Epizyme, Inc.
Form 10-Q
October 23, 2013
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 September 30, 2013

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 ¨
Non-accelerated filer x (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 October 18, 2013: 28,419,288 shares.

Table of Contents

PART I - FINANCIAL INFORMATION

Item 1. Financial Statements.

Consolidated Balance Sheets as of September 30, 2013 and December 31, 2012 2

Consolidated Statements of Operations and Comprehensive (Loss) Income for the Three and Nine Months Ended September 30, 2013 and 2012 3

Consolidated Statements of Cash Flows for the Nine Months Ended September 30, 2013 and 2012 4

Notes to Consolidated Financial Statements 5

Item 2. Management s Discussion and Analysis of Financial Condition and Results of Operations 14

Item 3. Quantitative and Qualitative Disclosures About Market Risk 24

Item 4. Controls and Procedures 24

PART II - OTHER INFORMATION

Item 1A. Risk Factors 24

Item 2. Use of Proceeds 51

Item 5. Other Information 52

Item 6. Exhibits 52

Signatures 53

Table of Contents

PART I FINANCIAL INFORMATION

Item 1. Financial Statements

EPIZYME, INC.

CONSOLIDATED BALANCE SHEETS (UNAUDITED)

(Amounts in thousands except share and per share data)

	September 30, 2013	December 31, 2012
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 139,575	$ 97,981
Accounts receivable	2,155	1,829
Prepaid expenses and other current assets	2,130	815
Total current assets	143,860	100,625
Property and equipment, net	2,028	2,140
Restricted cash and other assets	1,462	746
Total Assets	$ 147,350	$ 103,511
LIABILITIES AND STOCKHOLDERS EQUITY (DEFICIT)		
Current Liabilities:		
Accounts payable	$ 3,525	$ 2,967
Accrued expenses	5,384	4,328
Current portion of deferred revenue	21,556	28,208
Total current liabilities	30,465	35,503
Deferred revenue, net of current portion	29,150	41,237
Other long-term liabilities	452	1,741
Commitments and contingencies		
Redeemable convertible preferred stock, $0.0001 par value; 5,000,000 shares and 61,899,922 shares (Series A, B and C) authorized, respectively; 0 shares and 61,899,165 shares issued and outstanding, respectively; aggregate liquidation preference of $0 and $79,000, respectively		76,156
Stockholders Equity (Deficit):		
Common stock, $0.0001 par value; 125,000,000 shares and 90,000,000 shares authorized, respectively; 28,418,420 shares and 1,694,862 shares issued, respectively; 28,408,698 shares and 1,672,639 shares outstanding, respectively	3	
Treasury stock, at cost; 0 shares and 11,544 shares, respectively		
Additional paid-in capital	159,290	1,471
Accumulated deficit	(72,010)	(52,597)

Total stockholders equity (deficit)	87,283	(51,126)
Total Liabilities and Stockholders Equity (Deficit)	$ 147,350	$ 103,511

See notes to consolidated financial statements.

Table of Contents

EPIZYME, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE (LOSS) INCOME
(UNAUDITED)

(Amounts in thousands except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Collaboration revenue	$ 8,444	$ 15,331	$ 32,165	$ 36,327
Operating expenses:				
Research and development	14,584	9,258	41,882	27,385
General and administrative	3,587	1,630	9,664	5,175
Total operating expenses	18,171	10,888	51,546	32,560
Operating (loss) income	(9,727)	4,443	(19,381)	3,767
Other income (expense):				
Interest income	20	44	54	108
Other income (expense), net	3	(39)	(86)	(39)
Other income (expense), net	23	5	(32)	69
Net (loss) income	$ (9,704)	$ 4,448	$ (19,413)	$ 3,836
Less: accretion of redeemable convertible preferred stock to redemption value		159	264	326
Less: income allocable to participating securities		3,972		3,239
(Loss) income allocable to common stockholders basic	(9,704)	317	(19,677)	271
Undistributed income re-allocated to common stockholders		229		147
(Loss) income allocable to common stockholders diluted	$ (9,704)	$ 546	$ (19,677)	$ 418
(Loss) earnings per share allocable to common stockholders:				
Basic	$ (0.34)	$ 0.19	$ (1.49)	$ 0.17
Diluted	$ (0.34)	$ 0.18	$ (1.49)	$ 0.16
Weighted average shares outstanding:				
Basic	28,406	1,651	13,212	1,637
Diluted	28,406	3,017	13,212	2,641
Comprehensive (loss) income	$ (9,704)	$ 4,448	$ (19,413)	$ 3,836

See notes to consolidated financial statements.

Table of Contents

EPIZYME, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS (UNAUDITED)

(Amounts in thousands)

	Nine Months Ended September 30,	
	2013	**2012**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net (loss) income	$ (19,413)	$ 3,836
Adjustments to reconcile net loss to net cash (used in) provided by operating activities:		
Depreciation and amortization	534	440
Stock-based compensation	1,819	210
Loss on disposal of property and equipment		20
Changes in operating assets and liabilities:		
Accounts receivable	(326)	(3,663)
Prepaid expenses and other current assets	(1,315)	(553)
Accounts payable	364	(517)
Accrued expenses	1,052	1,933
Deferred revenue	(18,739)	45,952
Restricted cash and other assets	(716)	(495)
Other long-term liabilities	(1,289)	1,190
Net cash (used in) provided by operating activities	(38,029)	48,353
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property and equipment	(230)	(239)
Net cash used in investing activities	(230)	(239)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from sale of redeemable convertible preferred stock		21,961
Proceeds from initial public offering, net of commissions	82,491	
Proceeds from stock options exercised	171	3
Payment of redeemable convertible preferred stock issuance costs		(38)
Payment of initial public offering costs	(2,809)	
Net cash provided by financing activities	79,853	21,926
Net increase in cash and cash equivalents	41,594	70,040
Cash and cash equivalents, beginning of period	97,981	33,341
Cash and cash equivalents, end of period	$ 139,575	$ 103,381
SUPPLEMENTAL DISCLOSURES OF CASH FLOW INFORMATION:		

Purchases of property and equipment unpaid at period end	192	
Conversion of redeemable convertible preferred stock to common stock	76,420	
Accretion of redeemable convertible preferred stock to redemption value	264	326
Vesting of restricted stock liability		9
Initial public offering costs incurred but unpaid at period end	6	

See notes to consolidated financial statements.

Table of Contents

EPIZYME, INC.

NOTES TO 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 innovative personalized therapeutics for patients with genetically defined cancers. 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 genetically defined cancers.

The 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 consolidated financial statements should be read in conjunction with the financial statements and notes thereto included in the Company s Prospectus filed with the SEC pursuant to Rule 424(b)(4) on May 31, 2013 (the Prospectus).

The unaudited 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 September 30, 2013 and 2012 are referred to as the third quarter of 2013 and 2012, 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.

On June 5, 2013, the Company completed an initial public offering (IPO) of its common stock, which resulted in the sale of 5,913,300 shares, including all additional shares available to cover over-allotments, at a price of $15.00 per share. The Company received net proceeds before expenses from the IPO of $82.5 million after deducting underwriting discounts and commissions paid by the Company. In preparation for the IPO, the Company s Board of Directors and stockholders approved a one-for-three reverse stock split of the Company s common stock effective May 13, 2013. All share and per share amounts in the consolidated financial statements and notes thereto have been retroactively adjusted, where necessary, to give effect to this reverse stock split. In connection with the closing of the IPO, all of the Company s outstanding redeemable convertible preferred stock automatically converted to common stock at a one-for-three ratio as of June 5, 2013, resulting in an additional 20,633,046 shares of common stock of the Company becoming outstanding. Following these transactions, the Company s total issued common stock as of September 30, 2013 was 28,418,420 shares. The significant increase in shares outstanding in June 2013 is expected to impact the year-over-year comparability of the Company s (loss) earnings per share calculations through 2014.

2. Summary of Significant Accounting Policies

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

Recent Accounting Pronouncements

In July 2013, the Financial Accounting Standards Board issued Accounting Standards Update (ASU) No. 2013-11, *Presentation of an Unrecognized Tax Benefit When a Net Operating Loss Carryforward, a Similar Tax Loss, or a Tax Credit Carryforward Exists*. ASU 2013-11 amends Accounting Standards Codification (ASC) 740, *Income Taxes*, by providing guidance on the financial statement presentation of an unrecognized benefit when a net operating loss carryforward, a similar tax loss, or a tax credit carryforward exists. The ASU does not affect the recognition or measurement of uncertain tax positions under ASC 740. ASU 2013-11 will be effective for the Company for interim and annual periods beginning after December 15, 2013, with early adoption permitted. The Company does not expect the adoption of this ASU to have any impact on its consolidated financial statements.

Table of Contents

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 certain pricing models, discounted cash flow methodologies and similar techniques that use significant unobservable inputs.

The Company s financial instruments as of September 30, 2013 and December 31, 2012 consisted primarily of cash and cash equivalents, accounts receivable and accounts payable. The Company believes the carrying value of its cash, accounts receivable and accounts payable approximates the fair value due to the short-term nature of these instruments. As of September 30, 2013 and December 31, 2012, the Company s financial assets recognized at fair value consisted of the following:

	Fair Value as of September 30, 2013			
	Total	Level 1	Level 2	Level 3
	(In thousands)			
Cash equivalents	$ 121,409	$ 121,409	$	$
Total	$ 121,409	$ 121,409	$	$

	Fair Value as of December 31, 2012			
	Total	Level 1	Level 2	Level 3
	(In thousands)			
Cash equivalents	$ 97,375	$ 97,375	$	$
Total	$ 97,375	$ 97,375	$	$

4. Accrued Expenses

Accrued expenses consisted of the following:

	September 30, 2013	December 31, 2012
	(In thousands)	
Employee compensation and benefits	$ 1,882	$ 1,880
Current portion of contract termination obligations	538	1,274
Research and development and professional expenses	2,964	1,174
Accrued expenses	$ 5,384	$ 4,328

Contract termination obligations include estimated repayments related to the termination of a research agreement in June 2012 and estimated lease exit charges related to the Company s former facility at 325 Vassar Street in Cambridge, Massachusetts. The Company s obligation related to its termination of a research agreement was accelerated as a result of the closing of the Company s IPO, and, as a result, this termination obligation was paid in full in June 2013. As of December 31, 2012, the Company had recorded contract termination obligations of $3.0 million. During the nine months ended September 30, 2013, the Company recorded a net non-cash liability reduction of $0.1 million and made cash payments of $2.3 million, resulting in total remaining contract termination obligations of $0.6 million as of September 30, 2013. The non-current portion of contract termination obligations is included in other long-term liabilities.

Table of Contents

5. Income Taxes

The Company did not record a federal or state income tax provision or benefit for the three or nine months ended September 30, 2013 and 2012 due to the expected loss before income taxes to be incurred and the actual loss before income taxes incurred for the years ended December 31, 2013 and 2012, respectively, as well as the Company s continued maintenance of a full valuation allowance against its net deferred tax assets.

6. Commitments

In September 2013, the Company entered into an amendment to the operating lease agreement for its current office and laboratory facility in Cambridge, Massachusetts, under which the Company leased additional office space for aggregate rent payments of $2.5 million. These additional rent payments are expected to be paid from the commencement of the lease for this additional space, which is projected to be in the first quarter of 2014, through the end of the existing lease agreement, November 30, 2017.

7. Collaborations

Celgene

In April 2012, the Company entered into a collaboration and license agreement with Celgene Corporation and Celgene International Sàrl (collectively, Celgene) to discover, develop and commercialize, in all countries other than the United States, small molecule HMT inhibitors targeting the DOT1L HMT, including the Company s product candidate EPZ-5676, and any other HMT targets from the Company s product platform for patients with genetically defined cancers, excluding targets already selected by the Company s two other existing therapeutic collaborations (the available targets).

Agreement Structure

Under the terms of the 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 is eligible to earn up to $60.0 million in substantive clinical development milestone payments and up to $100.0 million in substantive regulatory milestone payments related to DOT1L, where milestones are considered to be substantive if (a) the consideration is commensurate with either the Company s performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the Company s performance to achieve the milestone; (b) the consideration relates solely to past performance; and (c) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. The Company is also eligible to earn up to $65.0 million in payments, including a combination of substantive clinical development milestone payments and an option exercise fee, and up to $100.0 million in substantive regulatory milestone payments for each available target as to which Celgene exercises its option during an initial option period ending in July 2015. Celgene has the right to extend the option period until July 2016 by making a significant option extension payment. As to DOT1L and each available target as to which Celgene exercises its option, the Company retains all product rights in the United States and is 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. Due to the uncertainty of pharmaceutical development and the high historical failure rates generally associated with pharmaceutical development, the Company may not receive any milestone or royalty payments from Celgene. The first potential milestone payment that the Company might be

entitled to receive under this agreement is a $25.0 million substantive milestone for achieving proof-of-concept, as defined in the agreement, for its DOT1L inhibitor.

The Company is obligated to conduct and solely fund research and development costs of the Phase I clinical trials for the DOT1L target and through the effectiveness of the first investigational new drug application (IND) for an HMT inhibitor directed to each available target selected by Celgene, after which Celgene and the Company will equally co-fund global development and each party will solely fund territory-specific development costs for its territory. In the third quarter of 2013, the Company recorded accounts receivable of $0.7 million related to non-Phase I global development costs subject to the co-funding provisions of the agreement. Co-funded amounts receivable from Celgene are recorded net in research and development expense.

Table of Contents

Collaboration Revenue

Through September 30, 2013, in addition to amounts allocated to Celgene s purchase of shares of the Company s series C redeemable convertible preferred stock, the Company had received a total of $68.0 million in upfront payments under the Celgene agreement, including a $3.0 million implied premium on Celgene s purchase of shares of the Company s series C redeemable convertible preferred stock. Through September 30, 2013, the Company has recognized $34.7 million of collaboration revenue in the consolidated statements of operations and comprehensive (loss) income related to this agreement, including $3.5 million and $10.8 million in the three and nine months ended September 30, 2013, respectively, and $6.7 million and $20.3 million in the three and nine months ended September 30, 2012, respectively. Revenue recognized in the three and nine months ended September 30, 2013 reflects the Company s current plan to complete the ongoing Phase I study of EPZ-5676 in 2014. As a result, the remaining deferred revenue of approximately $2.1 million attributed to this deliverable as of September 30, 2013 will be recognized ratably through December 31, 2014. As of September 30, 2013 and December 31, 2012, the Company had deferred revenue of $33.3 million and $44.1 million, respectively, related to this agreement.

Eisai

In April 2011, the Company entered into a collaboration and license agreement with Eisai Co. Ltd. (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 EPZ-6438, while retaining an opt-in right to co-develop, co-commercialize and share profits with Eisai as to licensed products in the United States. Additionally, as part of the research collaboration, the Company agreed to provide research and development services related to the licensed compounds through December 31, 2014.

Agreement Structure

Under the terms of the agreement, the Company has recorded a $3.0 million upfront payment, $7.0 million in preclinical research and development milestone payments, a $6.0 million clinical development milestone payment and cash payments and accounts receivable totaling $16.5 million for research and development services through September 30, 2013. The Company is eligible to earn up to $25.0 million in additional clinical development milestone payments, including substantive milestone payments of up to $10.0 million, up to $55.0 million in regulatory milestone payments and up to $115.0 million in sales-based milestone payments. The Company is also eligible to receive royalties at a percentage in the mid-single digits on any net product sales outside of the United States and at a percentage from the mid-single digits to low double-digits on any product sales in the United States, subject to reduction in specified circumstances. Due to the uncertainty of pharmaceutical development and the high historical failure rates generally associated with pharmaceutical development, the Company may not receive any additional milestone payments or royalty or profit share payments from Eisai. The next potential milestone payment that the Company might be entitled to receive under this agreement is a $10.0 million substantive milestone for the initiation of the Phase II portion of the Phase I/II clinical trial.

Eisai solely funds all research, development and commercialization costs for licensed compounds, except for the cost obligations that the Company will undertake if it exercises its opt-in right to co-develop, co-commercialize and share profits with Eisai as to licensed products in the United States. If the Company exercises its opt-in right to a licensed compound, the licensed compound would become a shared product as to which Eisai s obligation to pay royalties to the Company as to such shared product in the United States will terminate; Eisai and the Company will share in net profits or losses with respect to such shared product in the United States; 25.0% of specified past development costs will become creditable by Eisai against future milestones or royalties due to the Company, subject to specified limitations; Eisai and the Company will share equally in subsequent development costs allocated to the United States;

and all subsequent milestone payments that become payable by Eisai to the Company based on the shared product will be decreased by 50.0%.

Collaboration Revenue

Through September 30, 2013, the Company has recorded a total of $32.5 million in cash and accounts receivable and has recognized $30.5 million of collaboration revenue in the consolidated statements of operations and comprehensive (loss) income related to this agreement, including $1.9 million and $12.4 million in the three and nine months ended September 30, 2013,

Table of Contents

respectively, and $1.9 million and $9.3 million in the three and nine months ended September 30, 2012, respectively, with a $6.0 million clinical development milestone achieved and recognized as collaboration revenue in the nine months ended September 30, 2013 and a $4.0 million research milestone achieved and recognized as collaboration revenue in the nine months ended September 30, 2012. As of September 30, 2013 and December 31, 2012, the Company had deferred revenue of $2.0 million and $3.2 million, respectively, related to this agreement.

GSK

In January 2011, the Company entered into a collaboration and license agreement with Glaxo Group Limited, an affiliate of GlaxoSmithKline (GSK), to discover, develop and commercialize novel small molecule HMT inhibitors directed to available targets from the Company s product platform. Under the terms of the agreement, the Company has 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, 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 ends January 8, 2015.

Agreement Structure

Under the agreement, the Company has received a $20.0 million upfront payment, $8.0 million in preclinical research and development milestone payments and $6.0 million of fixed research funding. The Company is eligible to receive up to $21.0 million in additional substantive preclinical research and development milestone payments, up to $99.0 million in clinical development milestone payments, up to $240.0 million in regulatory milestone payments and up to $270.0 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 pharmaceutical development, the Company may not receive any additional milestone payments or royalty payments from GSK. The next potential milestone payment that the Company might be entitled to receive under this agreement is a substantive research milestone. However, 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, if any.

For each selected target in the collaboration, the Company is primarily responsible for research until the selection of the development candidate, and GSK will be solely responsible for subsequent development and commercialization. GSK provided a fixed amount of research funding during the second and third years of the research term and is obligated to provide research funding equal to 100.0% of research and development costs, subject to specified limitations, for any research activities conducted by the Company in the fourth year of the research term.

Collaboration Revenue

Through September 30, 2013, the Company has received a total of $34.0 million in payments under the GSK agreement and has recognized $18.7 million of collaboration revenue in the consolidated statements of operations and comprehensive (loss) income related to this agreement, including $3.0 million and $9.0 million in the three and nine months ended September 30, 2013, respectively, and $6.7 million in both the three and nine months ended September 30, 2012, with $4.0 million in preclinical research and development milestones achieved and recognized as collaboration revenue in the three and nine months ended September 30, 2012. As of September 30, 2013 and December 31, 2012, the Company had deferred revenue of $15.3 million and $22.0 million, respectively, related to this agreement.

Companion Diagnostics

Roche

In December 2012, Eisai and the Company entered into an agreement with Roche Molecular Systems, Inc. (Roche) under which Eisai and the Company are funding Roche s development of a companion diagnostic to identify patients who possess certain point mutations in EZH2. The development costs under the agreement with Roche are the responsibility of Eisai until such time, if any, as the Company exercises its opt-in right under the collaboration agreement with Eisai. Under the terms of the agreement, Eisai has agreed to pay Roche defined milestone payments of up to $21.0 million to develop and make commercially available the companion diagnostic. As a result, the cost of the companion diagnostic agreement, prior to the Company s potential future exercise of its opt-in right under the Eisai collaboration, will not be reflected in the Company s

Table of Contents

consolidated statements of operations and comprehensive (loss) income. If the Company exercises its opt-in right to co-develop, co-commercialize and share profits in the United States as to EPZ-6438, Eisai will be entitled to offset up to 25.0% of the funding amount it has previously paid to Roche against future milestone payments and royalties that Eisai may be obligated to pay to the Company under the Eisai collaboration and license agreement, and the Company will become obligated to fund up to half of the defined milestones that remain payable to Roche as of the time the Company opts-in.

Abbott

In February 2013, the Company entered into an agreement with Abbott Molecular Inc. (Abbott) under which the Company agreed to fund Abbott s development of a companion diagnostic to identify patients with the mixed lineage leukemia (MLL-r) genetic alteration targeted by the Company s EPZ-5676 product candidate. Under the terms of the agreement, the Company paid Abbott an upfront payment of $0.9 million upon the execution of the agreement, is obligated to make aggregate milestone-based development payments of up to $6.0 million and is obligated to reimburse Abbott for specified costs expected to be incurred in connection with Abbott conducting clinical trials to obtain the necessary regulatory approvals for the companion diagnostic (the reimbursable costs). The reimbursable costs are not to exceed $0.9 million unless any excess costs are agreed to in advance by both the Company and Abbott. In addition to the upfront payment, the Company expects to pay an aggregate of approximately $1.5 million in milestone-base development payments under this agreement during 2013.

8. Stock-Based Compensation

Total stock-based compensation expense related to stock options, restricted stock and the employee stock purchase plan was $0.9 million and $0.1 million for the three months ended September 30, 2013 and 2012, respectively, and $1.8 million and $0.2 million for the nine months ended September 30, 2013 and 2012, respectively.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
	(In thousands)		(In thousands)	
Research and development	$ 294	$ 55	$ 695	$ 104
General and administrative	575	37	1,124	106
Total	$ 869	$ 92	$ 1,819	$ 210

Stock Options

The weighted-average fair value of options, estimated as of the grant date using the Black-Scholes option pricing model, was $21.81 per option for those options granted during the three months ended September 30, 2013 and $7.57 and $1.70 per option for those options granted during the nine months ended September 30, 2013 and 2012, respectively. There were no stock options granted during the three months ended September 31, 2012. Key assumptions used to apply this pricing model were as follows:

	Nine Months Ended September 30, 2013	Nine Months Ended September 30, 2012
Risk-free interest rate	0.9%	0.7%
Expected life of options	6.0 years	6.0 years
Expected volatility of underlying stock	94.7%	98.8%
Expected dividend yield	0.0%	0.0%

Table of Contents

The following is a summary of stock option activity for the nine months ended September 30, 2013:

	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, 2012	3,492,694	$ 0.90		
Granted	1,483,328	7.51		
Exercised	(177,212)	0.97		
Forfeited or expired	(25,780)	2.74		
Outstanding at September 30, 2013	4,773,030	$ 2.94	7.7	$ 176,875
Exercisable at September 30, 2013	2,557,179	$ 0.70	6.7	$ 100,489

As of September 30, 2013, there was $10.4 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 2.8 years.

Restricted Stock

The following is a summary of restricted stock activity for the nine months ended September 30, 2013:

	Number of Shares	Weighted Average Grant Date Fair Value per Share
Outstanding at December 31, 2012	22,223	$ 0.60
Vested	(12,501)	0.60
Outstanding at September 30, 2013	9,722	$ 0.60

As of September 30, 2013, there was an insignificant amount of unrecognized compensation cost related to restricted stock that is expected to vest.

9. (Loss) Earnings Per Share

Basic (loss) earnings per share is computed by dividing (loss) income allocable to common stockholders by the weighted average number of common shares outstanding. During periods of income, participating securities are

allocated a proportional share of income determined by dividing total weighted average participating securities by the sum of the total weighted average common shares and participating securities (the two-class method). The Company s restricted stock and, prior to its automatic conversion, redeemable convertible preferred stock participate in any dividends declared by the Company and are therefore considered to be participating securities. Participating securities have the effect of diluting both basic and diluted earnings per share during periods of income. During periods of loss, no loss is allocated to participating securities since they have no contractual obligation to share in the losses of the Company. Diluted (loss) earnings per share is computed after giving consideration to the dilutive effect of stock options that are outstanding during the period, except where such non-participating securities would be anti-dilutive.

Table of Contents

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
	(In thousands except per share data)			
Net (loss) income	$ (9,704)	$ 4,448	$ (19,413)	$ 3,836
Less: accretion of redeemable convertible preferred stock to redemption value		159	264	326
Less: income allocable to participating securities		3,972		3,239
(Loss) income allocable to common stockholders	$ (9,704)	$ 317	$ (19,677)	$ 271
Weighted average shares outstanding	28,406	1,651	13,212	1,637
Basic (loss) earnings per share allocable to common stockholders	$ (0.34)	$ 0.19	$ (1.49)	$ 0.17

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
	(In thousands except per share data)			
Net (loss) income	$ (9,704)	$ 4,448	$ (19,413)	$ 3,836
Less: accretion of redeemable convertible preferred stock to redemption value		159	264	326
Less: income allocable to participating securities		3,743		3,092
(Loss) income allocable to common stockholders	$ (9,704)	$ 546	$ (19,677)	$ 418
Weighted average shares outstanding	28,406	1,651	13,212	1,637
Effect of dilutive securities		1,366		1,004
Diluted weighted average shares outstanding	28,406	3,017	13,212	2,641
Diluted (loss) earnings per share allocable to common stockholders	$ (0.34)	$ 0.18	$ (1.49)	$ 0.16

In June 2013, the Company issued an additional 5,913,300 shares of common stock in connection with its IPO and 20,633,046 shares of common stock in connection with the automatic conversion of its redeemable convertible preferred stock upon the closing of the IPO. The issuance of these shares resulted in a significant increase in the Company s shares outstanding, to 28,408,698 shares as of September 30, 2013, and weighted average shares outstanding for the three and nine months ended September 30, 2013 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 2014.

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 September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
	(In thousands)			
Stock options	4,773		4,773	
Unvested restricted stock	10		10	
Shares issuable under employee stock purchase plan	2		2	
	4,785		4,785	

Table of Contents

10. Related Party Transactions

In connection with its entry into the collaboration and license agreement with Celgene, on April 2, 2012, the Company sold Celgene 9,803,922 shares of its series C redeemable convertible preferred stock. As a result of this transaction, Celgene owned 12.5% of the Company s fully diluted equity as of December 31, 2012. Refer to Note 6, *Collaborations*, for additional information regarding this collaboration agreement. In the second quarter of 2013, in connection with the IPO, Celgene made an additional investment in the Company, acquiring an additional 66,666 shares of the Company s common stock. Additionally, as a result of the IPO, Celgene s shares of series C redeemable convertible preferred stock automatically converted to common stock of the Company at a one-for-three ratio, collectively resulting in Celgene owning 3,334,640 shares of the Company s common stock as of September 30, 2013, representing 10.0% of the Company s fully diluted equity and 11.7% of the voting interests of the Company as of September 30, 2013.

Under the Celgene collaboration agreement, the Company recognized $3.5 million and $10.8 million of collaboration revenue in the three and nine months ended September 30, 2013, respectively, and $6.7 million and $20.3 million of collaboration revenue in the three and nine months ended September 30, 2012, respectively. Additionally, in the three and nine months ended September 30, 2013, the Company recorded $0.7 million in co-funded accounts receivable from Celgene net in research and development expense. Accordingly, as of September 30, 2013 and December 31, 2012, the Company had recorded $33.3 million and $44.1 million of deferred revenue, respectively, and, as of September 30, 2013, the Company had accounts receivable of $0.7 million related to this collaboration arrangement.

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 personalized therapeutics for patients with genetically defined cancers;*
- *our ongoing and planned clinical trials, including the timing of anticipated results;*
- *our ability to receive research 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 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 innovative personalized therapeutics for patients with genetically defined cancers. 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. Genetic alterations can result in changes to the activity of HMTs, making them oncogenic. Our therapeutic strategy is to inhibit oncogenic HMTs to treat the underlying causes of the associated genetically defined cancers. The three months ended September 30, 2013 and 2012 are referred to as the third quarter of 2013 and 2012, respectively. Unless the context indicates otherwise, all references herein to our company include our wholly-owned subsidiary.

Our management s discussion and analysis of our financial condition and results of operations are based upon our unaudited 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. This discussion and analysis should

Table of Contents

be read in conjunction with these unaudited consolidated financial statements and the notes thereto as well as in conjunction with our Prospectus filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, or the Securities Act, with the Securities and Exchange Commission, or SEC, on May 31, 2013, which we refer to as the Prospectus.

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 September 30, 2013, we have raised an aggregate of $291.8 million to fund our operations, of which $133.3 million was non-equity funding through our collaboration agreements, $82.5 million was from our initial public offering, or IPO, which we completed in June 2013, and $76.0 million was from the sale of redeemable convertible preferred stock, which automatically converted to common stock upon the closing of our initial public offering. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity or debt financings and collaboration agreements.

We are a leader in the translation of the science of epigenetics into first-in-class personalized therapeutics for patients with genetically defined cancers and believe we are the first company to conduct a clinical trial of an HMT inhibitor. We are conducting both a Phase I clinical trial of our most advanced product candidate, EPZ-5676, an inhibitor targeting the DOT1L HMT, for the treatment of mixed lineage leukemia, or MLL-r, a genetically defined subtype of the two most common forms of acute leukemia, as well as a Phase I/II clinical trial of our second most advanced product candidate, EPZ-6438, an inhibitor targeting the EZH2 HMT, for the treatment of a genetically defined subtype of non-Hodgkin lymphoma. In 2014, we plan to pursue additional clinical studies for both EPZ-5676 and EPZ-6438 beyond the primary indications, including an expansion cohort for adult acute myeloid leukemia with a partial tandem duplication in the MLL gene, or MLL-PTD, for EPZ-5676, and an expansion cohort in synovial sarcoma and other INI1-deficient tumors for EPZ-6438. We also have a pipeline of other HMT inhibitors that are in preclinical development.

The clinical development plan for each of our therapeutic product candidates is directed towards patients with a particular genetically defined cancer. For each therapeutic product candidate, we intend to develop a companion diagnostic. We plan to include patients with the particular genetically defined cancer in our clinical trials beginning in Phase I with a view to assessing possible early evidence of potential therapeutic effect. As we are tailoring our personalized therapeutics for discrete patient populations with genetically defined cancers, we believe that many of our products may qualify for orphan drug designation in the United States and the European Union.

We have entered into strategic collaborations for certain of our therapeutic programs and corresponding companion diagnostics. Our three primary collaboration partners for our therapeutic programs are Celgene Corporation and Celgene International Sàrl, collectively, Celgene; Eisai Co., Ltd., or Eisai; and Glaxo Group Limited, an affiliate of GlaxoSmithKline, or GSK. We retain all product rights in the United States under the Celgene collaboration and an opt-in right to co-develop, co-commercialize and share profits as to licensed products in the United States under the Eisai collaboration.

Table of Contents

The following table summarizes key information about our two most advanced clinical programs, including the role of our collaboration partners:

Product Candidate	Description	Indication (Genetic Alteration)	Stage of Development	Commercial Rights	Diagnostic Collaborator
EPZ-5676	DOT1L inhibitor	MLL-r subtype of acute myeloid leukemia, or AML, and acute lymphoblastic leukemia, or ALL (Chromosomal translocation involving the MLL gene)	Phase I clinical trial ongoing	Epizyme: United States Celgene: Rest of world	Abbott Molecular Inc., or Abbott
EPZ-6438	EZH2 inhibitor	Non-Hodgkin lymphoma and potentially other solid tumors (Point mutation in EZH2)	Phase I/II clinical trial ongoing	Eisai: Worldwide rights, subject to Epizyme s opt-in on 50.0% of United States rights	Roche Molecular Systems, Inc., or Roche

Program highlights for the nine months ended September 30, 2013 include:

For EPZ-5676, we are nearing completion of the dose escalation stage of our Phase I clinical trial without any dose-limiting toxicities to date and plan to disclose top-line dose escalation data in the fourth quarter of 2013. Based on the data from the dose escalation stage, we plan to initiate an expansion cohort stage of this trial in the fourth quarter of 2013 that will be limited to patients with MLL-r and is expected to provide an initial assessment of therapeutic effect in MLL-r patients. We added five clinical sites, bringing the total number of clinical sites participating in this study to six, were granted orphan drug designation in the United States and were issued a notice of allowance from the United States Patent and Trademark Office with respect to our patent application covering the composition of matter of EPZ-5676. Further, we initiated plans to expand our clinical evaluation of EPZ-5676 in 2014 to include a Phase I trial of EPZ-5676 in pediatric patients with MLL-r and a clinical study in adult AML patients with MLL-PTD. MLL-PTD accounts for an estimated 5 to 7% of adult AML cases, with an estimated annual incidence of MLL-PTD in all patients in the major pharmaceutical markets of approximately 2,300 patients.

For EPZ-6438 (which Eisai refers to as E7438), we received notification that our clinical trial application was approved in France and are enrolling patients in a Phase I/II clinical trial without any dose-limiting toxicities observed to date. Based on the data from the dose escalation phase, we plan to initiate the Phase II portion of this study in 2014. Further, we initiated plans to expand our clinical evaluation of EPZ-6438 after the completion of the ongoing Phase I study in 2014 to include studies in patients with INI1-deficient tumors, such as synovial sarcoma and malignant rhabdoid tumors. In the major pharmaceutical markets,

synovial sarcoma has an estimated annual incidence of approximately 1,700 patients, and other INI1-deficient tumors have an estimated annual incidence of 700 patients.

For our discovery and preclinical stage product programs, we continued to progress the three target programs partnered with GSK as well as a number of other research programs directed to high priority HMTs in our pipeline.

Collaborations

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

Celgene

In April 2012, we entered into a collaboration and license agreement with Celgene, to discover, develop and commercialize, in all countries other than the United States, small molecule HMT inhibitors targeting DOT1L, including EPZ-5676, and any other HMT targets from our product platform for patients with genetically defined cancers, excluding targets already selected by our two other existing collaborations, which we refer to as the available targets.

Table of Contents

Agreement Structure

Under the terms of the 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 are eligible to earn up to $60.0 million in clinical development milestone payments and up to $100.0 million in regulatory milestone payments related to DOT1L. We are also eligible to earn up to $65.0 million in payments, including a combination of clinical development milestone payments and an option exercise fee, and up to $100.0 million in regulatory milestone payments for each available target as to which Celgene exercises its option during an initial option period ending in July 2015. Celgene has the right to extend the option period until July 2016 by making a significant option extension payment. As to DOT1L and each available target as to which Celgene exercises its option, we retain all product rights in the United States and are 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 reductions in specified circumstances. Due to the uncertainty of pharmaceutical development and the high historical failure rates generally associated with pharmaceutical development, we may not receive any milestone or royalty payments from Celgene. The first potential milestone payment that we might be entitled to receive under this agreement is $25.0 million for achieving proof-of-concept, as defined in the agreement, for our DOT1L inhibitor.

We are obligated to conduct and solely fund research and development costs of the Phase I clinical trials for the DOT1L target and through the effectiveness of the first investigational new drug application for an HMT inhibitor directed to each available target selected by Celgene, after which Celgene and we will equally co-fund global development and each party will solely fund territory-specific development costs for its territory. In the third quarter of 2013, we recorded accounts receivable of $0.7 million related to non-Phase I global development costs subject to the co-funding provisions of this agreement. Co-funded amounts receivable from Celgene are recorded net in research and development expense.

Collaboration Revenue

Through September 30, 2013, in addition to amounts allocated to Celgene s purchase of shares of our series C redeemable convertible preferred stock, we had received a total of $68.0 million in upfront payments under the Celgene agreement, including a $3.0 million implied premium on Celgene s purchase of our series C redeemable convertible preferred stock. Through September 30, 2013, we have recognized $34.7 million of collaboration revenue in the consolidated statements of operations and comprehensive (loss) income related to this agreement, including $3.5 million and $10.8 million in the three and nine months ended September 30, 2013, respectively, and $6.7 million and $20.3 million in the three and nine months ended September 30, 2012, respectively. As of September 30, 2013 and December 31, 2012, we had deferred revenue of $33.3 million and $44.1 million, respectively, related to this agreement.

Eisai

In April 2011, we entered into a collaboration and license agreement with Eisai under which we granted Eisai an exclusive worldwide license to our small molecule HMT inhibitors directed to EZH2, including EPZ-6438, while retaining an opt-in right to co-develop, co-commercialize and share profits with Eisai as to licensed products in the United States. Additionally, as part of the research collaboration, we agreed to provide research and development services related to the licensed compounds through December 31, 2014.

Agreement Structure

Under the terms of the agreement, we have recorded a $3.0 million upfront payment, $7.0 million in preclinical research and development milestone payments, a $6.0 million clinical development milestone payment and cash payments and accounts receivable totaling $16.5 million for research and development services through September 30, 2013. We are eligible to earn up to $25.0 million in additional clinical development milestone payments, up to $55.0 million in regulatory milestone payments and up to $115.0 million in sales-based milestone payments. We are also eligible to receive royalties at a percentage in the mid-single digits on any net product sales outside of the United States and at a percentage from the mid-single digits to low double-digits on any net product sales in the United States, subject to reduction in specified circumstances. Due to the uncertainty of pharmaceutical development and the high historical failure rates generally associated with pharmaceutical development, we may not receive any additional milestone payments or royalty or profit share payments from Eisai. The next potential milestone payment that we might be entitled to receive under this agreement is $10.0 million for the initiation of the Phase II portion the Phase I/II clinical trial.

Table of Contents

Eisai solely funds all research, development and commercialization costs for licensed compounds, except for the cost obligations that we will undertake if we exercise our opt-in right to co-develop, co-commercialize and share profits with Eisai as to licensed products in the United States. If we exercise our opt-in right as to a licensed compound, the licensed compound would become a shared product as to which Eisai s obligation to pay royalties to us as to such shared product in the United States will terminate; Eisai and we will share in net profits or losses with respect to such shared product in the United States; 25.0% of specified past development costs will become creditable by Eisai against future milestones or royalties due to us, subject to specified limitations; Eisai and we will share equally in subsequent development costs allocated to the United States; and all subsequent milestone payments that become payable by Eisai to us based on the shared product will be decreased by 50.0%.

Collaboration Revenue

Through September 30, 2013, we have recorded a total of $32.5 million in cash and accounts receivable and have recognized $30.5 million of collaboration revenue in the consolidated statements of operations and comprehensive (loss) income related to this agreement, including $1.9 million and $12.4 million in the three and nine months ended September 30, 2013, respectively, and $1.9 million and $9.3 million in the three and nine months ended September 30, 2012, respectively, with a $6.0 million clinical development milestone achieved recognized as collaboration revenue in the nine months ended September 30, 2013 and a $4.0 million research milestone achieved and recognized as collaboration revenue in the nine months ended September 30, 2012. As of September 30, 2013 and December 31, 2012, we had deferred revenue of $2.0 million and $3.2 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, 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 ends January 8, 2015.

Agreement Structure

Under the agreement, we have received a $20.0 million upfront payment, $8.0 million in preclinical research and development milestone payments and $6.0 million of fixed research funding. We are eligible to receive up to $21.0 million in additional preclinical research and development milestone payments, up to $99.0 million in clinical development milestone payments, up to $240.0 million in regulatory milestone payments and up to $270.0 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 pharmaceutical development, we may not receive any additional milestone payments or royalty payments from GSK. The next potential milestone payment that we might be entitled to receive under this agreement is a research milestone. However, due to the varying stages of development of each licensed target, we are not able to determine the next milestone that might be achieved, if any.

For each selected target in the collaboration, we are primarily responsible for research until the selection of the development candidate, and GSK will be solely responsible for subsequent development and commercialization. GSK provided a fixed amount of research funding during the second and third years of the research term and is obligated to

provide research funding equal to 100.0% of research and development costs, subject to specified limitations, for any research activities we conduct in the fourth year of the research term.

Table of Contents

Collaboration Revenue

Through September 30, 2013, we received a total of $34.0 million in payments under the GSK agreement and have recognized $18.7 million of collaboration revenue in the consolidated statements of operations and comprehensive (loss) income related to this agreement, including $3.0 million and $9.0 million in the three and nine months ended September 30, 2013, respectively, and $6.7 million in both the three and nine months ended September 30, 2012, with $4.0 million in preclinical research and development milestones achieved and recognized as collaboration revenue in the three and nine months ended September 30, 2012. As of September 30, 2013 and December 31, 2012, we had deferred revenue of $15.3 million and $22.0 million, respectively, related to this agreement.

Results of Operations

Collaboration Revenue

The following is a comparison of collaboration revenue for the three and nine months ended September 30, 2013 and 2012:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2013	2012	Decrease	2013	2012	Decrease
	(In millions)			(In millions)		
Collaboration revenue	$ 8.4	$ 15.3	$ (6.9)	$ 32.2	$ 36.3	$ (4.1)

Through September 30, 2013, our revenue consisted 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 funding and milestone payments earned under collaboration and license agreements with our collaboration partners.

During the third quarter of 2013, collaboration revenue consisted of $6.3 million recognized from deferred revenue related to upfront payments for licenses and $2.1 million in research and development funding. This revenue compares to $9.3 million recognized from deferred revenue related to upfront payments for licenses, $4.0 million in milestone revenue and $2.0 million in research and development funding recognized in the third quarter of 2012.

Collaboration revenue recognized from deferred revenue in the third quarter of 2013 comprised $3.5 million under our Celgene agreement, $0.4 million under our Eisai agreement and $2.4 million under our GSK agreement, as compared to $6.7 million under our Celgene agreement, $0.4 million under our Eisai agreement and $2.2 million under our GSK agreement in the third quarter of 2012. Milestone revenue in the third quarter of 2012 represents $4.0 million in preclinical research and development milestone achieved under our GSK agreement. Collaboration revenue recognized for research and development services in the third quarter of 2013 comprised $1.5 million under our Eisai agreement and $0.6 million under our GSK agreement, as compared to $1.5 million under our Eisai agreement and $0.5 million under our GSK agreement in the third quarter of 2012.

During the nine months ended September 30, 2013, collaboration revenue consisted of $19.2 million recognized from deferred revenue related to upfront payments for licenses, $6.0 million in milestone revenue and $7.0 million in research and development funding. This revenue compares to $23.7 million recognized from deferred revenue related to upfront payments for licenses, $8.0 million in milestone revenue and $4.6 million in research and development funding recognized in the nine months ended September 30, 2012.

Collaboration revenue recognized from deferred revenue in the nine months ended September 30, 2013 comprised $10.8 million under our Celgene agreement, $1.2 million under our Eisai agreement and $7.2 million under our GSK agreement, as compared to $20.3 million under our Celgene agreement, $1.2 million under our Eisai agreement and $2.2 million under our GSK agreement in the same period of the prior year. Milestone revenue in the nine months ended September 30, 2013 represents a $6.0 million clinical development milestone achieved under our Eisai agreement, as compared to a $4.0 million preclinical research and development milestone achieved under Eisai agreement and $4.0 million in preclinical research and development milestones achieved under our GSK agreement in the same period of the prior year. Collaboration revenue recognized for research and development services in the nine months ended September 30, 2013 comprised $5.2 million under our Eisai agreement and $1.8 million under our GSK agreement, as compared to $4.1 million under our Eisai agreement and $0.5 million under our GSK agreement in the same period of the prior year.

Table of Contents

Research and Development

The following is a comparison of research and development expenses for the three and nine months ended September 30, 2013 and 2012:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2013	2012	Increase	2013	2012	Increase
	(In millions)			(In millions)		
Research and development	$ 14.6	$ 9.3	$ 5.3	$ 41.9	$ 27.4	$ 14.5

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 clinical research organizations, or CROs, and other outside expenses. 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.

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