Guaranteed investment certificates <sup>(2)</sup>	1 year or less	5,046	—	(3	) 5,043
		47,851	12	(28	) 47,835

(1) Investments are designated as available-for-sale investments.

(2) Investments are designated as trading investments.

Unrealized gains and losses on available-for-sale securities are included as a component of comprehensive loss. At September 30, 2014, the Company did not have any securities in material unrealized loss positions. The Company reviews its investments to identify and evaluate investments that have an indication of possible other-than-temporary impairment. Factors considered in determining whether a loss is other-than-temporary include the length of time and extent to which fair value has been less than the cost basis, the financial condition and near-term prospects of the investee, and the Company's intent and ability to hold the investment for a period of time sufficient to allow for any anticipated recovery in market value. The Company does not intend to sell any investments prior to recovery of their amortized cost basis for any investments in an unrealized loss position.





Table of Contents

## 5. Other current assets

Other current assets consisted of the following (in thousands):

	September 30 2014	December 31 2013
Refundable research and development tax credits	\$809	\$809
Interest receivables	224	306
Prepaid expenses	859	667
Commodity taxes and other	290	227
Other	456	136
	\$2,638	\$2,145

## 6. Property and equipment, net

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

	September 30 2014	December 31 2013
Computer equipment	\$325	\$1,534
Office and other equipment	56	122
Laboratory equipment	283	1,794
Leasehold improvements	51	53
	\$715	\$3,503
Less: Accumulated depreciation	(191	) (3,181
	\$524	\$322

The Company incurred depreciation expense of \$39,000 and \$41,000 for the three months ended September 30, 2014 and 2013, and \$0.2 million and \$0.1 million for the nine months ended September 30, 2014 and 2013. During the nine months ended September 30, 2014, in connection with the Company's closure of its Canadian office as described further in Note 8, the Company disposed of several fully depreciated assets with a gross book value of \$3.2 million that were no longer in use.

## 7. Accounts payable and accrued liabilities

Accounts payable and accrued liabilities consisted of the following (in thousands):

	September 30 2014	December 31 2013
Accounts payable	\$1,934	\$1,302
Accrued expenses	2,239	2,335
Accrued compensation and benefits	1,215	1,608
Deferred rent	16	—
	\$5,404	\$5,245

Table of Contents

## 8. Restructuring

On October 1, 2013, the Company announced a plan to terminate approximately 75% of its total workforce in connection with the closing of its Montreal, Quebec and Princeton, New Jersey offices (the "Restructuring") due to the consolidation of Company operations to the San Diego facility. Restructuring activities were reported as a separate line item in the accompanying consolidated statement of operations and comprehensive loss and were comprised of the following components (in thousands):

Balance as of October 1, 2013	\$—	
Charges against the accrual	1,025	
Payments	(729)	)
Balance as of December 31, 2013	296	
Charges against the accrual	334	
Payments	(630)	)
Balance as of September 30, 2014	—	

The restructuring activities associated with the office closures were complete as of September 30, 2014, and all restructuring costs incurred have been paid to date.

## 9. Warrants

As of September 30, 2014 the following warrants for common stock were issued and outstanding:

Issue date	Expiration date	Exercise price		Number of warrants outstanding
April 4, 2011	April 4, 2016	\$6.66	(1)	13,274
April 4, 2011	April 4, 2016	\$6.74	(2)	1,384,647
November 21, 2012	November 21, 2017	\$7.86	(2)	1,067,792
				2,465,713

(1) - Denominated in Canadian Dollars. Exercise price stated in US Dollars as of September 30, 2014.

(2) - Denominated in US Dollars.

On April 4, 2011 and November 21, 2012 the Company's wholly owned subsidiary, MethylGene, completed private placement financing transactions which resulted in the issuance of common stock warrants. The April 4, 2011 common stock warrants were issued with an exercise price of CND\$7.46 and the November 21, 2012 common stock warrants were issued with an exercise price of CND\$8.70. During the third quarter 2014, the Company amended the majority of these outstanding warrant agreements to allow for the warrants to be denominated in U.S. Dollars. During the three and nine months ended September 30, 2014, warrants for 3,620 and 73,964 shares of the Company's common stock were exercised via cashless exercises and the Company issued a net of 2,346 and 43,526 shares.

## 10. Commitments and Contingencies

On June 24, 2014, the Company entered into a lease agreement (the "lease") with ARE-SD Region No. 20, LLC for approximately 18,000 square feet of completed office and laboratory space located in San Diego, California (the "property"). The office space under the lease will serve as the Company's new corporate headquarters, replacing the current facilities. The lease will commence in three phases, with 2,300 square feet of space which commenced on July 1, 2014 at an initial monthly rent of approximately \$5,900 per month, 14,000 square feet of space becoming available on or after February 1, 2015 at an initial monthly rent of \$14,000 per month, and the final 1,600 square feet of space becoming available on February 1, 2016 at an initial monthly rent of approximately \$4,200 per month. Each portion of the leased property will be subject to a 3% annual rent increase following availability. In addition to such base monthly rent, the Company will be obligated to pay triple net lease charges for operating



Table of Contents

expenses, taxes, insurance and utilities applicable to the leased property. The lease will expire on January 31, 2018 with respect to the entire premises.

Future minimum payments required under the lease are summarized as follows (in thousands):

Year Ending December 31:	
2014 (Three months remaining)	\$ 12
2015	224
2016	291
2017	304
2018	25
Total minimum lease payments	\$856

#### 11. Stock-based compensation

Total stock-based compensation expense by operating statement classification is presented below (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Research and development expense	\$859	\$(106)	\$1,929	\$(39)
General and administrative expense	1,732	1,857	2,893	2,035
	\$2,591	\$1,751	\$4,822	\$1,996

In connection with management changes during the first half of 2013, certain unvested stock options were canceled, resulting in a one-time favorable adjustment to stock-based compensation expense. During the three and nine months ended September 30, 2014, 11,778 and 46,427 shares were issued pursuant to stock option exercises amounting to net proceeds of \$0.1 million and \$0.5 million, respectively.

#### ITEM 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited financial statements and related notes included in this Quarterly Report on Form 10-Q and the audited financial statements and notes thereto as of and for the year ended December 31, 2013 and the related Management's Discussion and Analysis of Financial Condition and Results of Operations, both of which are contained in our Annual Report on Form 10-K filed by us with the Securities and Exchange Commission, or SEC, on March 17, 2014.

This Quarterly Report on Form 10-Q may contain "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Such forward-looking statements, which represent our intent, belief, or current expectations, involve risks and uncertainties. We use words such as "may," "will," "expect," "anticipate," "estimate," "intend," "plan," "predict," "potential," "believe," "should" and similar expressions to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements may include, but are not limited to, statements concerning projections about our accounting and finances, plans and objectives for the future, future operating and economic performance and other statements regarding future performance. Although we believe the expectations reflected in these forward-looking statements are reasonable, such statements are inherently subject to risk and we can give no assurances that our expectations will prove to be correct. You should not place undue reliance on these forward-looking statements, which apply only as of the date of this Quarterly Report on Form 10-Q. As a result of many factors, including without limitation those set forth under "Risk Factors" under Item 1A of Part II below, and elsewhere in this Quarterly Report on Form 10-Q, our actual results may differ materially from those anticipated in these forward-looking statements. We undertake no obligation to update these forward-looking statements to reflect events or circumstances after the date of this report or to reflect actual outcomes.

Mirati Therapeutics, Inc., or Mirati, was incorporated under the laws of the State of Delaware on April 29, 2013. On May 8, 2013, our Board of Directors approved and we entered into an arrangement agreement (the "Arrangement") with

15

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## Table of Contents

MethylGene Inc., a corporation incorporated under the Canada Business Corporations Act ("MethylGene"). Subject to the terms and conditions of the arrangement agreement, the shareholders of MethylGene received one share of our common stock in exchange for every 50 common shares of MethylGene, which had the effect of a 50-for-1 reverse split of the common shares pursuant to a court-approved plan of arrangement under Section 192 of the Canada Business Corporations Act. In addition, all outstanding options and warrants to purchase common shares of MethylGene became exercisable on a 50-for-1 basis for shares of our common stock, and a proportionate adjustment was made to the exercise price or conversion price, as applicable. Upon consummation of the Arrangement on June 28, 2013, MethylGene became our wholly-owned subsidiary. As a result, the discussion contained in this Management's Discussion and Analysis of Financial Condition and Results of Operations, including the financial information and related disclosure, reflect the consolidated operations of Mirati. Following the Arrangement, we began to conduct the majority of our business operations through Mirati.

### Overview

#### Company Overview

We are a clinical-stage biopharmaceutical company focused on developing a pipeline of targeted oncology products. We focus our development programs on drugs intended to treat specific subsets of cancer patients with unmet needs. Our pipeline consists of three product candidates: MGCD265, MGCD516 and mocetinostat. MGCD265 and MGCD516 are orally-bioavailable, multi-targeted kinase inhibitors with distinct target profiles that are in development to treat patients with non-small cell lung cancer, or NSCLC, squamous cell carcinoma of the head and neck, or HNSCC, and other solid tumors. MGCD265 is in Phase 1/2 clinical development and we recently opened the expansion cohorts for this program to enroll selected patients with NSCLC and other solid tumors that have certain genetic profiles. MGCD516 is in Phase 1 clinical development which commenced early in the third quarter of 2014. Mocetinostat is an orally-bioavailable, spectrum-selective histone deacetylase, or HDAC, inhibitor currently in Phase 2 clinical development for multiple indications. Based on prior single agent responses in Non Hodgkin's Lymphoma, or NHL, and promising preclinical work we recently initiated mocetinostat single agent Phase 2 trials in patients with NHL and bladder cancer in patient populations selected based upon a genetic defect in histone acetylation. We are also exploring patient selection strategies in an ongoing Phase 2 dose confirmation trial of mocetinostat in combination with Vidaza in myelodysplastic syndrome, or MDS. Mocetinostat was granted Orphan Drug Designation by the FDA in June 2014 for the treatment of patients with MDS in the United States and in August 2014 for the treatment of patients with diffuse large B-cell lymphoma. We continue preparations for a Phase 3 trial in MDS in combination with Vidaza. Our decision to move forward with this trial and the timing of doing so are dependent on the data from the three Phase 2 trials and the establishment of a collaboration for late-stage development or the availability of financing.

#### Liquidity Overview

We have not generated any revenue from product sales. To date, we have funded our operations primarily through the sale of our common stock and through up-front payments, research funding and milestone payments under previous collaborative arrangements. At September 30, 2014, we had \$37.7 million of cash, cash equivalents and short-term investments compared to \$62.1 million at December 31, 2013. We believe that our current cash, cash equivalents and short-term investments will be sufficient to fund our currently planned operations through the end of 2015.

We have incurred losses in each year since our inception. Our net losses were \$8.6 million and \$33.3 million for the three and nine months ended September 30, 2014 and \$29.4 million and \$41.6 million for the three and nine months ended September 30, 2013. As of September 30, 2014, we had an accumulated deficit of \$231.7 million. Substantially all of our operating losses resulted from expenses incurred in connection with our product development programs, our research activities and general and administrative costs associated with our operations.

To fund future operations we will need to raise additional capital. The amount and timing of future funding requirements will depend on many factors, including the timing and results of our ongoing development efforts, the potential expansion of our current development programs, potential new development programs and related general and administrative support. We anticipate that we will seek to fund our operations through public or private equity or debt financings or other sources, such as potential collaboration agreements. We cannot assure that anticipated additional financing will be available to us on favorable terms, or at all. Although we have previously been successful

in obtaining financing through our equity securities offerings, there can be no assurance that we will be able to do so in the future.

**Critical Accounting Policies and Significant Judgments and Estimates**

Our discussion and analysis of financial condition and results of operations are based upon our condensed consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make significant estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses and related disclosures. On an ongoing basis, our actual results may differ significantly from our estimates.

Table of Contents

There have been no material changes to our critical accounting policies and estimates from the information provided in Item 7, Management's Discussion and Analysis of Financial Condition and Results of Operations, included in our Annual Report on Form 10-K for the year ended December 31, 2013.

## Results of Operations

Comparison of the Three and Nine Months Ended September 30, 2014 and 2013

The following table summarizes our results of operations for the three and nine months ended September 30, 2014 and 2013 (in thousands):

	Three months ended		Increase (Decrease)	Nine months ended		Increase (Decrease)
	September 30,			September 30,		
	2014	2013		2014	2013	
Research and development expenses	\$7,029	\$5,492	\$1,537	\$19,472	\$15,477	\$3,995
General and administrative expenses	\$3,519	\$3,717	\$(198)	\$8,855	\$8,636	\$219
Restructuring costs	\$—	\$—	\$—	\$334	\$—	\$334
Other income/(expense), net	\$24	\$462	\$(438)	\$(106)	\$(789)	\$683
Change in fair value of warrant liability	\$1,907	\$(20,596)	\$22,503	\$(4,544)	\$(16,609)	\$12,065

## Research and development expenses

Research and development expenses consist primarily of:

- salaries and related expenses for personnel, including expenses related to stock options or other stock-based compensation granted to personnel in development functions;
- fees paid to external service providers such as Clinical Research Organizations ("CROs") and contract manufacturing organizations related to clinical trials;
- contractual obligations for clinical development, clinical sites, manufacturing and scale-up, and formulation of clinical drug supplies; and
- costs for facilities and amortization of equipment.

We record research and development expenses as incurred. We account for nonrefundable advance payments for goods and services that will be used in future research and development activities as expense when the services have been performed or when the goods have been received. At this time, due to the risks inherent in the clinical development process and the early stage of our product development programs we are unable to estimate with any certainty the costs we will incur in the continued development of MGCD265, MGCD516 and mocetinostat. The process of conducting clinical trials necessary to obtain regulatory approval and manufacturing scale-up to support expanded development and potential future commercialization is costly and time consuming. Any failure by us or delay in completing clinical trials, manufacturing scale up or in obtaining regulatory approvals could lead to increased research and development expense and, in turn, have a material adverse effect on our results of operations.

Our research and development efforts during the three and nine months ended September 30, 2014 and 2013 were focused primarily on our oncology programs, including our two lead kinase programs, MGCD265 and MGCD516, and our HDAC inhibitor, mocetinostat. For the three and nine months ended September 30, 2013, reclassifications were made to certain research and development program expenses line items to conform prior years' financial information to the current presentation. These reclassifications had no impact on total research and development expenses. The following table summarizes our research and development expenses, in thousands:



Table of Contents

	Three months ended September 30,		Nine months ended September 30,	
	2014	2013	2014	2013
Third-party clinical development costs:				
MGCD265	\$1,592	\$1,348	\$5,882	\$5,400
MGCD290	68	435	100	1,613
mocetinostat	1,375	1,072	3,154	1,927
MGCD516	968	1,086	2,056	1,742
Total third-party clinical development costs	4,003	3,941	11,192	10,682
Internal clinical development costs	3,026	1,747	8,280	5,473
Research and development, gross	7,029	5,688	19,472	16,155
Less: Investment tax credits	—	(196	) —	(678
Research and development expense	\$7,029	\$5,492	\$19,472	\$15,477

Research and development expenses for the three months ended September 30, 2014 were \$7.0 million compared to \$5.5 million during the three months ended September 30, 2013. The increase of \$1.5 million for the three months ended September 30, 2014 primarily relates to costs associated with ongoing clinical trials for MGCD265 and mocetinostat, costs associated with the recently initiated clinical trial for MGCD516, increased manufacturing costs related to ongoing development efforts and reduced investment tax credits, or ITCs. The reduction in ITCs is due to a reduction in the total research and development expenses that are eligible for ITCs, due to the closure of our Montreal facility during first quarter of 2014.

Research and development expenses for the nine months ended September 30, 2014 were \$19.5 million compared to \$15.5 million during the nine months ended September 30, 2013. The increase of \$4.0 million for the nine months ended September 30, 2014 reflects an increase in salaries and related expense, which is largely due to increased stock based compensation expense, an increase in expenses related to ongoing clinical trials and related manufacturing expenses, offset by a decrease in costs for MGCD290, for which we are no longer actively pursuing development internally and the absence of costs incurred in 2013 associated with management changes made during the second quarter of 2013.

Clinical development timelines, the probability of success, and development costs can differ materially from expectations, and the results from our clinical trials may not be favorable. If we are successful in progressing MGCD265, MGCD516 and mocetinostat into later stage development, we will require additional capital; however, while we are currently focused on advancing MGCD265, MGCD516 and mocetinostat, the amount and timing of our future research and development expenses will depend on the preclinical and clinical success of both our current development activities and potential development of new product candidates, as well as ongoing assessments of the commercial potential of such activities. We expect that our research and development expenses may increase if we are successful in advancing MGCD265, MGCD516, mocetinostat or any of our preclinical programs into advanced stages of clinical development.

**General and administrative expenses**

General and administrative expenses consist primarily of salaries and related benefits, including stock-based compensation related to our executive, finance, business development, legal and support functions. Other general and administrative expenses include rent and utilities, travel expenses and professional fees for auditing, tax and legal services.

General and administrative expenses for the three and nine months ended September 30, 2014 were \$3.5 million and \$8.9 million compared to \$3.7 million and \$8.6 million for the same periods in 2013. The decrease of \$0.2 million for the three months ended September 30, 2014 is the result of the absence of certain one-time costs incurred in 2013 associated with the listing of our shares of common stock on The NASDAQ Stock Market, LLC (or "NASDAQ"). The increase of \$0.3 million for the nine months ended September 30, 2013 is the result of an increase in salaries and related expense (which is primarily the result of higher stock based compensation costs), offset by one-time costs incurred in 2013 associated with the listing of our shares of common stock on NASDAQ.

**Restructuring costs**

For the nine months ended September 30, 2014, we incurred restructuring costs of \$0.3 million due to the closure of our Montreal, Quebec and Princeton, New Jersey facilities. We did not incur restructuring costs during the second and third quarters of 2014. The offices were closed due to the consolidation of our operations to our San Diego facility, with employee separation charges accounting for the majority of the restructuring costs. The restructuring activities associated with the office closures were substantially complete as of March 31, 2014.

## Table of Contents

### Change in fair value of warrant liability

The change in fair value of warrant liability represents expense or income associated with fair value adjustments to the warrant liability that were recorded during the period. During the three and nine months ended September 30, 2014, we recorded a gain of \$1.9 million and expense of \$4.5 million, respectively, and during the three and nine months ended September 30, 2013, we recorded expense of \$20.6 million and expense of \$16.6 million, respectively, associated with the change in fair value of warrant liability. During the third quarter 2014, we amended the majority of the outstanding warrant agreements to allow for the warrants to be denominated in U.S. Dollars. As a result of this amendment, the amended warrants qualified for equity classification and were reclassified into stockholders' equity at their fair value as of the amendment date, or \$36.8 million. Consequently due to the equity classification, revaluations of the fair value of the amended warrants are no longer required. The warrants that did not participate in the amendment will continue to be classified as a current liability with quarterly revaluations.

### Other income (expense), net

Other income (expense), net consists primarily of interest income and foreign exchange gains and losses. Other income (expense), net for the three months ended September 30, 2014 and 2013 was income of \$24 thousand and \$0.5 million, respectively. Other income (expense), net for the nine months ended September 30, 2014 and 2013 was expense of \$0.1 million and \$0.8 million, respectively. The decrease in expense for both the three and nine month periods primarily reflects the impact of foreign exchange rates as we fully operated in U.S. dollars for the nine months ended September 30, 2014.

### Liquidity and Capital Resources

To date, we have funded our operations primarily through the sale of our common stock and through up-front payments, research funding and milestone payments under previous collaborative arrangements. Since inception, we have primarily devoted our resources to funding research and development programs, including discovery research, preclinical and clinical development activities.

At September 30, 2014, we had \$37.7 million of cash, cash equivalents and short-term investments compared to \$62.1 million at December 31, 2013. We believe that our current cash, cash equivalents and short-term investments will fund our currently planned operations through the end of 2015.

To fund future operations we will need to raise additional capital. The amount and timing of future funding requirements will depend on many factors, including the timing and results of our ongoing development efforts, the potential expansion of our current development programs, potential new development programs and related general and administrative support. We anticipate that we will seek to fund our operations through public or private equity or debt financings or other sources, such as potential collaboration agreements. We cannot assure that anticipated additional financing will be available to us on favorable terms, or at all. Although we have previously been successful in obtaining financing through our equity securities offerings, there can be no assurance that we will be able to do so in the future.

### Cash Flows for the Nine Months Ended September 30, 2014 and 2013

#### Operating Activities

Cash used for operating activities for the nine months ended September 30, 2014 was \$24.2 million, compared to \$21.7 million for the nine months ended September 30, 2013, an increase of \$2.6 million. The current period increase in operating expenses reflects increased research and development expenses and general and administrative expenses, as discussed in "Results of Operations."

#### Investing Activities

Investing activities consist primarily of purchases, sales and maturities of short-term investments. Investing activities provided cash of \$14.3 million due to the sale and maturity of short-term investments in 2014 and \$10.9 million in 2013.

#### Financing Activities

Financing activities for the nine months ended September 30, 2014 consisted of proceeds from the exercise of stock options and generated cash flows of \$0.5 million compared to \$22,000 for the nine months ended September 30, 2013.

#### Off-Balance Sheet Arrangements

During the nine months ended September 30, 2014, we did not have any off-balance sheet arrangements (as defined by applicable SEC regulations) that are reasonably likely to have a current or future material effect on our financial condition, results of operations, liquidity, capital expenditures or capital resources.

## Table of Contents

### Recent Accounting Pronouncements

Occasionally, new accounting standards are issued or proposed by the Financial Accounting Standards Board, or other standard-setting bodies that we adopt by the effective date specified within the standard. Unless otherwise discussed, standards that do not require adoption until a future date are not expected to have a material impact on our financial statements upon adoption.

### ITEM 3. Quantitative and Qualitative Disclosures about Market Risk

#### Interest Rate Risk

Some of our short-term investments have market risk in that a change in prevailing interest rates may cause the principal amount of the investment to fluctuate. Financial instruments that potentially subject us to significant concentrations of credit risk consist primarily of cash, cash equivalents and short-term investments. We invest our excess cash primarily in commercial paper and debt instruments of financial institutions, corporations, U.S. government-sponsored agencies and the U.S. Treasury. We mitigate credit risk by maintaining a well-diversified portfolio and limiting the amount of investment exposure as to institution, maturity and investment type. We invest our excess cash in accordance with our investment policy.

Because of the short-term maturities of our cash equivalents and short-term investments, we do not believe that an increase in market rates would have any significant impact on the realized value of our investments. If a 10% change in interest rates were to have occurred on September 30, 2014, this change would not have had a material effect on the fair value of our investment portfolio as of that date.

#### Effects of Inflation

We do not believe that inflation and changing prices had a significant impact on our results of operations for any periods presented herein.

### ITEM 4. Controls and Procedures

#### Evaluation of Disclosure Controls and Procedures

As required by Rule 13a-15(b) and Rule 15d-15(b) of the Exchange Act, our management, including our principal executive officer and our principal financial officer, conducted an evaluation as of the end of the period covered by this Quarterly Report on Form 10-Q of the effectiveness of the design and operation of our disclosure controls and procedures. Based on that evaluation, management has concluded that as of March 31, 2013, the Company's disclosure controls and procedures were not effective due to a material weakness in internal control over financial reporting associated with the restatement of the calculation and disclosure of diluted loss per share for the three months ended March 31, 2013. As described below, management presented a remediation plan to the Company's Audit Committee. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis. Despite the existence of this material weakness, we believe the condensed consolidated financial statements included in this Form 10-Q for the quarter and nine months ended September 30, 2014 present, in all material respects, our financial position, results of operations, comprehensive loss and cash flows for the periods presented in conformity with U.S. generally accepted accounting principles.

#### Changes in Internal Control over Financial Reporting

As required by Rule 13a-15(d) and Rule 15d-15(d) of the Exchange Act, our management, including our principal executive officer and our principal financial officer, conducted an evaluation of the internal control over financial reporting to determine whether any changes occurred during the period covered by this Quarterly Report on Form 10-Q that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting. Based on that evaluation, our principal executive officer and principal financial officer concluded that, other than described below, there were no changes in our internal control over financial reporting during the period covered by this Quarterly Report on Form 10-Q that materially affected, or were reasonably likely to materially affect, our internal control over financial reporting.

In response to the material weakness in our internal controls noted in our Form 10Q filed with the SEC on May 14, 2014, on August 7, 2014, our Audit Committee approved management's remediation plan. The remediation plan adds

a new financial reporting process control for the calculation of diluted earnings per share. Our management is responsible for implementing the internal control remediation plan. The material weakness will not be considered remediated until the applicable remedial controls operate for a sufficient period of time and management has concluded, through testing, that these controls are operating effectively.

Table of Contents

PART II-OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS.

None.

ITEM 1A. Risk Factors.

You should consider carefully the following information about the risks described below, together with the other information contained in this Quarterly Report and in our other public filings in evaluating our business. The risk factors set forth below with an asterisk (\*) next to the title contain changes to the description of the risk factors associated with our business previously disclosed in Item 1A. of our annual report on Form 10-K for the year ended December 31, 2013. Additional risks and uncertainties that we are unaware of may also become important factors that affect us. If any of the following risks actually occurs, our business, financial condition, results of operations and future growth prospects would likely be materially and adversely affected. In these circumstances, the market price of our common stock would likely decline.

Risks Relating to Our Financial Position and Capital Requirements

\* We will require additional financing and may be unable to raise sufficient capital, which could lead us to delay, reduce or abandon development programs or commercialization.

Our operations have consumed substantial amounts of cash since inception. Our research and development expenses were \$7.0 million and \$5.5 million for the three months ended September 30, 2014 and 2013, respectively, and \$19.5 million and \$15.5 million for the nine months ended September 30, 2014 and 2013, respectively. We believe that our current cash and cash equivalents and short-term investments will sustain our operations through the end of 2015. Pursuant to our current plans, we do not anticipate initiating Phase 3 clinical trials with mocetinostat until data from our Phase 2 clinical trials is available and absent additional financing or the establishment of a collaboration for late-stage development. We have based these estimates on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. We will require substantial additional capital to pursue additional clinical development for our lead clinical programs, including conducting late-stage clinical trials, manufacturing clinical supplies and potentially developing other assets in our pipeline, and, if we are successful, to commercialize any of our current product candidates. If the FDA or any foreign regulatory agency, such as the European Medicines Agency, or EMA, requires that we perform studies or trials in addition to those that we currently anticipate with respect to the development of our product candidates, or repeat studies or trials, our expenses would further increase beyond what we currently expect. Any delay resulting from such further or repeat studies or trials could also result in the need for additional financing. We may not be able to adequately finance our development programs, which could limit our ability to move our programs forward in a timely and satisfactory manner or require us to abandon the programs, any of which would harm our business, financial condition and results of operations. Because successful development of our product candidates is uncertain, we are unable to estimate the actual funds we will require to complete research and development and commercialize our product candidates.

If we are unable to obtain funding from equity offerings or debt financings on a timely basis, we may be required to (1) seek collaborators for one or more of our product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available; (2) relinquish or license on unfavorable terms our rights to technologies or product candidates that we otherwise would seek to develop or commercialize ourselves; or (3) significantly curtail one or more of our research or development programs or cease operations altogether. We are a clinical-stage company with no approved products and no historical product revenue. Consequently, we expect that our financial and operating results will vary significantly from period to period.

We are a clinical-stage company that has incurred losses since its inception and expect to continue to incur substantial losses in the foreseeable future. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of uncertainty.

Our actual financial condition and operating results have varied significantly in the past and are expected to continue to fluctuate significantly from quarter-to-quarter or year-to-year due to a variety of factors, many of which are beyond

our control. Factors relating to our business that may contribute to these fluctuations include:

- the success of our clinical trials through all phases of clinical development;
- delays in the commencement, enrollment and timing of clinical trials;



Table of Contents

our ability to secure and maintain collaborations, licensing or other arrangements for the future development and/or commercialization of our product candidates, as well as the terms of those arrangements;

our ability to obtain, as well as the timeliness of obtaining, additional funding to develop our product candidates;

the results of clinical trials or marketing applications for product candidates that may compete with our product candidates;

competition from existing products or new products that may receive marketing approval;

potential side effects of our product candidates that could delay or prevent approval or cause an approved drug to be taken off the market;

any delays in regulatory review and approval of our product candidates;

our ability to identify and develop additional product candidates;

the ability of patients or healthcare providers to obtain coverage or sufficient reimbursement for our products;

our ability, and the ability of third parties such as Clinical Research Organizations, or CROs, to adhere to clinical study and other regulatory requirements;

the ability of third-party manufacturers to manufacture our product candidates and key ingredients needed to conduct clinical trials and, if approved, successfully commercialize our products;

the costs to us, and our ability as well as the ability of any third-party collaborators, to obtain, maintain and protect our intellectual property rights;

costs related to and outcomes of potential intellectual property litigation;

our ability to adequately support future growth;

our ability to attract and retain key personnel to manage our business effectively; and

our ability to build our finance infrastructure and, to the extent required, improve our accounting systems and controls.

Accordingly, the likelihood of our success must be evaluated in light of many potential challenges and variables associated with a clinical-stage company, many of which are outside of our control, and past operating or financial results should not be relied on as an indication of future results. Fluctuations in our operating and financial results could cause our share price to decline. It is possible that in some future periods, our operating results will be above or below the expectations of securities analysts or investors, which could also cause our share price to decline.

\* We have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future. We have never generated any revenue from product sales and may never be profitable.

We have derived limited revenue from our research and licensing agreements which has not been sufficient to cover the substantial expenses we have incurred in our efforts to develop our product candidates. Consequently, we have accumulated net losses since inception in 1995. Our net loss for the three and nine months ended September 30, 2014 and 2013 was \$8.6 million and \$29.4 million and \$33.3 million and \$41.6 million, respectively. As of September 30, 2014, we had an accumulated deficit of \$231.7 million. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital. Such losses are expected to increase in the future as we continue the development of our product candidates and seek regulatory approval and commercialization for our product candidates. We are unable to predict the extent of any future losses or when we will become profitable, if ever. Even if we do achieve profitability, we may not be able to sustain or increase profitability on an ongoing basis.

We do not anticipate generating revenue from sales of products for the foreseeable future, if ever. If any of our product candidates fail in clinical trials or do not gain regulatory approval, or if any of our product candidates, if approved, fail to achieve market acceptance, we may never become profitable. If one or more of our product candidates is approved for commercial sale and we retain commercial rights, we anticipate incurring significant costs associated with commercializing any such approved product candidate. Therefore, even if we are able to generate revenue from the sale of any approved product, we may never become profitable. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our ability to generate future revenue from product sales depends heavily on our success in:

completing development and clinical trial programs for our product candidates;

entering into collaboration and license agreements;

22

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Table of Contents

seeking and obtaining marketing approvals for any product candidates that successfully complete clinical trials; establishing and maintaining supply and manufacturing relationships with third parties; successfully commercializing any product candidates for which marketing approval is obtained; and successfully establishing a sales force and marketing and distribution infrastructure.

Raising additional funds through debt or equity financing will be dilutive and raising funds through licensing agreements may be dilutive, restrict operations or relinquish proprietary rights.

To the extent that additional capital is raised through the sale of equity or convertible debt securities, the issuance of those securities could result in substantial dilution for our current stockholders and the terms may include liquidation or other preferences that adversely affect the rights of our current stockholders. Existing stockholders may not agree with our financing plans or the terms of such financings. Moreover, the incurrence of debt financing could result in a substantial portion of our operating cash flow being dedicated to the payment of principal and interest on such indebtedness and could impose restrictions on our operations. In addition, if we raise additional funds through collaboration and licensing arrangements, it may be necessary to relinquish potentially valuable rights to our products or proprietary technologies, or to grant licenses on terms that are not favorable to us. Additional funding may not be available to us on acceptable terms, or at all.

\* We may incur losses associated with foreign currency fluctuation.

Our headquarters were previously located in Canada and many of our material contracts were entered into in Canada. A significant portion of our expenditures are in foreign currencies, most notably in Canadian dollars; therefore, we are subject to foreign currency fluctuations which may, from time to time, impact (positively or negatively) our financial position and results of operations. Exchange rates can fluctuate significantly and cannot be easily predicted; thus, we may experience significant shifts in currency exchange variances in the future. We maintain bank accounts in both Canadian dollars and U.S. dollars and do not hedge our positions. Our functional currency at September 30, 2014 was the U.S. dollar. Prior to January 1, 2013 our functional currency was the Canadian dollar.

\* As a public company in the United States, we are subject to the Sarbanes-Oxley Act. We can provide no assurance that we will, at all times, in the future be able to report that our internal controls over financial reporting are effective. Companies that file reports with the Securities and Exchange Commission, or the SEC, including us, are subject to the requirements of Section 404 of the Sarbanes-Oxley Act of 2002. Section 404 requires management to establish and maintain a system of internal control over financial reporting, and annual reports on Form 10-K filed under the Securities Exchange Act of 1934, as amended, or the Exchange Act, must contain a report from management assessing the effectiveness of a company's internal control over financial reporting. Ensuring that we have adequate internal financial and accounting controls and procedures in place to produce accurate financial statements on a timely basis remains a costly and time-consuming effort that needs to be re-evaluated frequently. Failure on our part to have effective internal financial and accounting controls would cause our financial reporting to be unreliable, could have a material adverse effect on our business, operating results, and financial condition, and could cause the trading price of our common stock to fall dramatically. We and our independent registered public accounting firm have identified a material weakness in our internal controls that is described in greater detail in "Item 4-Controls and Procedures." We are in the process of implementing measures designed to improve our internal control over financial reporting to remediate the control deficiencies that led to our material weakness. We cannot assure you that the measures we have taken to date, or any measures we may take in the future, will be sufficient to remediate the control deficiencies that led to our material weakness in our internal control over financial reporting or to avoid potential future material weaknesses. In addition, our independent registered public accounting firm has never performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act because no such evaluation has been required. Had our independent registered public accounting firm performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act, additional significant deficiencies or material weaknesses may have been identified. If we are unable to successfully remediate any significant deficiency or material weakness in our internal control over financial reporting, or identify any additional significant deficiencies or material weaknesses that may exist, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports in addition to applicable stock exchange listing requirements, investors may

lose confidence in our financial reporting, and our stock price may decline as a result.

As a "smaller reporting company" (as defined in the Exchange Act) we will be required to comply with Section 404 of the Sarbanes-Oxley Act although, as an "emerging growth company" (as defined in the JOBS Act) and a smaller reporting company, we are not required to comply with Section 404(b) which requires attestation from our external auditors on our internal control over financial reporting. We are subject to Section 404(a), which requires management to provide a report regarding the effectiveness of internal

Table of Contents

controls. We were previously listed on the Toronto Stock Exchange, or TSX, from June 2004 until July 2013 and were subject to similar governance requirements under Multi-lateral Instrument 52-109. We are required to review all of our control processes to align them to the SOX 404 requirements. Failure to provide assurance that our financial controls are effective could lead to lack of confidence by investors which could lead to a lower share price. When and if we become a "large accelerated filer" or an "accelerated filer" and are no longer an "emerging growth company" (each as defined in the Exchange Act or the Securities Act of 1933, as amended, or the Securities Act), our independent registered public accounting firm will be required to attest to the effectiveness of our internal control over financial reporting. The rules governing the standards that must be met for management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation. To comply with the requirements of being a reporting company under the Exchange Act, we may need to upgrade our systems including information technology, implement additional financial and management controls, reporting systems and procedures, and hire additional accounting and finance staff.

We will incur significant increased costs as a result of operating as a U.S. public company and continuing to be a Canadian "reporting issuer."

Although we de-listed from the TSX effective as of July 26, 2013, we will continue to be subject to Canadian reporting obligations until we meet certain prescribed thresholds which would allow us to apply to cease being a Canadian "reporting issuer." We may incur significant additional accounting, reporting and other expenses in order to maintain our listing on The NASDAQ Capital Market, and fulfill our obligations as a Canadian "reporting issuer." As a U.S. listed public company, we incur significant additional legal, accounting and other expenses that we did not incur as a company listed on the TSX. Shareholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and 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, any new regulations or disclosure obligations may increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

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 JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will 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 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. If we do continue to be an emerging growth company, 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 rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less-active trading market for our common stock and our stock price may be more volatile.

We will remain an emerging growth company until the earliest of (1) 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, (2) the end of the fiscal year in which we have total annual gross revenue of \$1 billion or more during such fiscal year, (3) the date on which we issue more than \$1 billion in non-convertible debt in a three-year period or (4) 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.

Decreased disclosures in our SEC filings due to our status as an emerging growth company may make it harder for investors to analyze our results of operations and financial prospects.

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

We are currently a "smaller reporting company," meaning that we are not an investment company, an asset-backed issuer, or a majority-owned subsidiary of a parent company that is not a smaller reporting company and had a public float of less than \$75 million and annual revenue of less than \$50 million during the most recently completed fiscal year. We will not be a smaller

## Table of Contents

reporting company as of 2015. However, similar to emerging growth companies, smaller reporting companies are able to provide simplified executive compensation disclosures in their filings, are exempt from the provisions of Section 404(b) of the Sarbanes-Oxley Act requiring that independent registered public accounting firms provide an attestation report on the effectiveness of internal control over financial reporting, and have certain other decreased disclosure obligations in their SEC filings, including, among other things, only being required to provide two years of audited financial statements in annual reports.

Decreased disclosures in our SEC filings due to our status as a smaller reporting company may make it harder for investors to analyze our results of operations and financial prospects.

### Risks Relating to Our Business and Industry

\* Our research and development programs and product candidates are at an early stage of development. As a result we are unable to predict if or when we will successfully develop or commercialize our product candidates.

Our clinical-stage product candidates as well as our other pipeline assets are at an early stage of development and will require significant further investment and regulatory approvals prior to commercialization. We currently have no product candidates beyond Phase 2 clinical trials. MGCD265 is currently in Phase 1 and Phase 1/2 clinical trials, mocetinostat is currently in Phase 2 clinical trials and MGCD516 is in Phase 1 clinical trial. Each of our product candidates will require additional clinical development, management of clinical, preclinical and manufacturing activities, obtaining regulatory approval, obtaining manufacturing supply, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenues from product sales. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. In addition, some of our product development programs contemplate the development of companion diagnostics. Companion diagnostics are subject to regulation as medical devices and we may be required to obtain marketing approval for accompanying companion diagnostics before we may commercialize our product candidates. We plan on conducting a dose confirmation trial and obtaining a Special Protocol Assessment, or SPA, with the FDA prior to initiating Phase 3 trials with mocetinostat. In addition, we do not anticipate initiating a Phase 3 clinical trial with mocetinostat absent additional financing or the establishment of a collaboration for late-stage development.

Even if we obtain the required financing or establish a collaboration to enable us to conduct late-stage clinical development of our product candidates and pipeline assets, we cannot be certain that such clinical development would be successful, or that we will obtain regulatory approval or be able to successfully commercialize any of our product candidates and generate revenue. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and the clinical trial process may fail to demonstrate that our product candidates are safe and effective for their proposed uses. Any such failure could cause us to abandon further development of any one or more of our product candidates and may delay development of other product candidates. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Any delay in, or termination of, our clinical trials will delay and possibly preclude the filing of any new drug applications, or NDAs, with the FDA and, ultimately, our ability to commercialize our product candidates and generate product revenue.

We have not previously submitted an NDA to the FDA, or similar drug approval filings to comparable foreign authorities, for any product candidate, and we cannot be certain that any of our product candidates will receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market one or more of our product candidates, our revenues will be dependent, in part, upon our or our future collaborators' ability to obtain regulatory approval of the companion diagnostics to be used with our product candidates, if required, as well as the size of the markets in the territories for which we gain regulatory approval and have commercial rights. If the markets for patient subsets that we are targeting are not as significant as we estimate, we may not generate significant revenues from sales of such

products, if approved.

All of our product candidates are subject to extensive regulation, which can be costly and time consuming, cause delays or prevent approval of such product candidates for commercialization.

The clinical development of product candidates is subject to extensive regulation by the FDA in the United States and by comparable regulatory authorities in foreign markets. Product development is a very lengthy and expensive process, and its outcome is inherently uncertain. The product development timeline can vary significantly based upon the product candidate's novelty and complexity. Regulations are subject to change and regulatory agencies have significant discretion in the approval process.



## Table of Contents

Numerous statutes and regulations govern human testing and the manufacture and sale of human therapeutic products in the United States, Canada and other countries where we intend to market our products. Such legislation and regulation bears upon, among other things, the approval of protocols and human testing, the approval of manufacturing facilities, safety of the product candidates, testing procedures and controlled research, review and approval of manufacturing, preclinical and clinical data prior to marketing approval including adherence to good manufacturing practices, or GMP, during production and storage as well as regulation of marketing activities including advertising and labeling.

In order to obtain regulatory approval for the commercial sale of any of our product candidates, we must demonstrate through preclinical studies and clinical trials that the potential product is safe and effective for use in humans for each target indication. The failure to adequately demonstrate the safety and efficacy of a product under development could delay or prevent regulatory approval of our product candidates.

No assurance can be given that current regulations relating to regulatory approval will not change or become more stringent in the United States or foreign markets. Regulatory agencies may also require that additional trials be run in order to provide additional information regarding the safety or efficacy of any compound for which we seek regulatory approval. Moreover, any regulatory approval of a drug which is eventually obtained may entail limitations on the indicated uses for which that drug may be marketed. Furthermore, product approvals may be withdrawn or limited in some way if problems occur following initial marketing or if compliance with regulatory standards is not maintained. Regulatory agencies could become more risk adverse to any side effects or set higher standards of safety and efficacy prior to reviewing or approving a product. This could result in a product not being approved. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

We may not be successful in establishing development and commercialization collaborations which could adversely affect, and potentially prohibit, our ability to develop our product candidates.

Because developing pharmaceutical products, conducting clinical trials, obtaining regulatory approval, establishing manufacturing capabilities and marketing approved products are expensive, we may seek to enter into collaborations with companies that have more resources and experience in order to continue to develop and commercialize our product candidates. We also may be required due to financial or scientific constraints to enter into additional corporate collaboration agreements to research and/or to develop and commercialize our product candidates. The establishment and realization of such collaborations may be not be possible or may be problematic. There can be no assurance that we will be able to establish such additional collaborations on favorable terms, if at all, or that our current or future collaborative arrangements will be successful or maintained for any specific product candidate or indication. If we are unable to reach successful agreements with suitable partners for the ongoing development and commercialization of our product candidates, we may face increased costs, we may be forced to limit the scope and number of our product candidates we can commercially develop or the territories in which we commercialize such product candidates and we may be unable to commercialize products or programs for which a suitable partner cannot be found. If we fail to achieve successful partnerships, our operating results and financial condition will be materially and adversely affected. In addition, the terms of any collaboration agreements may place restrictions on our activities with respect to other products, including by limiting our ability to grant licenses or develop products with other third parties, or in different indications, diseases or geographical locations, or may place additional obligations on us with respect to development or commercialization of our product candidates. If we fail to comply with or breach any provision of a collaborative or license agreement, a collaborator may have the right to terminate, in whole or in part, such agreement or to seek damages.

Some of our collaboration agreements are complex and involve sharing or division of ownership of certain data, know-how and intellectual property rights among the various parties. Accordingly our collaborators could interpret certain provisions differently than we or our other partners which could lead to unexpected or inadvertent disputes with partners. In addition, these agreements might make additional partnering or mergers and acquisitions difficult. There is no assurance that a collaborator who is acquired by a third party would not attempt to change certain contract provisions that could negatively affect our collaboration. The acquiring company may also not accept the terms or assignment of our contracts and may seek to terminate the agreements. Any one of our partners could breach covenants, restrictions and/or sub-license agreement provisions leading us into disputes and potential breaches of our

agreements with other partners.

\* If we or third parties are unable to successfully develop companion diagnostics for our kinase inhibitor product candidates, or experience significant delays in doing so, we may not achieve marketing approval or realize the full commercial potential of such product candidates.

A key part of our strategy for our kinase inhibitor and HDAC development program, including MGCD265, MGCD516, and mocetinostat is to identify patients or types of tumors that express specific genetic markers, which will require the use and development of companion diagnostics. We expect that the FDA and comparable foreign regulatory authorities will require the regulatory approval of a companion diagnostic as a condition to approving these product candidates. We do not have experience

Table of Contents

or capabilities in developing or commercializing diagnostics and plan to rely in large part on third parties to perform these functions. We do not currently have any long-term arrangements in place with any third party to develop or commercialize companion diagnostics for any of our therapeutic product candidates.

Companion diagnostics are subject to regulation by the FDA and comparable foreign regulatory authorities as medical devices and will likely require separate regulatory approval prior to commercialization.

If we or third parties are unable to successfully develop companion diagnostics for our kinase inhibitor or HDAC product candidates, or experience delays in doing so:

- the development of these product candidates may be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials;

- these product candidates may not receive marketing approval if their safe and effective use depends on a companion diagnostic; and

- we may not realize the full commercial potential of these product candidates that receive marketing approval if, among other reasons, we are unable to appropriately identify patients or types of tumors with the specific genetic alterations targeted by these product candidates.

Even if our HDAC or kinase inhibitor product candidates and any associated companion diagnostics are approved for marketing, the need for companion diagnostics may slow or limit adoption of our product candidates. Although we believe genetic testing is becoming more prevalent in the diagnosis and treatment of cancer, our product candidates may be perceived negatively compared to alternative treatments that do not require the use of companion diagnostics, either due to the additional cost of the companion diagnostic or the need to complete additional procedures to identify genetic markers prior to administering our product candidates.

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

We may not be able to obtain an SPA prior to initiating Phase 3 clinical trials of mocetinostat. Even if obtained, an SPA would not guarantee any particular outcome from regulatory review.

We plan to submit an SPA to the FDA prior to initiating any Phase 3 development of mocetinostat. The FDA's SPA process creates a written agreement between the sponsoring company and the FDA regarding clinical trial design and other clinical trial issues that can be used to support approval of a product candidate. The SPA is intended to provide assurance that if the agreed upon clinical trial protocols are followed and the clinical trial endpoints are achieved, the data may serve as the primary basis for an efficacy claim in support of an NDA. However, SPA agreements are not a guarantee of an approval of a product candidate or any permissible claims about the product candidate. In particular, SPAs are not binding on the FDA if previously unrecognized public health concerns arise during the performance of the clinical trial, if other new scientific concerns regarding product candidate safety or efficacy arise or if the sponsoring company fails to comply with the agreed upon clinical trial protocols. We cannot guarantee that we will obtain an SPA for the Phase 3 development of mocetinostat or that an SPA, if obtained, would ultimately aid in obtaining regulatory approval.

We rely upon third-party contractors and service providers for the execution of some aspects of our development programs. Failure of these collaborators to provide services of a suitable quality and within acceptable timeframes may cause the delay or failure of our development programs.

We outsource certain functions, tests and services to CROs, medical institutions and collaborators as well as outsourcing manufacturing to collaborators and/or contract manufacturers and we rely on third parties for quality assurance, clinical monitoring, clinical data management and regulatory expertise. In particular, we rely on CROs to run our clinical trials on our behalf. There is no assurance that such individuals or organizations will be able to provide the functions, tests, drug supply or services as agreed upon or to acceptable quality standards, and we could suffer significant delays in the development of our products or processes.

In some cases there may be only one or few providers of such services, including clinical data management or manufacturing services. In addition, the cost of such services could increase significantly over time. We rely on third parties as mentioned above to enroll qualified patients and conduct, supervise and monitor our clinical trials. Our reliance on these third parties and collaborators for clinical development activities reduces our control over these activities, but does not relieve us of our regulatory responsibilities, including ensuring that our clinical trials are conducted in accordance with good clinical practices, or GCP, regulations and the investigational plan and protocols

contained in the regulatory agency applications. In addition, these third parties may not complete activities on schedule or may not manufacture compounds under GMP conditions. Preclinical studies may not be performed or completed in accordance with good laboratory practices, or GLP, regulatory requirements or our trial design. If we or our CROs fail to comply with GCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, the EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials

Table of Contents

before approving any marketing applications. If these third parties or collaborators do not successfully carry out their contractual duties or meet expected deadlines, obtaining regulatory approval for manufacturing and commercialization of our product candidates may be delayed or prevented. We rely substantially on third-party data managers for our clinical trial data. There is no assurance that these third parties will not make errors in the design, management or retention of our data or data systems. There is no assurance these third parties will pass FDA or regulatory audits, which could delay or prohibit regulatory approval.

Our CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other product development activities, which could harm our competitive position. If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. Further, switching or adding additional CROs involves additional cost and requires management time and attention. In addition, there is a natural transition period when a new CRO commences work. As a result, delays may occur, which could materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

The timelines of our clinical trials may be impacted by numerous factors and any delays may adversely affect our ability to execute our current business strategy.

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 a delay or unsuccessful completion of clinical trials include:

- 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 CROs and clinical trial sites;
- delays in obtaining required institutional review board 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;
- clinical sites dropping out of a trial to the detriment of enrollment;
- time required to add new clinical sites; and
- delays by our contract manufacturers to produce and deliver a sufficient supply of clinical trial materials.

For example, due to the targeted indications and patient population we intend to focus on for development of our kinase inhibitor product candidates, the number of study sites and patient populations available to us may be relatively limited, and therefore enrollment of suitable patients to participate in clinical trials for these product candidates may take longer than would be the case if we were pursuing broader indications or patient populations.

If initiation or completion of any of our clinical trials for our product candidates are delayed for any of the above reasons, our development costs may increase, our approval process could be delayed, any periods after commercial launch and before expiration of patent protection may be reduced and our competitors may have more time to bring products to market before we do. Any of these events could impair the commercial potential of our product candidates and could have a material adverse effect on our business.

## Table of Contents

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. Results of our trials could reveal a high and unacceptable severity and prevalence of side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial, or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

Additionally if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- regulatory authorities may require additional warnings on the label;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- 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 any product candidate, if approved, and could significantly harm our business, results of operations and prospects.

We are and continue to be subject to stringent government regulations concerning the clinical testing of our products.

We will also continue to be subject to government regulation of any product that receives regulatory approval.

Numerous statutes and regulations govern human testing and the manufacture and sale of human therapeutic products in the United States and other countries where we intend to market our products. Such legislation and regulation bears upon, among other things, the approval of protocols and human testing, the approval of manufacturing facilities, testing procedures and controlled research, the review and approval of manufacturing, preclinical and clinical data prior to marketing approval, including adherence to GMP during production and storage, and marketing activities including advertising and labeling.

Clinical trials may be delayed or suspended at any time by us or by the FDA or other similar regulatory authorities if it is determined at any time that patients may be or are being exposed to unacceptable health risks, including the risk of death, or if compounds are not manufactured under acceptable GMP conditions or with acceptable quality. Current regulations relating to regulatory approval may change or become more stringent. The agencies may also require additional trials be run in order to provide additional information regarding the safety, efficacy or equivalency of any compound for which we seek regulatory approval.

Moreover, any regulatory approval of a drug which is eventually obtained may entail limitations on the indicated uses for which that drug may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate. In addition, if the FDA or a comparable foreign regulatory authority approves any of our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with GMPs and GCPs for any clinical trials that we conduct post-approval. Furthermore, product approvals may be withdrawn or limited in some way if problems occur following initial marketing or if compliance with regulatory standards is not maintained. Similar restrictions are imposed in foreign markets. Regulatory agencies could become more risk adverse to any side effects or set higher standards of safety and efficacy prior to reviewing or approving a product. This could result in a product not being approved. If we, or any future marketing collaborators or contract manufacturers, fail to comply with applicable regulatory requirements, we may be subject to sanctions including fines, product recalls or seizures and related publicity

requirements, injunctions, total or partial suspension of production, civil penalties, suspension or withdrawals of previously granted regulatory approvals, warning or untitled letters, refusal to approve pending applications for marketing approval of new products or of supplements to approved applications, import or export bans or restrictions, and criminal prosecution and penalties. Any of these penalties could delay or prevent the promotion, marketing or sale of our products and product candidates.

## Table of Contents

The FDA's policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

We have no experience in commercial manufacturing and depend on others for the production of our product candidates at suitable levels of quality and quantity. Any problems or delays in the manufacture of our products would have a negative impact on our ability to successfully execute our development and commercialization strategies. We do not currently have nor do we plan to acquire the infrastructure or capability internally to manufacture our clinical drug supplies for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale. We rely on collaborators and/or third parties for development, scale-up, formulation, optimization, management of clinical trial and commercial scale manufacturing and commercialization. There are no assurances we can scale-up, formulate or manufacture any product candidate in sufficient quantities with acceptable specifications for the conduct of our clinical trials or for the regulatory agencies to grant approval of such product candidate. We have not yet commercialized any products and have no commercial manufacturing experience. To be successful, our products must be properly formulated, scalable, stable and safely manufactured in clinical trial and commercial quantities in compliance with GMP and other regulatory requirements and at acceptable costs. Should any of our suppliers or our collaborators be unable to supply or be delayed in supplying us with sufficient supplies, no assurance can be given that we will be able to find alternative means of supply in a short period of time. Should such parties' operations suffer a material adverse effect, the manufacturing of our products would also be adversely affected. Furthermore, key raw materials could become scarce or unavailable. There may be a limited number of third parties who can manufacture our products. We may not be able to meet specifications previously established for compounds during scale-up and manufacturing.

Our reliance on third parties to manufacture our product candidates will expose us and our partners to risks including the following, any of which could delay or prevent the commercialization of our products, result in higher costs, or deprive us of potential product revenue:

Contract manufacturers can encounter difficulties in achieving the scale-up, optimization, formulation, volume production of a compound as well as maintaining quality control with appropriate quality assurance. They may also experience shortages of qualified personnel. Contract manufacturers are required to undergo a satisfactory GMP inspection prior to regulatory approval and are obliged to operate in accordance with FDA, International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use, or ICH, European and other nationally mandated GMP regulations and/or guidelines governing manufacturing processes, stability testing, record keeping and quality standards. A failure of these contract manufacturers to follow GMP and to document their adherence to such practices or failure of an inspection by a regulatory agency may lead to significant delays in the availability of material for clinical study, leading to delays in our trials.

For each of our current product candidates we will initially rely on a limited number of contract manufacturers. Changing these or identifying future manufacturers may be difficult. Changing manufacturers requires re-validation of the manufacturing processes and procedures in accordance with FDA, ICH, European and other mandated GMP regulations and/or guidelines. Such re-validation may be costly and time-consuming. It may be difficult or impossible for us to quickly find replacement manufacturers on acceptable terms, if at all.

Our contract manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to produce, store and distribute our products successfully.

The successful commercialization of our product candidates, if approved, will depend on achieving market acceptance and we may not be able to gain sufficient acceptance to generate significant revenue.

Even if our product candidates are successfully developed and receive regulatory approval, they may not gain market acceptance among physicians, patients, healthcare payors such as private insurers or governments and other funding parties and the medical community. The degree of market acceptance for any of our products will depend on a number of factors, including:

• demonstration of the clinical efficacy and safety of our products;



the prevalence and severity of any adverse side effects;  
limitations or warnings contained in the product's approved labeling;  
cost-effectiveness and availability of acceptable pricing;

30

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Table of Contents

competitive product profile versus alternative treatment methods and the superiority of alternative treatment or therapeutics;  
the effectiveness of marketing and distribution methods and support for the products; and  
coverage and reimbursement policies of government and third-party payors to the extent that our products could receive regulatory approval but not be approved for coverage by or receive adequate reimbursement from government and quasi-government agencies or other third-party payors.

Disease indications may be small subsets of a disease that could be parsed into smaller and smaller indications as different subsets of diseases are defined. This increasingly fine characterization of diseases could have negative consequences; including creating an approved indication that is so small as not to have a viable market for us. If future technology allows characterization of a disease in a way that is different from the characterization used for large pivotal studies, it may make those studies invalid or reduce their usefulness, and may require repeating all or a portion of the studies. Future technology may supply better prognostic ability which could reduce the portion of patients projected to need a new therapy. Even after being cleared by regulatory authorities, a product may later be shown to be unsafe or not to have its purported effect, thereby preventing its widespread use or requiring withdrawal from the market.

\* If we fail to obtain coverage and adequate reimbursement for our products, our revenue-generating ability will be diminished and there is no assurance that the anticipated market for our products will be sustained.

We believe that there will be many different applications for products successfully derived from our technologies and that the anticipated market for products under development will continue to expand. However, due to competition from existing or new products and the yet-to-be established commercial viability of our products, no assurance can be given that these beliefs will prove to be correct. Physicians, patients, formularies, payors or the medical community in general may not accept or utilize any products that we or our collaborative partners may develop. Other drugs may be approved during our clinical testing which could change the accepted treatments for the disease targeted and make our product candidate obsolete.

Our and our collaborators' ability to commercialize our products successfully will depend, in part, on the extent to which coverage and adequate reimbursement for such products and related treatments will be available from governmental health payor programs at the federal and state levels, including Medicare and Medicaid, private health insurers, managed care plans and other organizations. No assurance can be given that third-party coverage and adequate reimbursement will be available that will allow us to maintain price levels sufficient for the realization of an appropriate return on our investment in product development.

Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance. Coverage decisions may depend upon clinical and economic standards that disfavor new drug products when more established or lower cost therapeutic alternatives are already available or subsequently become available. Even if we obtain coverage for our product candidates, the resulting reimbursement payment rates might not be adequate or may require co-payments that patients find unacceptably high. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates.

In the United States, in Canada and in many other countries, pricing and/or profitability of some or all prescription pharmaceuticals and biopharmaceuticals are subject to varying degrees of government control. Healthcare reform and controls on healthcare spending may limit the price we charge for any products and the amounts thereof that we can sell. In particular, in the United States, the federal government and private insurers have changed and have considered ways to change, the manner in which healthcare services are provided. In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, PPACA, became law in the United States. PPACA substantially changes the way healthcare is financed by both governmental and private insurers and significantly affects the healthcare industry. The provisions of PPACA of importance to our product candidates include the following:

an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;

an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program, to 23.1% and 13.0% of the average manufacturer price for most branded and generic drugs, respectively;

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

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

Table of Contents

- extension of a manufacturer's 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 a manufacturer's 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 and its implementing regulations (as described below);
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since PPACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect on April 1, 2013 and will stay in effect through 2024 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Moreover, the recently enacted Drug Supply Chain Security Act imposes new obligations on manufacturers of pharmaceutical products related to product and tracking and tracing. Among the requirements of this new legislation, manufacturers will be required to provide certain information regarding the drug products to individuals and entities to which product ownership is transferred, label drug product with a product identifier, and keep certain records regarding the drug product. The transfer of information to subsequent product owners by manufacturers will eventually be required to be done electronically. Manufacturers will also be required to verify that purchasers of the manufacturers' products are appropriately licensed. Further, under this new legislation, manufacturers will have drug product investigation, quarantine, disposition, and notification responsibilities related to counterfeit, diverted, stolen, and intentionally adulterated products, as well as products that are the subject of fraudulent transactions or which are otherwise unfit for distribution such that they would be reasonably likely to result in serious health consequences or death. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly, our financial operations.

We anticipate that PPACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and additional downward pressure on the reimbursement we may receive for any approved product. Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, the Middle Class Tax Relief and Job Creation Act of 2012 required the Centers for Medicare & Medicaid Services, or CMS, to reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which revised schedule served as a base for 2014 and will be the base for future years. Beginning January 1, 2016, there will be major changes to the payment formula under the Medicare Clinical Laboratory Fee Schedule, or CLFS. Under the Protecting Access to Medicare Act of 2014, or PAMA, which was signed to law in April 2014, clinical laboratories must report laboratory test payment data for each Medicare-covered clinical diagnostic lab test that it furnishes during a time period to be defined by future regulations. The reported data must include the payment rate (reflecting all discounts, rebates, coupons and other price concessions) and the volume of each test that was paid by each private payor (including health insurance issuers, group health plans, Medicare Advantage plans and Medicaid managed care organizations). Beginning in 2017, the Medicare payment rate for each clinical diagnostic lab test will be equal to the weighted median amount for the test from the most recent data collection period. The payment rate will apply to laboratory tests furnished by a hospital laboratory if the test is separately paid under the hospital outpatient prospective payment system. Levels of reimbursement may be impacted by current and future legislation, regulation or reimbursement policies of third-party payors in a manner that may harm the demand and reimbursement available

for our products, including our companion diagnostic, which in turn, could harm our future product pricing and sales. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products.

## Table of Contents

Competition in our targeted market area is intense and this field is characterized by rapid technological change. Therefore developments by competitors may substantially alter the predicted market or render our product candidates uncompetitive.

There are several hundred drugs in clinical development today in the area of oncology therapeutics. We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. In the oncology market, our major competitors include, but are not limited to: Amgen Inc.; ArQule Inc. and its partners Kyowa Hakko Kirin Pharma Inc. and Daiichi Sankyo Company Limited; Aveo Pharmaceuticals Inc.; Bristol-Myers Squibb Company; Exelixis Inc.; F. Hoffman-LaRoche Ltd.; GlaxoSmithKline PLC; Novartis AG; and Pfizer, among others.

Many companies have filed, and continue to file, patent applications in oncology which may or could affect our program. Some of these patent applications may have already been allowed or issued, and others may issue in the future. These companies include, but are not limited to: Bristol-Myers Squibb; Compugen Limited; Exelixis; GlaxoSmithKline; Novartis; and Pfizer. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed, and additional patents granted, in the future, as well as additional research and development programs expected in the future.

In addition to companies that have HDAC inhibitors or kinase inhibitors addressing oncology indications, our competition also includes hundreds of private and publicly traded companies that operate in the area of oncology but have therapeutics with different mechanisms of action. The oncology market in general is highly competitive with over 1,000 molecules currently in clinical development.

Developments by others may render our products or technologies non-competitive or obsolete or we may not be able to keep pace with technological developments. Our competitors may have developed or may be developing technologies which may be the basis for competitive products. Some of these products may prove to be more effective and less costly than the products developed or being developed by us. Our competitors may obtain regulatory approval for their products more rapidly than we do which may change the standard of care in the indications we are targeting, rendering our technology or products non-competitive or obsolete. Others may develop treatments or cures superior to any therapy we are developing or will develop. Moreover, alternate, less toxic forms of medical treatment may be developed which may be competitive with our products.

Many of the organizations which could be considered to be our competitors have substantially more financial and technical resources, more extensive discovery research, preclinical research and development capabilities and greater manufacturing, marketing, distribution, production and human resources than we do. Many of our current or potential competitors have more experience than us in research, preclinical testing and clinical trials, drug commercialization, manufacturing and marketing, and in obtaining domestic and foreign regulatory approvals. In addition, failure, unacceptable toxicity, lack of sales or disappointing sales or other issues regarding competitors' products or processes could have a material adverse effect on our product candidates, including our clinical candidates or our lead compounds. Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and brand recognition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, receiving FDA, EMA or other regulatory approval or discovering, developing and commercializing medicines before we do, which would have a material adverse impact on our business.

\* Even though we have obtained orphan drug designation for mocetinostat for MDS and Diffuse Large B-Cell Lymphoma, we may not be able to obtain or maintain the benefits associated with orphan drug status, including market exclusivity.

Regulatory authorities in some jurisdictions, including the United States and the European Union, 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 generally defined as a patient population of fewer than 200,000 individuals annually in the United States. In June 2014, the FDA granted orphan drug status to mocetinostat for the treatment of patients with MDS in the United States, and in August 2014 the FDA

granted orphan drug status to mocetinostat for the treatment of Diffuse Large B-Cell Lymphoma 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 may be entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same drug for that same indication for that time period. We can provide no assurance that another drug will not receive marketing approval prior to our product candidates. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union 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

Table of Contents

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, even after a drug is granted orphan exclusivity and approved, the FDA can subsequently approve another drug containing the same active ingredient for the same condition before the expiration of the seven year exclusivity period 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. In the European Union, the EMA may deny marketing approval for a product candidate if it determines such product candidate is structurally similar to an approved product for the same indication.

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 product candidates may obtain approval before us and receive orphan drug exclusivity, which could block us from entering the market.

We will not be able to successfully commercialize our product candidates without establishing sales and marketing capabilities internally or through collaborators.

We currently have no sales and marketing staff. We may not be able to find suitable sales and marketing staff and collaborators for all of our product candidates. We have no prior experience in the marketing, sale and distribution of pharmaceutical products and there are significant risks involved in building and managing a sales organization, including our ability to hire, retain and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any collaborators may not be adequate or successful or could terminate or materially reduce the effort they direct to our products. The development of a marketing and sales capability will require significant expenditures, management resources and time. The cost of establishing such a sales force may exceed any potential product revenue, or our marketing and sales efforts may be unsuccessful. If we are unable to develop an internal marketing and sales capability in a timely fashion, or at all, or if we are unable to enter into a marketing and sales arrangement with a third party on acceptable terms, we may be unable to successfully develop and seek regulatory approval for our product candidates and/or effectively market and sell approved products, if any.

We are subject to competition for our skilled personnel and may experience challenges in identifying and retaining key personnel that could impair our ability to conduct our operations effectively.

Our future success depends on our ability to retain our executive officers and to attract, retain and motivate qualified personnel. If we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy. Although we have not experienced problems attracting and retaining highly qualified personnel in the recent past, our industry has experienced a high rate of turnover of management personnel in recent years. Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, scientific and medical personnel, especially Charles M. Baum, M.D., Ph.D., our President and Chief Executive Officer, Mark J. Gergen, our Executive Vice President and Chief Operations Officer, Isan Chen, M.D., our Executive Vice President and Chief Medical and Development Officer, James Christensen, Ph.D. our Chief Scientific Officer, and Jamie A. Donadio, our Vice President of Finance, whose services are critical to the successful implementation of our product candidate acquisition, development and regulatory strategies, as well as the management of our financial operations. We are not aware of any present intention of any of these individuals to leave our Company. In order to induce valuable employees to continue their employment with us, we have provided stock options that vest over time. The value to employees of stock options that vest over time is significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies.

Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us at any time, with or without notice. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could harm our business, financial condition and prospects. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level and senior managers as well as junior, mid-level and senior scientific and medical



personnel.

We may also experience growth in the number of our employees and the scope of our operations, especially in clinical development. This growth will place a significant strain on our management, operations and financial resources and we may have difficulty managing this future potential growth. No assurance can be provided that we will be able to attract new employees to assist in our growth. Many of the other pharmaceutical companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. We also may employ consultants or part-time and contract employees. There can be no assurance that these individuals are retainable. While we have been able to attract and retain skilled and experienced personnel and consultants in the past, no assurance can be given that we will be able to do so in the future.

34

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Table of Contents

\* Our current and future relationships with customers and third-party payors in the United States and elsewhere may be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse, false claims, transparency, health information privacy and security and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.

As a pharmaceutical company, even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, certain federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. Our current and future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, which may constrain the business or financial arrangements and relationships through which we sell, market and distribute any drugs for which we obtain marketing approval. In addition, we may be subject to transparency laws and patient privacy regulation by U.S. federal and state governments and by governments in foreign jurisdictions in which we conduct our business. The laws that may affect our ability to operate include:

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

federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which impose 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, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

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

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose obligations on covered healthcare

- providers, health plans, and healthcare clearinghouses, as well as their business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

the federal Open Payments program, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to "payments or other transfers of value" made to physicians, which is defined to include doctors, dentists, optometrists, podiatrists and chiropractors, and teaching hospitals and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by the physicians and their immediate family members, and contains requirements for manufacturers to submit reports to CMS and on the 90th day of each calendar year, and disclosure of such information to be made by CMS on a publicly available website which began in September 2014; and

analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of available statutory and regulatory exceptions, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent healthcare reform legislation has strengthened these laws. For example, PPACA, among other things, amends the intent requirement of the federal Anti-Kickback Statute such that 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. Moreover, 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

Table of Contents

for purposes of the False Claims Act. To the extent that any of our product candidates is ultimately sold in countries other than the United States, we may be subject to similar laws and regulations in those countries. If we or our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, imprisonment, exclusion from participation in government healthcare programs, and the curtailment or restructuring of our operations, any of which could have a material adverse effect on our business. If any of the physicians or other healthcare providers or entities with whom we expect to do business, including any of our collaborators, is found not to be in compliance with applicable laws, it may be subject to criminal, civil or administrative sanctions, including exclusion from participation in government healthcare programs, which could also materially affect our business. We may become subject to the risk of product liability claims.

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk if we commercialize any products. Human therapeutic products involve the risk of product liability claims and associated adverse publicity. Currently, the principal risks we face relate to patients in our clinical trials, who may suffer unintended consequences. Claims might be made by patients, healthcare providers, pharmaceutical companies or others. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates, if approved. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our product candidates;
- injury to our reputation;
- withdrawal of clinical trial participants;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue from product sales; and
- the inability to commercialize any our product candidates, if approved.

We may not have or be able to obtain or maintain sufficient and affordable insurance coverage, and without sufficient coverage any claim brought against us could have a materially adverse effect on our business, financial condition or results of operations. We run clinical trials through investigators that could be negligent through no fault of our own and which could affect patients, cause potential liability claims against us and result in delayed or stopped clinical trials. We are required in many cases by contractual obligations to indemnify collaborators, partners, third-party contractors, clinical investigators and institutions. These indemnifications could result in a material impact due to product liability claims against us and/or these groups. We currently carry \$10 million in product liability insurance, which we believe is appropriate for our clinical trials. Although we maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

Our business involves the controlled use of hazardous materials and as such we are subject to environmental and occupational safety laws. Continued compliance with these laws may incur substantial costs and failure to maintain compliance could result in liability for damages that may exceed our resources.

Our preclinical research, manufacturing and development processes involve the controlled use of hazardous and radioactive materials. We are subject to federal, provincial and local laws and regulations governing the use, manufacture, storage, handling and disposal of such materials and certain waste products. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. The risk of

## Table of Contents

accidental contamination or injury from these materials cannot be completely eliminated. In the event of such an accident, we could be held liable for any damages that result, and any such liability could exceed our resources. We may not be adequately insured against this type of liability. We may be required to incur significant costs to comply with environmental laws and regulations in the future, and our operations, business or assets may be materially adversely affected by current or future environmental laws or regulations.

We may have to dedicate resources to the settlement of litigation.

Securities legislation in both the United States and Canada makes it relatively easy for stockholders to sue. This could lead to frivolous law suits which could take substantial time, money, resources and attention or force us to settle such claims rather than seek adequate judicial remedy or dismissal of such claims.

If we are required to defend patent infringement actions brought by third parties, or if we sue to protect our own patent rights or otherwise to protect our proprietary information and to prevent its disclosure, we may be required to pay substantial litigation costs and managerial attention may be diverted from business operations even if the outcome is in our favor. If we are required to defend our patents or trademarks against infringement by third parties, we may be required to pay substantial litigation costs and managerial attention and financial resources may be diverted from our research and development operations even if the outcome is in our favor.

We may be vulnerable to disruption, damage and financial obligation as a result of system failures.

Despite the implementation of security measures, any of the internal computer systems belonging to us, our collaborators or our third party service providers are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failure. Any system failure, accident or security breach that causes interruptions in our own, in collaborators' or in third party service vendors' operations could result in a material disruption of our drug discovery programs. To the extent that any disruption or security breach results in a loss or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we may incur liability, our drug discovery programs may be adversely affected and the further development of our product candidates may be delayed. Furthermore, we may incur additional costs to remedy the damages caused by these disruptions or security breaches.

### Risks Relating to Our Intellectual Property

We may not obtain adequate protection for our product candidates through patents and other intellectual property rights and as such our competitive advantage in the marketplace may be compromised.

Our success depends, in part, on our ability to secure and protect our patents, trade secrets, trademarks and other intellectual property rights and to operate without infringing on the proprietary rights of others or having third parties circumvent the rights that we own or license. We have filed and are actively pursuing patent applications in the United States, Canada, Japan, Europe and other major markets via the Patent Cooperation Treaty or directly in countries of interest. The patent positions of healthcare companies, universities and biopharmaceutical companies, including ours, are uncertain and involve complex questions of law and fact for which important legal issues may remain unresolved. Therefore, there is no assurance that our pending patent applications will result in the issuance of patents or that we will develop additional proprietary products which are patentable. Moreover, patents issued or to be issued to us may not provide us with any competitive advantage. Further, if the patent applications we hold or in-license with respect to our programs, product candidates and companion diagnostic fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize, future products. Our patents may be challenged by third parties in patent litigation. In addition, it is possible that third parties with products that are very similar to ours will circumvent our patents by means of alternate designs or processes or file applications or be granted patents that would block or hurt our efforts. There are no assurances that our patent counsel, lawyers or advisors have given us correct advice or counsel. Opinions from such patent counsel or lawyers may not be correct or based on incomplete facts. We cannot be certain that we are the first to invent or first to file for patent protection for the inventions covered by pending patent applications and, if we are not, we may be subject to priority disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or

enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that if challenged, our patents would be declared by a court to be valid or enforceable or that even if found valid and enforceable, a competitor's technology or product would be found by a court to infringe our patents. We may analyze patents or patent applications of our competitors that we believe are relevant to our activities, and consider that we are free to operate in relation to our product candidates, but our competitors may achieve issued claims, including in patents we consider to be unrelated, which block our efforts or may potentially result in our product candidates or our activities infringing such claims. The possibility exists that others will develop products which have the same effect as our products on an independent

Table of Contents

basis which do not infringe our patents or other intellectual property rights, or will design around the claims of patents that we have had issued that cover our products. The steps we have taken to protect our intellectual property may not prevent the misappropriation of our proprietary information and technologies, particularly in foreign countries where laws or law enforcement practices may not protect proprietary rights as fully as in Canada, the United States or Europe. Unauthorized disclosure of our proprietary information could also harm our competitive position. We could also inadvertently use our collaborators' data inappropriately which could lead to liability. We may file patent applications but have claims restricted or we may not be able to supply sufficient data to satisfy a patent office to support our claims and, as a result, may not obtain the original claims desired or we may receive restricted claims. Alternatively, it is possible that we may not receive any patent protection from an application.

Maintaining our patents and applications requires timely payment of fees and other associated costs in the countries of filing, and we could inadvertently abandon a patent or patent application (or trademark or trademark application) due to non-payment of fees, or as a result of a failure to comply with filing deadlines or other requirements of the prosecution process, resulting in the loss of protection of certain intellectual property rights in a certain country. Alternatively, we, our collaborators or our patent counsel may take action resulting in a patent or patent application becoming abandoned which may not be able to be reinstated, or if reinstated, may suffer patent term adjustments. Any of these outcomes could hurt our ability to gain full patent protection for our products. Registered trademarks in Canada, the United States and other countries that belong to us are subject to the same risks as described above for patents and patent applications.

Many of our collaboration agreements are complex and may call for licensing or cross-licensing of potentially blocking patents, know-how or intellectual property. Due to the potential overlap of data, know-how and intellectual property rights there can be no assurance that one of our collaborators will not dispute our right to send data or know-how or other intellectual property rights to third parties and this may potentially lead to liability or termination of a program. There are no assurances that the actions of our collaborators would not lead to disputes or cause us to default with other collaborators. We cannot be certain that a collaborator will not challenge the validity of licensed patents.

We cannot be certain that any country's patent and/or trademark office will not implement new rules which could affect how we draft, file, prosecute and/or maintain patents and patent applications. We cannot be certain that increasing costs for drafting, filing, prosecuting and maintaining patent applications and patents will not restrict our ability to file for patent protection, or to prosecute applications through to grant. We may be forced to abandon or return the rights to specific patents due to a lack of financial resources. There is no assurance that we could enter into licensing arrangements at a reasonable cost, or develop or obtain alternative technology in respect of patents issued to third parties that incidentally cover our products. Any inability to secure licenses or alternative technology could result in delays in the introduction of some of our products or even lead to prohibition of the development, manufacture or sale of certain products by us.

We have filed applications for trademark registrations in connection with our product candidates in various jurisdictions, including the United States. We intend to file further applications for other possible trademarks for our product candidates. No assurance can be given that any of our trademark applications will be registered in the United States or elsewhere, or that the use of any registered or unregistered trademarks will confer a competitive advantage in the marketplace. Furthermore, even if we are successful in our trademark registrations, the FDA and regulatory authorities in other countries have their own process for drug nomenclature and their own views concerning appropriate proprietary names. No assurance can be given that the FDA or any other regulatory authority will approve of any of our trademarks or will not request reconsideration of one of our trademarks at some time in the future. The loss, abandonment, or cancellation of any of our trademarks or trademark applications could negatively affect the success of the product candidates to which they relate.

Moreover, some of our know-how and technology which is not patented or not patentable may constitute trade secrets. Therefore, we require our consultants, advisors and collaborators to enter into confidentiality agreements and our employees to enter into invention, non-disclosure and non-compete agreements. However, no assurance can be given that such agreements will provide for a meaningful protection of our trade secrets, know-how or other proprietary information in the event of any unauthorized use or disclosure of information. Furthermore, we cannot provide



assurance that any of our employees, consultants, contract personnel or collaborators, either accidentally or through willful misconduct, will not cause serious impact to our programs and/or our strategy. All of our employees have signed confidentiality agreements, but there can be no assurance that they will not inadvertently or through their misconduct give trade secrets away.

Third-party intellectual property infringement claims may result in a reduction in the scope of our patent protection and competitive exclusivity with respect to our product candidates. Patent litigation, including defense against third-party intellectual property claims, may result in us incurring substantial costs.

Patent applications which may relate to or affect our business may have been filed by others. Such patent applications or patents resulting therefrom may conflict with our technologies, patents or patent applications and reducing the scope of our patent protection. Such events could cause us to stop or change the course of our research and development or modify our intellectual property strategies. We could also become involved in interference proceedings in connection with one or more of our patents or

Table of Contents

patent applications to determine priority of invention. There can be no guarantees that an interference proceeding would be successful or that such an outcome could be reversed on appeal. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of such interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees.

No assurance can be given that our patents, once issued, would be declared by a court to be valid or enforceable, or that we would not be found to infringe a competitor's patent.

Third parties may assert that we are using their proprietary information without authorization. Third parties may also have or obtain patents and may claim that technologies licensed to or used by us infringe their patents. Because patent applications can take many years to issue, third parties may have currently pending patent applications which may later result in issued patents that our product candidates or companion diagnostic may infringe, or which such third parties claim are infringed by the use of our technologies. If any third-party patents are held by a court of competent jurisdiction to cover any aspect of our product candidates, including the formulation or method of use of such product candidate, 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. In any such case, such a license may not be available on commercially reasonable terms or at all. In addition, any legal action that seeks damages or an injunction to stop us from carrying on our commercial activities relating to the affected technologies could subject us to monetary liability. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources.

Parties making claims against us for infringement of their intellectual property rights 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 claim of infringement against us, we could be required to redesign our infringing products or obtain a license from such third party to continue developing and commercializing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms, or at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. It may be impossible to redesign our products and technology, or it may require substantial time and expense, which could force us to cease commercialization of one or more of our product candidates, or some of our business operations, which could materially harm our business. In addition, in any such proceeding, we may be required to pay substantial damages, including treble damages and attorneys' fees in the event we are found liable for willful infringement.

Our intellectual property may be infringed upon by a third party.

Third parties may infringe one or more of our issued patents or trademarks. We cannot predict if, when or where a third party may infringe one or more of our issued patents or trademarks. We may attempt to invalidate a competitor's patent. There is no assurance such action will ultimately be successful and, even if initially successful, it could be overturned upon appeal. There is no assurance that we would be successful in a court of law to prove that a third party is infringing one or more of our issued patents. Even if we are successful in proving in a court of law that a third party is infringing one or more of our issued patents there can be no assurance that we would be successful in halting their infringing activities, for example, through a permanent injunction, or that we would be fully or even partially financially compensated for any harm to our business. We may be forced to enter into a license or other agreement with the infringing third party at terms less profitable or otherwise less commercially acceptable to us than if the license or agreement were negotiated under conditions between those of a willing licensee and a willing licensor. We may not become aware of a third party infringer within legal timeframes that would enable us to seek adequate compensation, or at all, thereby possibly losing the ability to be compensated for any harm to our business. Such a third-party may be operating in a foreign country where the infringer is difficult to locate, where we do not have issued patents and/or the patent laws may be more difficult to enforce. Some third-party infringers may be able to sustain the costs of complex patent infringement litigation more effectively than we can because they have substantially greater resources. Any inability to stop third-party infringement could result in loss in market share of

some of our products or even lead to a delay, reduction and/or inhibition of the development, manufacture or sale of certain products by us. There is no assurance that a product produced and sold by a third-party infringer would meet our or other regulatory standards or would be safe for use. Such third-party infringer products could irreparably harm the reputation of our products thereby resulting in substantial loss in market share and profits.

#### Risks Related to Our Shares of Common Stock

Our share price is volatile and may be influenced by numerous factors that are beyond our control.

A low share price and low market valuation may make it difficult to raise sufficient additional cash due to the significant dilution to current stockholders. Market prices for shares of biotechnology and biopharmaceutical companies such as ours are often volatile. Factors such as clinical and regulatory developments regarding our products or processes, developments regarding

Table of Contents

potential or future third-party collaborators, announcements of technological innovations, new commercial products, patents, the development of proprietary rights by us or by others or any litigation relating to these rights, regulatory actions, general conditions in the biotechnology and pharmaceutical industries, failure to meet analysts' expectations, publications, financial results or public concern over the safety of biopharmaceutical and biotechnological products, economic conditions in the United States, Canada or abroad, terrorism and other factors could have a significant effect on the share price for our shares of common stock. Any setback or delay in the clinical development of our programs could result in a significant decrease in our share price. In recent years the stock of other biotechnology and biopharmaceutical companies has experienced extreme price fluctuations that have been unrelated to the operating performance of the affected companies. There can be no assurance that the market price of our shares of common stock will not experience significant fluctuations in the future, including fluctuations that are unrelated to our performance. These fluctuations may result due to macroeconomic and world events, national or local events, general perception of the biotechnology industry or to a lack of liquidity. In addition other biotechnology companies or our competitors' programs could have positive or negative results that impact their stock prices and their results, or stock fluctuations could have a positive or negative impact on our stock price regardless whether such impact is direct or not.

Stockholders may not agree with our business, scientific, clinical and financial strategy, including additional dilutive financings, and may decide to sell their shares or vote against such proposals. Such actions could materially impact our stock price. In addition, portfolio managers of funds or large investors can change or change their view on us and decide to sell our shares. These actions could have a material impact on our stock price. In order to complete a financing, or for other business reasons, we may elect to consolidate our shares of common stock. Investors may not agree with these actions and may sell our shares. We may have little or no ability to impact or alter such decisions.

\* Our principal stockholders control the majority of our shares, and their actions may significantly influence matters submitted to our stockholders for approval and our share price.

Based on the information available to us, as of June 30, 2014, our stockholders and their affiliates who owned more than 5% of our outstanding common stock collectively owned approximately 60% of our outstanding common stock. Baker Bros. Advisors, L.L.C., or Baker Brothers, and Tavistock Life Sciences Co., or Tavistock, and their affiliates collectively own approximately 35% of our outstanding common stock. In addition, in conjunction with certain financing transactions, we granted to Baker Brothers and Tavistock each the right to nominate a member of our Board of Directors and the right to appoint an observer on our Board of Directors. Collectively Baker Brothers and Tavistock may have significant influence over matters submitted to our stockholders for approval, including the election and removal of directors and the approval of any merger, consolidation, or sale of all or substantially all of our assets. Furthermore, as a thinly traded stock, if Baker Brothers, Tavistock or any of other of our major stockholders determine to exit from the industry or from their holdings in us, for whatever reason, the impact on our share price could be detrimental over a prolonged period of time.

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 2013 Equity Incentive Plan, or the 2013 Plan, and our 2013 Employee Stock Purchase Plan, or the ESPP, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. Any increase in the number of shares outstanding as a result of the exercise of outstanding options, the vesting or settlement of outstanding stock awards, or the purchase of shares pursuant to the ESPP will cause our stockholders to experience additional dilution, which could cause our stock price to fall.

Our ability to use our U.S. net operating loss carryforwards and certain other tax attributes may be limited. Under Section 382 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three year period, the corporation's ability to use its pre-change U.S. net operating loss carryforwards, or NOLs, and other pre-change U.S. tax attributes (such as research tax credits) to offset its post-change income may be limited. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change U.S. net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which

Table of Contents

could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

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

We have never declared or paid any cash dividends on our common shares, and we currently expect that our earnings and cash flow will primarily be retained and used in our operations, including servicing any debt obligations we may have now or in the future. Accordingly, although we do not anticipate paying any dividends in the foreseeable future, we may not be able to generate sufficient cash flow in order to allow us to pay future dividends on, or make any distributions with respect to our common stock. As a result, capital appreciation, if any, of our common stock would be our stockholders' sole source of gain on their investment in our common stock for the foreseeable future.

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

Recent Sales of Unregistered Securities

During the nine months ended September 30, 2014, we issued and sold the following unregistered securities:

Warrant exercise

In 2011 and 2012, we issued common stock warrants in connection with the issuance of common stock through private placements. The warrant certificates provide that the warrant holder may elect to exercise their warrant and, in lieu of making the cash payment upon such exercise, receive upon such exercise a net number of common shares determined according to a formula prescribed in the warrant certificate. For the nine months ended September 30, 2014, four holders of warrants to purchase an aggregate of 73,964 shares of common stock elected to net exercise their warrants, resulting in the issuance of an aggregate of 43,526 shares of our common stock.

The issuances of the securities described above were deemed to be exempt from registration under the Securities Act of 1933, as amended, in reliance on Rule 506 of Regulation D in that each issuance of securities was to an accredited investor under Rule 501 of Regulation D and did not involve a public offering. The recipients of securities in each of these transactions acquired the securities for investment only and not with a view to or for sale in connection with any distribution thereof and appropriate legends were affixed to the securities issued in these transactions. There were no underwriters employed in connection with any of the transactions set forth above.

Use of Proceeds

We commenced our first public offering in the United States pursuant to a registration statement on Form S-1 (File No. 333-191544) that was declared effective by the SEC on October 23, 2013 and registered an aggregate of 3,250,000 shares of our common stock for sale to the public at price of \$17.50 per share for an aggregate offering price of approximately \$56.9 million. On October 29, 2013, we completed the offering. On November 27, 2013 the underwriters exercised their option to purchase an additional 87,500 shares of our common stock at a price of \$17.50 per share and an aggregate additional offering price of approximately \$1.5 million. Jefferies LLC and Leerink Swann LLC acted as joint book-running managers for the offering, and Piper Jaffray & Co. served as co-manager for the offering.

The underwriting discounts and commissions connected with the offering totaled approximately \$3.5 million. We incurred additional costs of approximately \$0.7 million in offering expenses, which when added to the underwriting discounts and commissions paid by us, amounts to total fees and costs of approximately \$4.2 million. Thus, net offering proceeds to us, after deducting underwriting discounts and commissions and offering costs, were \$54.2 million. No offering costs were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

As of September 30, 2014 we have used approximately \$17.5 million of these funds for preclinical and clinical development of our two lead kinase programs, MGCD265 and MGCD516, and our HDAC inhibitor, mocetinostat and related administrative support. We plan to use the remaining net proceeds from our public offering to fund our ongoing and planned clinical and dose confirmation trials for our lead product candidates and for research and development activities, working capital and other general corporate purposes. Our expected use of net proceeds from our public offering represents our current intentions based upon our present plans and business condition. We cannot predict with certainty all of the particular uses for our current funds, or the amounts that we will actually spend on the

uses set forth above. The amounts and timing of our actual use of these funds will vary depending on numerous factors, including our ability to obtain additional financing, the relative success and cost of our research, preclinical and clinical development programs, and the amount and timing of additional revenues. As a result, our management will have

Table of Contents

broad discretion in the application of these funds, and investors will be relying on our judgment regarding the application of the net proceeds of the offering.

ITEM 3. Defaults Upon Senior Securities

None.

ITEM 4. Mine Safety Disclosures

Not applicable.

ITEM 5. Other Information

JOBS Act

On April 5, 2012, the JOBS Act was enacted. Section 107 of the JOBS Act provides that an “emerging growth company” can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. In other words, an “emerging growth company” can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

We are relying on other exemptions and reduced reporting requirements provided by the JOBS Act. Subject to certain conditions set forth in the JOBS Act, as an “emerging growth company,” we rely on certain of these exemptions, including without limitation with respect to, (1) providing an auditor’s attestation report on our system of internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and (2) complying 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, known as the auditor discussion and analysis. We will remain an “emerging growth company” until the earliest of (a) the last day of the fiscal year in which we have total annual gross revenues of \$1 billion or more, (b) the last day of our fiscal year following the fifth anniversary of the date of the completion of our initial public offering, (c) the date on which we have issued more than \$1 billion in non-convertible debt during the previous three years or (d) the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

Internal Control Over Financial Reporting

Pursuant to Section 404(a) of the Sarbanes-Oxley Act, commencing the year following our first annual report required to be filed with the SEC, our management will be required to report on the effectiveness of our internal control over financial reporting. To comply with the requirements of being a reporting company under the Exchange Act, we may need to upgrade our systems, including information technology, implement additional financial and management controls, reporting systems and procedures and hire additional accounting and finance staff.



Table of Contents

ITEM 6. Exhibits  
EXHIBIT INDEX

Exhibit number	Description of document
2.1	Arrangement Agreement, dated May 8, 2013, by and between MethylGene Inc. and the Company. <sup>(2)</sup>
3.1	Amended and Restated Certificate of Incorporation. <sup>(1)</sup>
3.2	Bylaws. <sup>(1)</sup>
4.1	Form of Common Stock Certificate. <sup>(2)</sup>
31.1	Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.
31.2	Certification of the Principal Financial Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.
32.1	Certifications Pursuant to U.S.C. Section 1350, As Adopted Pursuant to Section 906 of the Public Company Accounting Reform and Investor Protection Act of 2002.
10.1	Lease Agreement, dated June 24, 2014, by and between the Company and ARE-SD Region No. 20, LLC. <sup>(3)</sup>
101.INS	XBRL Instance Document.
101.SCH	XBRL Taxonomy Extension Schema Document.
101.CAL	XBRL Taxonomy Extension Schema Document.
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.

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- (1) Incorporated by reference to Mirati Therapeutics, Inc.'s Registration Statement on Form 10-12B (No. 001-35921), filed with the Securities and Exchange Commission on May 10, 2013.
- (2) Incorporated by reference to Mirati Therapeutics, Inc.'s Amended Registration Statement on Form 10-12B/A (No. 001-35921), filed with the Securities and Exchange Commission on June 14, 2013.
- (3) Incorporated by reference to Exhibit 99.1 to Mirati Therapeutics, Inc.'s Current Report on Form 8-K, filed with the Securities and Exchange Commission on June 27, 2014.

SIGNATURES

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

MIRATI THERAPEUTICS, INC.

Date: November 10, 2014

by: /s/ Charles M. Baum, M.D., Ph.D.  
Chief Executive Officer

Date: November 10, 2014

by: /s/ Mark J. Gergen  
Executive Vice President and  
Chief Operations Officer