Epizyme, Inc.
Form 10-Q
August 06, 2015
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

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

For the quarterly period ended June 30, 2015

OR

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

For the transition period from _____ to _____

Commission File Number: 001-35945

EPIZYME, INC.

(Exact name of registrant as specified in its charter)

Delaware	**26-1349956**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
400 Technology Square, Cambridge, Massachusetts	**02139**
(Address of principal executive offices)	**(Zip code)**

617-229-5872

(Registrant s telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes x 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 x 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 definitions of accelerated filer, large accelerated filer and smaller reporting company in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer ¨	Accelerated filer x
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 x

The number of shares outstanding of the registrant s common stock as of July 31, 2015: 41,329,700 shares.

Table of Contents

PART I - FINANCIAL INFORMATION

Item 1. Financial Statements.

PART II - OTHER INFORMATION

Table of Contents

PART I FINANCIAL INFORMATION

Item 1. Financial Statements

EPIZYME, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS (UNAUDITED)

(Amounts in thousands except share and per share data)

	June 30, 2015	December 31, 2014
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 236,695	$ 190,095
Accounts receivable	723	2,075
Prepaid expenses and other current assets	2,011	2,840
Total current assets	239,429	195,010
Property and equipment, net	4,856	3,620
Restricted cash and other assets	709	573
Total Assets	$ 244,994	$ 199,203
LIABILITIES AND STOCKHOLDERS EQUITY		
Current Liabilities:		
Accounts payable	$ 3,022	$ 8,300
Accrued expenses	9,476	7,043
Current portion of capital lease obligation	534	
Current portion of deferred revenue	174	1,702
Total current liabilities	13,206	17,045
Capital lease obligation, net of current portion	1,017	
Deferred revenue, net of current portion	21,449	21,449
Other long-term liabilities	416	427
Commitments and contingencies		
Stockholders Equity:		
Preferred stock, $0.0001 par value; 5,000,000 shares authorized; 0 shares issued and outstanding		
Common stock, $0.0001 par value; 125,000,000 shares authorized; 41,240,338 shares and 34,426,012 shares issued and outstanding, respectively	4	3
Additional paid-in capital	407,072	271,364
Accumulated deficit	(198,170)	(111,085)
Total stockholders equity	208,906	160,282

Total Liabilities and Stockholders Equity	$ 244,994	$ 199,203

See notes to condensed consolidated financial statements.

Table of Contents

EPIZYME, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS (UNAUDITED)

(Amounts in thousands except per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
Collaboration revenue	$ 736	$ 9,494	$ 1,647	$ 22,885
Operating expenses:				
Research and development	20,551	17,499	77,602	32,846
General and administrative	5,970	5,306	11,207	10,262
Total operating expenses	26,521	22,805	88,809	43,108
Loss from operations	(25,785)	(13,311)	(87,162)	(20,223)
Other income, net:				
Interest income, net	6	26	37	42
Other income	20	12	40	24
Other income, net	26	38	77	66
Loss before income taxes	(25,759)	(13,273)	(87,085)	(20,157)
Income tax expense		113		113
Net loss	$ (25,759)	$ (13,386)	$ (87,085)	$ (20,270)
Loss per share allocable to common stockholders:				
Basic and Diluted	$ (0.63)	$ (0.40)	$ (2.29)	$ (0.63)
Weighted average shares outstanding:				
Basic and Diluted	41,087	33,156	38,056	32,064
Comprehensive loss	$ (25,759)	$ (13,386)	$ (87,085)	$ (20,270)

See notes to condensed consolidated financial statements.

Table of Contents

EPIZYME, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS (UNAUDITED)

(Amounts in thousands)

	Six Months Ended June 30,	
	2015	**2014**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (87,085)	$ (20,270)
Adjustments to reconcile net loss to net cash (used in) provided by operating activities:		
Acquired in-process research and development	40,000	
Depreciation and amortization	653	362
Stock-based compensation	4,910	3,095
Loss on disposal of property and equipment	6	
Changes in operating assets and liabilities:		
Accounts receivable	1,352	31,476
Prepaid expenses and other current assets	829	(235)
Accounts payable	(5,212)	(142)
Accrued expenses	2,433	(410)
Deferred revenue	(1,528)	(9,387)
Restricted cash and other assets	(136)	169
Other long-term liabilities	(11)	8
Net cash (used in) provided by operating activities	(43,789)	4,666
CASH FLOWS FROM INVESTING ACTIVITIES:		
Acquisition of in-process research and development	(40,000)	
Purchases of property and equipment	(229)	(824)
Net cash used in investing activities	(40,229)	(824)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Payment under capital lease obligation	(181)	
Proceeds from public offering, net of commissions	130,712	101,283
Proceeds from stock options exercised	215	1,334
Excess tax benefit from stock option plan		28
Issuance of shares under employee stock purchase plan	239	201
Payment of public offering costs	(367)	(649)
Proceeds from reimbursement of public offering costs		269
Net cash provided by financing activities	130,618	102,466

Net increase in cash and cash equivalents	46,600	106,308
Cash and cash equivalents, beginning of period	190,095	123,564
Cash and cash equivalents, end of period	$ 236,695	$ 229,872
SUPPLEMENTAL DISCLOSURE OF CASH FLOW INFORMATION:		
Purchases of property and equipment unpaid at period end	15	113
Equipment acquired under capital lease	1,732	
Income taxes paid	2	241

See notes to condensed consolidated financial statements.

Table of Contents

EPIZYME, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (UNAUDITED)

1. Overview and Basis of Presentation

Epizyme, Inc. (collectively referred to with its wholly owned, controlled subsidiary, Epizyme Securities Corporation, as Epizyme or the Company) is a clinical stage biopharmaceutical company that discovers, develops and plans to commercialize novel epigenetic therapies for cancer patients. The Company has built a proprietary product platform that it uses to create small molecule inhibitors of a 96-member class of enzymes known as histone methyltransferases (HMTs). Genetic alterations can result in changes to the activity of HMTs, making them oncogenic. The Company s therapeutic strategy is to inhibit oncogenic HMTs to treat the underlying causes of the associated cancers.

The condensed consolidated financial statements of the Company included herein have been prepared, without audit, pursuant to the rules and regulations of the Securities and Exchange Commission (SEC). Certain information and footnote disclosures normally included in financial statements prepared in accordance with accounting principles generally accepted in the United States of America have been condensed or omitted from this report, as is permitted by such rules and regulations. Accordingly, these condensed consolidated financial statements should be read in conjunction with the financial statements and notes thereto included in the Company s Annual Report on Form 10-K for the fiscal year ended December 31, 2014 (the Annual Report).

The unaudited condensed consolidated financial statements include the accounts of Epizyme and its subsidiary. All intercompany transactions and balances of subsidiaries have been eliminated in consolidation. In the opinion of management, the information furnished reflects all adjustments, all of which are of a normal and recurring nature, necessary for a fair presentation of the results for the reported interim periods. The Company considers events or transactions that occur after the balance sheet date but before the financial statements are issued to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. The three months ended June 30, 2015 and 2014 are referred to as the second quarter of 2015 and 2014, respectively. The results of operations for interim periods are not necessarily indicative of results to be expected for the full year or any other interim period.

In March 2015, the Company conducted a public offering of its common stock, selling 6,000,000 shares at a price of $20.75 per share. The Company received net proceeds before expenses from the sale of these 6,000,000 shares of $117.0 million after deducting underwriting discounts and commissions paid by the Company. In April 2015, the Company issued and sold an additional 701,448 shares in connection with the March 2015 public offering at a price of $20.75 per share pursuant to the underwriters option to purchase additional shares that the Company granted in connection with such public offering. The Company received net proceeds before expenses from the sale of these 701,448 shares of $13.7 million after deducting underwriting discounts and commissions paid by the Company.

2. Summary of Significant Accounting Policies

In the six months ended June 30, 2015, the Company updated its accounting policy regarding property and equipment as a result of property and equipment acquired pursuant to a capital lease.

Property and Equipment

The Company records property and equipment at cost. Property and equipment acquired under a capital lease is recorded at the lesser of the present value of the minimum lease payments under the capital lease or the fair value of the leased property at lease inception.

Table of Contents

The Company calculates depreciation and amortization using the straight-line method over the following estimated useful lives:

Asset Category	Useful Lives
Laboratory equipment	5 - 20 years
Office furniture and equipment	3 - 10 years or term of respective lease, if shorter
Leasehold improvements	3 - 10 years or term of respective lease, if shorter

Amortization of capital lease assets is included in depreciation expense. The Company capitalizes expenditures for new property and equipment and improvements to existing facilities and charges the cost of maintenance to expense. The Company eliminates the cost of property retired or otherwise disposed of, along with the corresponding accumulated depreciation, from the related accounts, and the resulting gain or loss is reflected in the results of operations.

Additionally, the Company updated its accounting policies as a result of the amended and restated collaboration and license agreement the Company executed with Eisai Co., Ltd. (Eisai), pursuant to which the Company recorded the reacquisition of worldwide rights, excluding Japan, to its EZH2 program, including tazemetostat (also known as EPZ-6438), as an acquisition of in-process research and development.

Acquired In-Process Research and Development

The Company records upfront payments that relate to the acquisition of a development-stage product candidate as research and development expense in the period in which they are incurred, provided that the acquired development-stage product candidate did not also include processes or activities that would constitute a business, the product candidate has not achieved regulatory approval for marketing and, absent obtaining such approval, has no alternative future use.

There have been no other material changes to the significant accounting policies previously disclosed in the Company s Annual Report.

Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2014-09, *Revenue from Contracts with Customers*. ASU 2014-09 amends Accounting Standards Codification (ASC) 605, *Revenue Recognition*, by outlining a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers. ASU 2014-09 was originally pronounced to become effective for the Company for interim and annual periods beginning after December 15, 2016. In July 2015, the FASB approved a one-year deferral of the effective date of ASU 2014-09. This ASU will be effective for annual and interim periods beginning on or after December 15, 2017. Early adoption is permitted, however not before the original effective date of annual periods beginning after December 15, 2016. The Company is evaluating the impact that this ASU may have on its consolidated financial statements, if any.

In August 2014, the FASB issued ASU No. 2014-15, *Disclosure of Uncertainties about an Entity s Ability to Continue as a Going Concern*. ASU 2014-15 amends ASC 205-40, *Presentation of Financial Statements Going Concern*, by providing guidance on determining when and how reporting entities must disclose going-concern

uncertainties in their financial statements, including requiring management to perform interim and annual assessments of an entity s ability to continue as a going concern within one year of the date of issuance of the entity s financial statements and providing certain disclosures if there is substantial doubt about the entity s ability to continue as a going concern. ASU 2014-15 will be effective for the Company for annual periods ending after December 15, 2016 and interim periods within annual periods beginning after December 15, 2016. The Company is still evaluating the impact of this ASU on its consolidated financial statements; however, it is disclosure-only in nature.

In April 2015, the FASB issued ASU No. 2015-05, *Customer s Accounting for Fees Paid in a Cloud Computing Arrangement*. ASU 2015-05 amends ASC 350-40, *Internal-Use Software*, by providing customers with guidance on determining whether a cloud computing arrangement contains a software license that should be accounted for as internal-use software. ASU 2015-05 will be effective for the Company for annual periods beginning after December 15, 2015 and interim periods within annual periods beginning after December 15, 2015. The Company is evaluating the impact that this ASU may have on its consolidated financial statements, if any.

Table of Contents

In June 2015, the FASB issued ASU No. 2015-10, *Technical Corrections and Improvements*. ASU 2015-10 covers a wide range of Topics in the ASC. The amendments in this ASU represent changes to clarify the ASC, correct unintended application of guidance, or make minor improvements to the ASC that are not expected to have a significant effect on current accounting practice or create a significant administrative cost to most entities. Additionally, some of the amendments will make the ASC easier to understand and easier to apply by eliminating inconsistencies, providing needed clarifications, and improving the presentation of guidance in the ASC. The amendments in ASU 2015-10 that require transition guidance are effective for all entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2015. All other amendments will be effective upon the issuance of this ASU. The Company does not anticipate that the adoption of this standard will have a material impact on its consolidated financial statements and footnote disclosures.

3. Fair Value Measurements

The Company classifies fair value based measurements using a three-level hierarchy that prioritizes the inputs used to measure fair value. This hierarchy requires entities to maximize the use of observable inputs and minimize the use of unobservable inputs. The three levels of inputs used to measure fair value are as follows: Level 1, quoted market prices in active markets for identical assets or liabilities; Level 2, observable inputs other than quoted market prices included in Level 1 such as quoted market prices for markets that are not active or other inputs that are observable or can be corroborated by observable market data; and Level 3, unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities, including estimates and assumptions developed by the Company, reflective of those that a market participant would use, as inputs to certain pricing models, discounted cash flow methodologies and similar techniques that use significant unobservable inputs.

The Company s financial instruments as of June 30, 2015 and December 31, 2014 consisted primarily of cash and cash equivalents, accounts receivable and accounts payable. As of June 30, 2015 and December 31, 2014, the Company s financial assets recognized at fair value consisted of the following:

	Fair Value as of June 30, 2015			
	Total	Level 1	Level 2	Level 3
		(In thousands)		
Cash equivalents	$ 196,924	$ 196,924	$	$
Total	$ 196,924	$ 196,924	$	$

	Fair Value as of December 31, 2014			
	Total	Level 1	Level 2	Level 3
		(In thousands)		
Cash equivalents	$ 184,257	$ 184,257	$	$
Total	$ 184,257	$ 184,257	$	$

7

Table of Contents

4. Accrued Expenses

Accrued expenses consisted of the following:

	June 30, 2015	December 31, 2014
	(In thousands)	
Employee compensation and benefits	$ 2,068	$ 2,623
Research and development and professional expenses	7,408	4,420
Accrued expenses	$ 9,476	$ 7,043

5. Income Taxes

The Company did not record a federal or state income tax provision or benefit for the three and six months ended June 30, 2015 due to the expected loss before income taxes to be incurred for the year ending December 31, 2015, as well as the Company s continued maintenance of a full valuation allowance against its net deferred tax assets.

The Company recorded $0.1 million of income tax expense in the three and six months ended June 30, 2014 due to provision-to-return adjustments identified related to the year ended December 31, 2013. The Company did not record a federal or state income tax provision or benefit for the three or six months ended June 30, 2014 related to the year ending December 31, 2014, due to the then expected loss before income taxes to be incurred for the year ending December 31, 2014, as well as the Company s continued maintenance of a full valuation allowance against its net deferred tax assets.

6. Commitments and Contingencies

Commitments

In the first quarter of 2015, the Company acquired computer equipment pursuant to a capital lease. Future minimum equipment lease payments under this capital lease, net of imputed interest, as of June 30, 2015 are as follows:

Future minimum lease payments in year ending December 31:	(In thousands)
2015	$ 332
2016	665
2017	665
2018	111
Total future minimum lease payments	1,773
Less: Amount representing imputed interest on equipment lease	(222)

Capital lease obligation	$	1,551

The Company also entered into an agreement in June 2015 to lease approximately 4,000 square feet of office space in Durham, North Carolina through July 2017. Total future minimum lease payments under this office lease agreement are approximately $0.2 million.

In connection with the amended and restated collaboration and license agreement that the Company executed with Eisai in March 2015, the Company and Eisai entered into an amended and restated letter agreement related to their December 2012 companion diagnostic agreement with Roche Molecular Systems (Roche). Upon the execution of the amended and restated letter agreement with Eisai, the Company assumed responsibility for up to $15.5 million of the remaining development costs under the agreement with Roche. Eisai continues to be responsible for up to $1.0 million of the remaining Japan-specific development costs under the agreement with Roche.

Table of Contents

Contingencies

In connection with the execution of the amended and restated collaboration and license agreement with Eisai, the Company agreed to pay Eisai up to a total of $20.0 million upon the achievement of specified clinical development milestones and up to a total of $50.0 million upon the achievement of specified regulatory milestones. In addition, the Company may be required to pay Eisai royalties at a percentage in the mid-teens on worldwide net sales of any EZH2 product, excluding net sales in Japan.

7. Collaborations

Eisai

In April 2011, the Company entered into a collaboration and license agreement with Eisai under which the Company granted Eisai an exclusive worldwide license to its small molecule HMT inhibitors directed to the EZH2 HMT, including the Company s product candidate tazemetostat, while retaining an opt-in right to co-develop, co-commercialize and share profits with Eisai as to licensed products in the United States (the original agreement). Additionally, as part of the research collaboration, the Company provided research and development services related to the licensed compounds through December 31, 2014.

The Company recognized $2.0 million and $3.6 million of collaboration revenue in the three and six months ended June 30, 2014, respectively, under the original agreement. As of December 31, 2014, the Company had completed its performance obligations under the original agreement. Accordingly, the Company had no remaining deferred revenue as of December 31, 2014 related to the original agreement.

In March 2015, the Company entered into an amended and restated collaboration and license agreement with Eisai, under which the Company reacquired worldwide rights, excluding Japan, to its EZH2 program, including tazemetostat. Under the amended and restated collaboration and license agreement, the Company is responsible for global development, manufacturing and commercialization outside of Japan of tazemetostat and any other EZH2 product candidates, with Eisai retaining development and commercialization rights in Japan, as well as a right to elect to manufacture tazemetostat and any other EZH2 product candidates in Japan.

Under the original agreement, Eisai was solely responsible for funding all research, development and commercialization costs for EZH2 compounds. Under the amended and restated collaboration and license agreement, the Company is solely responsible for funding global development, manufacturing and commercialization costs for EZH2 compounds outside of Japan, including up to $15.5 million of the remaining development costs due under a Roche companion diagnostic agreement, and Eisai is solely responsible for funding Japan-specific development and commercialization costs for EZH2 compounds.

The Company recorded the reacquisition of worldwide rights, excluding Japan, to the EZH2 program, including tazemetostat, under the amended and restated collaboration and license agreement with Eisai as an acquisition of an in-process research and development asset. As this asset was acquired without corresponding processes or activities that would constitute a business, has not achieved regulatory approval for marketing and, absent obtaining such approval, has no alternative future use, the Company recorded the $40.0 million upfront payment made to Eisai in March 2015 as research and development expense in the condensed consolidated statements of operations and comprehensive loss for the three months ended March 31, 2015. The Company has also agreed to pay Eisai up to $20.0 million in clinical development milestone payments, up to $50.0 million in regulatory milestone payments and royalties at a percentage in the mid-teens on worldwide net sales of any EZH2 product, excluding net sales in Japan.

The Company is eligible to receive from Eisai royalties at a percentage in the mid-teens on net sales of any EZH2 product in Japan.

Table of Contents

Celgene

In April 2012, the Company entered into a collaboration and license agreement with Celgene Corporation and Celgene International Sàrl, an affiliate of Celgene Corporation (Celgene Corporation and its affiliated entities are collectively referred to as Celgene), to discover, develop and commercialize, in all countries other than the United States, small molecule HMT inhibitors targeting the DOT1L HMT, including pinometostat (also known as EPZ-5676), and any HMT targets from its product platform, other than the EZH2 HMT including tazemetostat and targets covered by its collaboration with Glaxo Group Limited, an affiliate of GlaxoSmithKline (GSK), which the Company refers to as the available targets. On July 8, 2015, the Company entered into an amendment and restatement of its collaboration and license agreement with Celgene Corporation and Celgene RIVOT Ltd., an affiliate of Celgene Corporation. Refer to Note 11, *Subsequent Events*, for additional information.

Agreement Structure

Under the original agreement, the Company granted Celgene an exclusive license, for all countries other than the United States, to small molecule HMT inhibitors targeting the DOT1L HMT, including pinometostat, and an option, on a target-by-target basis, to exclusively license, for all countries other than the United States, rights to small molecule HMT inhibitors targeting any HMT targets, other than the EZH2 HMT including tazemetostat and targets covered by the Company s collaboration and license agreement dated January 8, 2011 with GSK. Under the original agreement, Celgene s option was exercisable during an option period that would have expired on July 9, 2015.

Under the original agreement, the Company received a $65.0 million upfront payment and $25.0 million from the sale of its series C redeemable convertible preferred stock to an affiliate of Celgene, of which $3.0 million was considered a premium and included as collaboration arrangement consideration for a total upfront payment of $68.0 million. In addition, the Company has received a $25.0 million clinical development milestone payment and $6.7 million of global development co-funding through June 30, 2015. The Company was also eligible to receive $35.0 million in an additional clinical development milestone payment and up to $100.0 million in regulatory milestone payments related to DOT1L as well as up to $65.0 million in payments, including a combination of clinical development milestone payments and an option exercise fee for each available target as to which Celgene had the right to exercise its option during an initial option period that ended in July 2015 (each a selected target), and up to $100.0 million in regulatory milestone payments for each selected target. As to DOT1L and each selected target, the Company retained all product rights in the United States and was eligible to receive royalties for each target at defined percentages ranging from the mid-single digits to the mid-teens on net product sales outside of the United States subject to reduction in specified circumstances

The Company is obligated to conduct and solely fund research and development costs of the Phase 1 clinical trials for pinometostat. For all remaining DOT1L program development costs, Celgene and the Company will equally co-fund global development and each party will solely fund territory-specific development costs for its territory.

Collaboration Revenue

Through June 30, 2015, in addition to amounts allocated to Celgene s purchase of shares of the Company s series C redeemable convertible preferred stock, the Company had recorded a total of $99.8 million in cash and accounts receivable under the Celgene agreement, including the $3.0 million implied premium on Celgene s purchase of shares of the Company s series C redeemable convertible preferred stock. Through June 30, 2015, the Company has recognized $71.5 million of collaboration revenue related to this agreement, including $0.1 million and $0.2 million in the three and six months ended June 30, 2015, respectively, and $1.2 million and $2.9 million in the three and six months ended June 30, 2014, respectively, and $6.7 million of global development co-funding as a reduction to

research and development expense, including $0.4 million and $0.9 million in the three and six months ended June 30, 2015, respectively, and $0.9 million and $1.3 million in the three and six months ended June 30, 2014, respectively, in the condensed consolidated statements of operations and comprehensive loss. As of June 30, 2015 and December 31, 2014, the Company had deferred revenue of $21.6 million and $21.7 million, respectively, related to this agreement.

GSK

In January 2011, the Company entered into a collaboration and license agreement with GSK, to discover, develop and commercialize novel small molecule HMT inhibitors directed to available targets from the Company s platform. Under the terms of the agreement, the Company granted GSK exclusive worldwide license rights to HMT inhibitors directed to three

Table of Contents

targets. Additionally, as part of the research collaboration, the Company agreed to provide research and development services related to the licensed targets pursuant to agreed upon research plans during a research term that ended January 8, 2015. In March 2014, the Company and GSK amended certain terms of this agreement for the third licensed target, revising the license terms with respect to candidate compounds and amending the corresponding financial terms, including reallocating milestone payments and increasing royalty rates as to the third target. The Company substantially completed all research obligations under this agreement by the end of the first quarter of 2015 and completed the transfer of the remaining data and materials for these programs to GSK in the second quarter of 2015.

Agreement Structure

Under the agreement, the Company recorded a $20.0 million upfront payment, a $3.0 million payment upon the execution of the March 2014 agreement amendment, $6.0 million of fixed research funding, $15.0 million of preclinical research and development milestone payments and $9.0 million for research and development services. The Company is eligible to receive up to $18.0 million in additional preclinical research and development milestone payments, up to $109.0 million in clinical development milestone payments, up to $275.0 million in regulatory milestone payments and up to $218.0 million in sales-based milestone payments. In addition, GSK is required to pay the Company royalties, at percentages from the mid-single digits to the low double-digits, on a licensed product-by-licensed product basis, on worldwide net product sales, subject to reduction in specified circumstances. Due to the uncertainty of pharmaceutical development and the high historical failure rates generally associated with drug development, the Company may not receive any additional milestone payments or royalty payments from GSK. Due to the varying stages of development of each licensed target, the Company is not able to determine the next milestone that might be achieved under this agreement, if any. GSK became solely responsible for development and commercialization for each licensed target in the collaboration when the research term ended on January 8, 2015.

Collaboration Revenue

Through June 30, 2015, the Company received a total of $53.0 million in cash under the GSK agreement, which the Company recognized as collaboration revenue in the condensed consolidated statements of operations and comprehensive loss, including $0.6 million and $1.4 million in the three and six months ended June 30, 2015, respectively, and $6.3 million and $16.4 million in the three and six months ended June 30, 2014, respectively, including a $1.0 million preclinical research and development milestone achieved and recognized as collaboration revenue in the three months ended June 30, 2014 and an additional $2.0 million preclinical research and development milestone achieved and recognized as collaboration revenue in the six months ended June 30, 2014. As of December 31, 2014, the Company had deferred revenue of $1.4 million related to this agreement, which was fully recognized as collaboration revenue by June 30, 2015.

Companion Diagnostics

Roche

In December 2012, Eisai and the Company entered into an agreement with Roche under which Eisai and the Company agreed to fund Roche s development of a companion diagnostic to identify patients who possess certain point mutations in EZH2. At the same time, Eisai and the Company entered into a letter agreement pursuant to which Eisai agreed to be responsible for the development costs under the Roche agreement. In October 2013, this agreement was amended to include additional point mutations in EZH2. Under the terms of the amended agreement, Roche is to be paid up to a total of $21.5 million to develop and to make commercially available the companion diagnostic.

In connection with the March 2015 execution of the amended and restated collaboration and license agreement with Eisai, the Company and Eisai entered into an amended and restated letter agreement, pursuant to which the Company agreed to be responsible for up to $15.5 million of the remaining development costs under the agreement with Roche. Eisai continues to be responsible for up to $1.0 million of the remaining Japan-specific development costs under the agreement with Roche.

8. Stock-Based Compensation

Total stock-based compensation expense related to stock options, restricted stock units and the employee stock purchase plan was $2.5 million and $1.6 million for the three months ended June 30, 2015 and 2014, respectively, and $4.9 million and $3.1 million for the six months ended June 30, 2015 and 2014, respectively.

Table of Contents

Stock-based compensation expense is classified in the condensed consolidated statements of operations and comprehensive loss as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
	(In thousands)			
Research and development	$ 1,437	$ 815	$ 2,795	$ 1,496
General and administrative	1,057	817	2,115	1,599
Total	$ 2,494	$ 1,632	$ 4,910	$ 3,095

Stock Options

The weighted-average fair value of options, estimated as of the grant date using the Black-Scholes option pricing model, was $12.46 and $17.15 per option for those options granted during the three months ended June 30, 2015 and 2014, respectively, and $14.68 and $22.53 per option for those options granted during the six months ended June 30, 2015 and 2014, respectively. Key assumptions used to apply this pricing model were as follows:

	Six Months Ended June 30,	
	2015	2014
Risk-free interest rate	1.5%	1.6%
Expected life of options	6.0 years	6.0 years
Expected volatility of underlying stock	84.2%	93.4%
Expected dividend yield	0.0%	0.0%

The following is a summary of stock option activity for the six months ended June 30, 2015:

	Number of Options	Weighted Average Exercise Price per Share	Weighted Average Remaining Contractual Term (In years)	Aggregate Intrinsic Value (In thousands)
Outstanding at December 31, 2014	2,959,506	$ 10.66		
Granted	763,557	20.65		
Exercised	(100,931)	2.13		
Forfeited or expired	(273,385)	16.78		
Outstanding at June 30, 2015	3,348,747	$ 12.70	7.1	$ 41,909
Exercisable at June 30, 2015	1,649,769	$ 5.47	5.4	$ 31,791

As of June 30, 2015, there was $20.6 million of unrecognized compensation cost related to stock options that are expected to vest. These costs are expected to be recognized over a weighted average remaining vesting period of 2.7 years.

Table of Contents

Restricted Stock Units

The following is a summary of restricted stock unit activity for the six months ended June 30, 2015:

	Number of Units	Weighted Average Grant Date Fair Value per Unit
Outstanding at December 31, 2014		$
Granted	37,313	18.49
Outstanding at June 30, 2015	37,313	$ 18.49

As of June 30, 2015, there was $1.1 million of unrecognized compensation cost related to restricted stock units that are expected to vest, including $0.6 million of unrecognized compensation cost related to restricted stock units to be issued in the first quarter of 2016, pursuant to a February 2015 employment agreement with the Company s chief financial officer, for which service is currently being provided. These costs are expected to be recognized over a weighted average remaining vesting period of 3.6 years.

9. Loss Per Share

Basic and diluted loss per share allocable to common stockholders are computed as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
	(In thousands except per share data)			
Net loss	$ (25,759)	$ (13,386)	$ (87,085)	$ (20,270)
Weighted average shares outstanding	41,087	33,156	38,056	32,064
Basic and diluted loss per share allocable to common stockholders	$ (0.63)	$ (0.40)	$ (2.29)	$ (0.63)

In February 2014, the Company issued 3,673,901 shares of common stock in connection with a public offering. In March 2015, the Company issued 6,000,000 shares of common stock in connection with a public offering. In April 2015, the Company issued and sold an additional 701,448 shares of common stock in connection with the March 2015 public offering at a price of $20.75 per share pursuant to the underwriters option to purchase additional shares that the Company granted in connection with such public offering. The issuance of these shares contributed to a significant increase in the Company s shares outstanding, to 41,240,338 shares as of June 30, 2015, and in the weighted average shares outstanding for the three and six months ended June 30, 2015 when compared to the comparable prior year periods and is expected to continue to impact the year-over-year comparability of the Company s (loss) earnings per share calculations through 2015.

The following common stock equivalents were excluded from the calculation of diluted loss per share allocable to common stockholders because their inclusion would have been anti-dilutive:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
	(In thousands)			
Stock options	3,349	4,074	3,349	4,074
Unvested restricted stock units	37		37	
Shares issuable under employee stock purchase plan	7	7	7	7
	3,393	4,081	3,393	4,081

Table of Contents

10. Related Party Transactions

The Company s collaboration partner Celgene has made a series of equity investments in the Company, owning 3,674,640 shares of common stock representing 8.2% of the Company s fully diluted equity and 8.9% of the voting interests of the Company as of June 30, 2015. Refer to Note 7, *Collaborations*, and Note 11, *Subsequent Event*, for additional information regarding our original agreement with Celgene entered into in April 2012 and the amended and restated agreement with Celgene entered into in July 2015.

Under the Celgene collaboration agreement, the Company recognized $0.1 million and $0.2 million of collaboration revenue in the three and six months ended June 30, 2015, respectively, and $1.2 million and $2.9 million of collaboration revenue in the three and six months ended June 30, 2014, respectively. As of June 30, 2015 and December 31, 2014, the Company recorded $21.6 million and $21.7 million of deferred revenue related to the Celgene collaboration arrangement, respectively. Additionally, in the three and six months ended June 30, 2015, the Company recorded $0.4 million and $0.9 million, respectively, and $0.9 million and $1.3 million in the three and six months ended June 30, 2014, respectively, in global development co-funding from Celgene. As of June 30, 2015 and December 31, 2014, the Company recorded accounts receivable of $0.5 million and $1.1 million, respectively, related to this collaboration arrangement.

11. Subsequent Event

On July 8, 2015, the Company entered into an amendment and restatement of its collaboration and license agreement dated April 2, 2012 with Celgene. Refer to Note 7, *Collaborations*, for additional information regarding the original agreement.

Agreement Structure

Under the original agreement, the Company granted Celgene an exclusive license, for all countries other than the United States, to small molecule HMT inhibitors targeting DOT1L, including pinometostat, and an option, on a target-by-target basis, to exclusively license, for all countries other than the United States, rights to small molecule HMT inhibitors targeting any other HMT targets, excluding the EZH2 HMT including tazemetostat and targets covered by the Company s collaboration and license agreement with GSK. Under the original agreement, Celgene s option was exercisable during an option period that would have expired on July 9, 2015. Under the amended and restated collaboration and license agreement:

Celgene retains its exclusive license to small molecule HMT inhibitors targeting DOT1L, including pinometostat,

Celgene s other option rights have been narrowed to HMT inhibitors targeting three predefined targets (the Option Targets),

The exclusive licenses to HMT inhibitors targeting two of the Option Targets that Celgene may acquire have been expanded to include the United States, with the exclusive license to the third Option Target continuing to be for all countries other than the United States,

Celgene s option period has been extended for each of the Option Targets and is exercisable at the time of the Company s investigational new drug application (IND) filing for an HMT inhibitor targeting the applicable Option Target, upon the payment by Celgene at such time of a pre-specified development milestone-based license payment,

Celgene s license may be maintained beyond the end of Phase 1 clinical development for each of the Option Targets, upon payment by Celgene at such time of a pre-specified development milestone-based license payment, and

The Company s research and development obligations with respect to each Option Target under the amended agreement have been extended for at least an additional three years, subject to Celgene exercising its option with respect to such Option Target at IND filing. Subject to the Company s opt-out rights, the Company s research and development obligations have been expanded to include the completion of a Phase 1 clinical trial as to each Option Target following Celgene s exercise of its option at IND filing.

Under the amended agreement, the Company received a $10.0 million upfront payment in exchange for the Company s extension of Celgene s option rights to the Option Targets and the Company s research and development obligations. In addition, the Company is eligible to earn an aggregate of up to $75.0 million in development milestones and license payments, up to $365.0

Table of Contents

million in regulatory milestone payments and up to $170.0 million in sales milestone payments related to the three Option Targets. The Company is also eligible to receive royalties on each of the Option Targets as specified in the amended and restated agreement.

Due to the uncertainty of pharmaceutical development and the high historical failure rates generally associated with drug development, the Company may not receive any additional milestone or royalty payments from Celgene.

The amended agreement eliminated the right of first negotiation that the Company had granted to Celgene under the original agreement with respect to business combination transactions that the Company may desire to pursue with third parties.

The Company is primarily responsible for the research strategy under the collaboration. During each applicable option period the Company is required to use commercially reasonable efforts to carry out a mutually agreed-upon research plan for each Option Target. Subject to the Company s opt-out right, for the DOT1L target and each of the Option Targets, the Company is required to conduct and solely fund development costs of the Phase 1 clinical trials for HMT inhibitors directed to such targets, including for pinometostat. After the completion of Phase 1 development, as to DOT1L and the Option Target for which the Company retains U.S. rights, Celgene and the Company will equally co-fund global development and each party will solely fund territory-specific development costs for its respective territory; and, as to the other two Option Targets, after the completion of Phase 1 development, Celgene will solely fund all development costs on a worldwide basis.

Table of Contents

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

Forward-looking Information

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. These statements may be identified by such forward-looking terminology as anticipate, believe, estimate, expect, intend, may, plan, predict, project, target, potential, will, would, could, should, continue, and similar statements or variations of such terms. Our forward-looking statements are based on a series of expectations, assumptions, estimates and projections about our company, are not guarantees of future results or performance and involve substantial risks and uncertainty. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements. Our business and our forward-looking statements involve substantial known and unknown risks and uncertainties, including the risks and uncertainties inherent in our statements regarding:

our plans to develop and commercialize novel epigenetic therapies for cancer patients;

our ongoing and planned clinical trials, including the timing of initiation of the trials and anticipated results of the trials;

our ability to receive global development co-funding and achieve anticipated milestones under our collaborations;

the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;

the rate and degree of market acceptance and clinical utility of our products;

our commercialization, marketing and manufacturing capabilities and strategy;

our intellectual property position;

our ability to identify additional products or product candidates with significant commercial potential that are consistent with our commercial objectives; and

our estimates regarding expenses, future revenue, capital requirements and needs for additional financing. All of our forward-looking statements are as of the date of this Quarterly Report on Form 10-Q only. In each case, actual results may differ materially from such forward-looking information. We can give no assurance that such expectations or forward-looking statements will prove to be correct. An occurrence of or any material adverse change

in one or more of the risk factors or risks and uncertainties referred to in this Quarterly Report on Form 10-Q or included in our other public disclosures or our other periodic reports or other documents or filings filed with or furnished to the Securities and Exchange Commission, or the SEC, could materially and adversely affect our business, prospects, financial condition and results of operations. Except as required by law, we do not undertake or plan to update or revise any such forward-looking statements to reflect actual results, changes in plans, assumptions, estimates or projections or other circumstances affecting such forward-looking statements occurring after the date of this Quarterly Report on Form 10-Q, even if such results, changes or circumstances make it clear that any forward-looking information will not be realized. Any public statements or disclosures by us following this Quarterly Report on Form 10-Q which modify or impact any of the forward-looking statements contained in this Quarterly Report on Form 10-Q will be deemed to modify or supersede such statements in this Quarterly Report on Form 10-Q.

Management Overview

We are a clinical stage biopharmaceutical company that discovers, develops and plans to commercialize novel epigenetic therapies for cancer patients. We have built a proprietary product platform that we use to create small molecule inhibitors of a 96-member class of enzymes known as histone methyltransferases, or HMTs. HMTs are part of the system of gene regulation, referred to as epigenetics, that controls gene expression. Genetic alterations can result in changes to the activity of HMTs, making them oncogenic. These altered HMTs are referred to as oncogenes. The HMT target class has many potential oncogenes and, we believe, presents the opportunity to create, develop and commercialize multiple epigenetic therapeutics. Our therapeutic strategy is to treat the underlying causes of specific cancers by blocking the misregulated activity of oncogenic HMTs.

Our management s discussion and analysis of our financial condition and results of operations are based upon our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q, which have been prepared by us in accordance with accounting principles generally accepted in the United States of America, or GAAP, for interim periods and with Regulation S-X promulgated under the Securities Exchange Act of 1934, as amended, or the Exchange Act. This discussion and analysis should be read in conjunction with these unaudited condensed consolidated financial statements and the notes thereto as well as in conjunction with our Annual Report on Form 10-K for the fiscal year ended December 31, 2014, or

Table of Contents

our Annual Report. The three months ended June 30, 2015 and 2014 are referred to as the second quarter of 2015 and 2014, respectively. Unless the context indicates otherwise, all references herein to our company include our wholly-owned subsidiary.

We commenced active operations in early 2008, and since inception, have incurred significant operating losses. As we are a clinical stage company, we expect to continue to incur significant expenses and operating losses over the next several years. Since our inception and through June 30, 2015, we have raised an aggregate of $581.5 million to fund our operations, of which $191.0 million was non-equity funding through our collaboration agreements, $314.5 million was from the sale of common stock in our public offerings and $76.0 million was from the sale of redeemable convertible preferred stock. As of June 30, 2015, we had $236.7 million in cash and cash equivalents. In addition, in July 2015, we received an upfront payment of $10.0 million in connection with the execution of our amended and restated collaboration and license agreement with Celgene Corporation and Celgene RIVOT Ltd., an affiliate of Celgene Corporation (Celgene Corporation and its affiliated entities are collectively referred to as Celgene).

We are a leader in the translation of the science of epigenetics into first-in-class, novel epigenetic therapies for cancer patients and currently have two HMT inhibitors in clinical development for the treatment of patients with specific cancers. We believe we are the first company to conduct clinical trials of HMT inhibitors.

Our lead product candidate, tazemetostat (also known as EPZ-6438), is an inhibitor that targets the EZH2 HMT. We are currently conducting a Phase 1/2 clinical trial of tazemetostat in patients with relapsed or refractory B-cell lymphoma or advanced solid tumors. In 2014, we and our collaboration partner Eisai Co. Ltd., (Eisai), completed enrollment in the dose escalation portion of this Phase 1/2 clinical trial and disclosed the first clinical responses to treatment with tazemetostat from this ongoing Phase 1/2 clinical trial.

In March 2015, we reacquired global rights to develop, manufacture and commercialize tazemetostat outside of Japan from Eisai and substantially completed the transition of the EZH2 HMT program related activities from Eisai, including tazemetostat, by June 2015. We continued to dose patients within the dose escalation, dose expansion, and clinical pharmacology portions of the Phase 1/2 trial of tazemetostat throughout the second quarter of 2015 and initiated five-arm Phase 2 portion of the Phase 1/2 trial in June 2015. We expect to enroll in the Phase 2 portion of this trial approximately 150 relapsed or refractory NHL patients, prospectively stratified by cell of origin and EZH2 mutational status, with diffuse large B-cell lymphoma or follicular lymphoma. We also plan to commence a Phase 2 trial in adult patients with INI1-negative tumors or synovial sarcoma and a Phase 1 trial in pediatric patients with INI1-negative tumors or synovial sarcoma in the second half of 2015.

In 2012, we initiated a Phase 1 clinical trial of pinometostat (also known as EPZ-5676), an inhibitor targeting the DOT1L HMT and our second most advanced product candidate, in adult patients with MLL-r, an acute leukemia with genetic alterations of the *MLL* gene. In 2013, we completed enrollment in the dose escalation portion of this Phase 1 clinical trial and, in 2014, we completed enrollment in a 90 mg/m^2/day expansion cohort and disclosed the first clinical responses to treatment with pinometostat in heavily pretreated and relapsed or refractory patients with MLL-r. In the first quarter of 2015, we began enrolling additional patients in a second expansion cohort to investigate the activity of pinometostat at a dose of 54 mg/m^2/day. In August 2015, we announced that we would voluntarily cease patient enrollment into the Phase 1 study in adult patients with MLL-r due to insufficient efficacy of pinometostat as a monotherapy in the third quarter of 2015. We expect to present final study results after all patients conclude treatment and related data analyses are complete. We are continuing to conduct a Phase 1 dose escalation trial of pinometostat in pediatric patients with MLL-r, which we initiated in 2014.

In addition to our clinical programs, we also have a pipeline of wholly-owned HMT inhibitors that are in preclinical development that target our other prioritized HMTs. These programs are directed to a variety of hematological and

solid tumors.

In July 2015, we entered into an amended and restated collaboration agreement with Celgene. Under the amended agreement, we received a $10.0 million upfront payment in exchange for our extension of Celgene s option rights to license HMT inhibitors targeting three pre-defined targets and our research and development obligations. The option rights allow Celgene to

Table of Contents

acquire global rights for two of the targets and rights outside the United States for the third target. In addition, we are eligible to earn an aggregate of up to $75.0 million in development milestones and license payments, up to $365.0 million in regulatory milestone payments, up to $170.0 million in sales milestone payments as well as royalties on net product sales in Celgene s territories at defined percentages, subject to reductions in specified circumstances related to the three pre-defined targets. As to DOT1L, we retain all product rights in the United States and are eligible to receive royalties at defined percentages on annual net product sales outside of the United States, subject to reductions in specified circumstances.

The following table summarizes key information about our clinical product candidates:

Table of Contents

Program highlights for the three months ended June 30, 2015 include:

For tazemetostat, we substantially completed transition of the EZH2 HMT program related activities, including tazemetostat, to Epizyme under the amended and restated collaboration and license agreement with Eisai. We continued dosing patients who remained on study in the dose escalation and dose expansion (800 mg and 1600 mg twice daily oral administration) portions of the Phase 1/2 clinical trial and continued enrolling patients in a clinical pharmacology study evaluating the effect of food on blood levels of tazemetostat. In May 2015, we initiated a five-arm Phase 2 portion of the Phase 1/2 clinical trial and commenced enrolling patients in this trial in June 2015. We provided an update on NHL patients from the Phase 1 portion of the Phase 1/2 clinical trial at the International Conference on Malignant Lymphoma in June 2015. We plan to present additional updated data from patients with advanced solid tumors in the Phase 1 portion of the Phase 1/2 clinical trial at the European Cancer Congress, hosted by the European Society of Medical Oncology in September 2015.

For pinometostat, we continued enrolling adult MLL-r patients in a 54 mg/m^2/day Phase 1 expansion cohort and pediatric MLL-r patients in our ongoing Phase 1 dose escalation trial in the second quarter of 2015. In August 2015, we announced that we would voluntarily cease patient enrollment into the Phase 1 study in adult patients with MLL-r due to insufficient efficacy of pinometostat as a monotherapy in the third quarter of 2015. We are continuing to conduct a Phase 1 dose escalation trial of pinometostat in pediatric patients with MLL-r and expect to complete enrollment in the pediatric study in the second half of 2015.

We continued to progress a number of other research programs directed to HMTs in our pipeline and presented research data at the American Association for Cancer Research Annual Meeting in April 2015 on several additional HMTs, including CARM1, PRMT6, SMYD3 and SETDB1.

Collaborations

The key terms of our primary collaboration agreements are as follows:

Eisai

In April 2011, we entered into a collaboration and license agreement with Eisai, which we refer to as the original agreement, under which we granted Eisai an exclusive worldwide license to our small molecule HMT inhibitors directed to the EZH2 HMT, including our product candidate tazemetostat, while retaining an opt-in right to co-develop, co-commercialize and share profits with Eisai on licensed products in the United States. Additionally, as part of the research collaboration, we provided research and development services related to the licensed compounds through December 31, 2014.

In March 2015, we entered into an amended and restated collaboration and license agreement with Eisai, under which we reacquired worldwide rights, excluding Japan, to our EZH2 program, including tazemetostat. Under the amended and restated collaboration and license agreement, we are responsible for global development, manufacturing and commercialization outside of Japan of tazemetostat and any other EZH2 product candidates, and Eisai has retained development and commercialization rights in Japan, as well as a right to elect to manufacture tazemetostat and any other EZH2 product candidates in Japan.

Under the original agreement, Eisai was solely responsible for funding all research, development and commercialization costs for EZH2 compounds. Under the amended and restated collaboration and license agreement, we are solely responsible for funding global development, manufacturing and commercialization costs for EZH2 compounds outside of Japan, including up to $15.5 million of the remaining development costs due under a Roche companion diagnostic agreement, and Eisai is solely responsible for funding Japan-specific development and commercialization costs for EZH2 compounds.

We recorded a $40.0 million upfront payment made to Eisai in March 2015 in connection with the amended and restated collaboration and license agreement as research and development expense in the three months ended March 31, 2015. We have also agreed to pay Eisai up to $20.0 million in clinical development milestone payments, up to $50.0 million in regulatory milestone payments and royalties at a percentage in the mid-teens on worldwide net sales of any EZH2 product, excluding net sales in Japan. We are eligible to receive from Eisai royalties at a percentage in the mid-teens on net sales of any EZH2 product in Japan.

Table of Contents

Celgene

In April 2012, we entered into a collaboration and license agreement with Celgene Corporation and Celgene International Sàrl, an affiliate of Celgene Corporation, to discover, develop and commercialize, in all countries other than the United States, small molecule HMT inhibitors targeting DOT1L, including pinometostat, and any other HMT targets from our product platform, other than the EZH2 HMT including tazemetostat and targets covered by our collaboration with Glaxo Group Limited, an affiliate of GlaxoSmithKline (GSK), which we refer to as the available targets. In July 2015, we entered into an amendment and restatement of our collaboration and license agreement with Celgene.

Agreement Structure

Under the original agreement, we granted Celgene an exclusive license, for all countries other than the United States, to small molecule HMT inhibitors targeting DOT1L, including pinometostat, and an option, on a target-by-target basis, to exclusively license, for all countries other than the United States, rights to small molecule HMT inhibitors targeting any other HMT targets, other than the EZH2 HMT including tazemetostat and targets covered by our collaboration and license agreement with GSK. Under the original agreement, Celgene s option was exercisable during an option period that would have expired on July 9, 2015. Under the amended and restated collaboration and license agreement:

Celgene retains its exclusive license to small molecule HMT inhibitors targeting DOT1L, including pinometostat,

Celgene s other option rights have been narrowed to HMT inhibitors targeting three predefined targets (the Option Targets),

The exclusive licenses to HMT inhibitors targeting two of the Option Targets that Celgene may acquire have been expanded to include the United States, with the exclusive license to the third Option Target continuing to be for all countries other than the United States,

Celgene s option period has been extended for each of the Option Targets and is exercisable at the time of the Company s IND filing for an HMT inhibitor targeting the applicable Option Target, upon the payment by Celgene at such time of a pre-specified development milestone-based license payment,

Celgene s license may be maintained beyond the end of Phase 1 clinical development for each of the Option Targets, upon payment by Celgene at such time of a pre-specified development milestone-based license payment, and

Our research and development obligations with respect to each Option Target under the amended agreement have been extended for at least an additional three years, subject to Celgene exercising its option with respect to such Option Target at IND filing. Subject to our opt-out rights, our research and development obligations have been expanded to include the completion of a Phase 1 clinical trial as to

each Option Target following Celgene s exercise of its option at IND filing.

Under the amended agreement, we received a $10.0 million upfront payment in exchange for our extension of Celgene s option rights to the Option Targets and our research and development obligations. In addition, we are eligible to earn an aggregate of up to $75.0 million in development milestones and license payments, up to $365.0 million in regulatory milestone payments and up to $170.0 million in sales milestone payments related to the three Option Targets. As to DOT1L, the Company retains all product rights in the United States and is eligible to receive royalties at defined percentages ranging from the mid-single digits to the mid-teens on annual net product sales outside of the United States, subject to reductions in specified circumstances. As to the Option Target for which Celgene s option rights do not include the United States, if Celgene exercises its option as to such Option Target, we will retain all product rights in the United States and will be eligible to receive royalties, once an initial threshold of net product sales (for which we will not receive royalties) is exceeded, at defined percentages ranging from the mid-single digits to the low-double digits, on net product sales outside of the United States, subject to reductions in

Table of Contents

specified circumstances. As to the other two Option Targets, if Celgene exercises its option as to those Option Targets, we will be eligible to receive royalties, once an initial threshold of net product sales (for which we will not receive royalties) is exceeded, for each such Option Target at defined percentages ranging from the mid-single digits to the low-double digits, on net product sales on a worldwide basis, subject to reductions in specified circumstances.

Under the original agreement, we received a $65.0 million upfront payment and $25.0 million from the sale of our series C redeemable convertible preferred stock to an affiliate of Celgene, of which $3.0 million was considered a premium and included as collaboration arrangement consideration for a total upfront payment of $68.0 million. In addition, we have recorded a $25.0 million clinical development milestone payment and $6.7 million of global development co-funding through June 30, 2015. We remain eligible to earn $35.0 million in an additional clinical development milestone payment and up to $100.0 million in regulatory milestone payments related to DOT1L. We are also eligible to receive royalties on each of the Option Targets as specified in the amended and restated agreement.

Due to the uncertainty of pharmaceutical development and the high historical failure rates generally associated with drug development, we may not receive any additional milestone or royalty payments from Celgene.

The amended agreement eliminated the right of first negotiation that we had granted to Celgene under the original agreement with respect to business combination transactions that we may desire to pursue with third parties.

We are primarily responsible for the research strategy under the collaboration. During each applicable option period we are required to use commercially reasonable efforts to carry out a mutually agreed-upon research plan for each Option Target. Subject to our opt-out right, for the DOT1L target and each of the Option Targets, we are required to conduct and solely fund development costs of the Phase 1 clinical trials for HMT inhibitors directed to such targets, including for pinometostat. After the completion of Phase 1 development, as to DOT1L and the Option Target for which we retain U.S. rights, we and Celgene will equally co-fund global development and each party will solely fund territory-specific development costs for its respective territory. For the other two Option Targets, after the completion of Phase 1 development, Celgene will solely fund all development costs on a worldwide basis.

Collaboration Revenue

Through June 30, 2015, in addition to amounts allocated to Celgene s purchase of shares of our series C redeemable convertible preferred stock, we recorded a total of $99.8 million in cash and accounts receivable under the Celgene agreement, including the $3.0 million implied premium on Celgene s purchase of our series C redeemable convertible preferred stock. Through June 30, 2015, we recognized $71.5 million of collaboration revenue, including $0.1 million and $0.2 million in the three and six months ended June 30, 2015, respectively, and $1.2 million and $2.9 million in the three and six months ended June 30, 2014, respectively, and $6.7 million of global development co-funding as a reduction to research and development expense, including $0.4 million and $0.9 million in the three and six months ended June 30, 2015, respectively, and $0.9 million and $1.3 million in the three and six months ended June 30, 2014, respectively, in the condensed consolidated statements of operations and comprehensive loss related to this agreement. As of June 30, 2015 and December 31, 2014, we had deferred revenue of $21.6 million and $21.7 million, respectively, related to this agreement.

GSK

In January 2011, we entered into a collaboration and license agreement with GSK to discover, develop and commercialize novel small molecule HMT inhibitors directed to available targets from our product platform. Under the terms of the agreement, we granted GSK exclusive worldwide license rights to HMT inhibitors directed to three

targets. Additionally, as part of the research collaboration provided for in the agreement, we agreed to provide research and development services related to the licensed targets pursuant to agreed upon research plans during a research term that ended January 8, 2015. In March 2014, we and GSK amended certain terms of this agreement for the third licensed target, revising the license terms with respect to candidate compounds and amending the corresponding financial terms, including reallocating milestone payments and increasing royalty rates as to the third target. We substantially completed our research obligations under this agreement by the end of the first quarter of 2015 and completed the transfer of the remaining data and materials for these programs to GSK in the second quarter of 2015.

Table of Contents

Agreement Structure

Under the agreement, we recorded a $20.0 million upfront payment, a $3.0 million payment upon the execution of the March 2014 agreement amendment, $6.0 million of fixed research funding, $15.0 million of preclinical research and development milestone payments and $9.0 million for research and development services. We are eligible to receive up to $18.0 million in additional preclinical research and development milestone payments, up to $109.0 million in clinical development milestone payments, up to $275.0 million in regulatory milestone payments and up to $218.0 million in sales-based milestone payments. In addition, GSK is required to pay us royalties at percentages from the mid-single digits to the low double-digits, on a licensed product-by-licensed product basis, on worldwide net product sales, subject to reductions in specified circumstances. Due to the uncertainty of pharmaceutical development and the high historical failure rates generally associated with drug development, we may not receive any additional milestone payments or royalty payments from GSK. Due to the varying stages of development of each licensed target, the Company is not able to predict the next milestone that might be achieved under this agreement, if any. GSK became solely responsible for development and commercialization for each licensed target in the collaboration when the research term ended on January 8, 2015.

For each licensed target in the collaboration, we were primarily responsible for research until the earlier of selection of a development candidate for the target or January 8, 2015. GSK has been solely responsible for subsequent development and commercialization since the research term ended on January 8, 2015. GSK provided a fixed amount of research funding during the second and third years of the research term and research funding equal to 100.0% of research and development costs, subject to specified limitations, for research activities we conducted in the fourth year of the research term.

Collaboration Revenue

Through June 30, 2015, we received a total of $53.0 million in cash which we recognized as collaboration revenue in the condensed consolidated statements of operations and comprehensive loss related to this agreement, including $0.6 million and $1.4 million in the three and six months ended June 30, 2015, respectively, and $6.3 million and $16.4 million in the three and six months ended June 30, 2014, respectively, including a $1.0 million preclinical research and development milestone achieved and recognized as collaboration revenue in the three months ended June 30, 2014 and an additional $2.0 million preclinical research and development milestone achieved and recognized as collaboration revenue in the six months ended June 30, 2014. As of December 31, 2014, we had deferred revenue of $1.4 million related to this agreement, which we fully recognized as collaboration revenue by June 30, 2015.

Results of Operations

Collaboration Revenue

The following is a comparison of collaboration revenue for the three and six months ended June 30, 2015 and 2014:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2015	2014	Decrease	2015	2014	Decrease
	(In millions)					
Collaboration revenue	$ 0.7	$ 9.5	$ (8.8)	$ 1.6	$ 22.9	$ (21.3)

Our revenue consists of collaboration revenue, including amounts recognized from deferred revenue related to upfront payments for licenses or options to obtain licenses in the future, research and development services revenue earned

and milestone payments earned under collaboration and license agreements with our collaboration partners.

During the three months ended June 30, 2015, collaboration revenue consisted of $0.7 million recognized from deferred revenue related to upfront payments for licenses. This revenue compares to $4.1 million recognized from deferred revenue related to upfront payments for licenses, $1.0 million in milestone revenue and $4.4 million in research and development funding recognized in the three months ended June 30, 2014.

Table of Contents

Collaboration revenue recognized from deferred revenue in the three months ended June 30, 2015 consisted of $0.1 million under our Celgene agreement and $0.6 million under our GSK agreement, as compared to $1.2 million under our Celgene agreement, $2.5 million under our GSK agreement and $0.4 million under our original Eisai agreement in the three months ended June 30, 2014. We did not recognize any collaboration revenue for research and development services in the three months ended June 30, 2015, as compared to $2.8 million under our GSK agreement and $1.6 million under our original Eisai agreement in the three months ended June 30, 2014. Milestone revenue in the three months ended June 30, 2014 consisted of a $1.0 million preclinical research and development milestone achieved under our GSK agreement in April 2014. We had no milestone revenue in the three months ended June 30, 2015.

During the six months ended June 30, 2015 collaboration revenue consisted of $1.2 million recognized from deferred revenue related to upfront payments for licenses and $0.4 million in research and development funding. This revenue compares to $10.9 million recognized from deferred revenue related to upfront payments for licenses, $3.0 million in milestone revenue and $9.0 million in research and development funding recognized in the six months ended June 30, 2014.

Collaboration revenue recognized from deferred revenue in the six months ended June 30, 2015 consisted of $0.2 million under our Celgene agreement and $1.0 million under our GSK agreement, as compared to $2.9 million under our Celgene agreement, $0.8 million under our Eisai agreement and $7.2 million under our GSK agreement in the six months ended June 30, 2014. Collaboration revenue recognized for research and development services in the six months ended June 30, 2015 consisted of $0.4 million under our GSK agreement, as compared to $2.8 million under our Eisai agreement and $6.2 million under our GSK agreement in the six months ended June 30, 2014. Milestone revenue in the six months ended June 30, 2014 consisted of $3.0 million in preclinical research and development milestones achieved under our GSK agreement. We had no milestone revenue in the six months ended June 30, 2015.

Research and Development

The following is a comparison of research and development expenses for the three and six months ended June 30, 2015 and 2014:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2015	2014	Increase	2015	2014	Increase
	(In millions)					
Research and development	$ 20.6	$ 17.5	$ 3.1	$ 77.6	$ 32.8	$ 44.8

Research and development expenses consist of expenses incurred in performing research and development activities, including compensation and benefits for full-time research and development employees, facilities expenses, overhead expenses, clinical trial and related clinical manufacturing expenses, fees paid to third party contract research organizations, or CROs, and other outside expenses, including, in the first quarter of 2015, the $40.0 million upfront payment to Eisai in connection with our amended and restated collaboration and license agreement under which we reacquired worldwide rights, excluding Japan, to our EZH2 program, including tazemetostat. As we advance our product platform, we are conducting research on several prioritized HMT targets. Our research and development team is organized such that the strategy, design, management and evaluation of results of all of our research and development plans is accomplished internally while some of our research and development activities are executed using our multinational network of CROs. In the early phases of development, our research and development costs are often devoted to enhancing our product platform and are not necessarily allocable to specific targets. In circumstances, such as our Celgene collaboration, where our collaboration and license agreements provide for equally co-funded

global development under joint risk sharing collaborations, amounts received from collaboration partners for such co-funding are recorded as a reduction to research and development expense.

Table of Contents

The following table illustrates the components of our research and development expenses:

Product Program (Phase as of the latest period end)	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
	(In millions)			
External research and development expenses:				
Tazemetostat (Phase 1/2) and related EZH2 programs	$ 8.6	$ 0.9	$ 50.0	$ 1.5
Pinometostat (Phase 1) and related DOT1L programs	1.4	3.4	3.4	6.5
Discovery and preclinical stage product programs, collectively	3.5	7.0	9.7	12.7
Internal research and development expenses	7.1	6.2	14.5	12.1
Total research and development expenses	$ 20.6	$ 17.5		