Epizyme, Inc.
Form 10-Q
May 08, 2018

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended March 31, 2018

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 No

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

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

Large accelerated filer		Accelerated filer
Non-accelerated filer	(Do not check if a smaller reporting company)	Smaller reporting company
		Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act).

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

The number of shares outstanding of the registrant's common stock as of May 1, 2018: 69,487,808 shares.

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,” “c
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 patients with cancer and other serious diseases;
- our ongoing and planned clinical trials, including the timing of initiation and enrollment in the trials, the timing of availability of data from the trials and the anticipated results of the trials;
- our ability to achieve anticipated milestones under our collaborations;
- the timing of and our ability to apply for, 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; and
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing.

All of our forward-looking statements are made 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.

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, 2017, or our Annual Report. The three months ended March 31, 2018 and 2017 are referred to as the first quarter of 2018 and 2017, respectively. Unless the context indicates otherwise, all references herein to our company include our wholly owned subsidiary.

PART I – FINANCIAL INFORMATION

Item 1. Financial Statements
EPIZYME, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS (UNAUDITED)

(Amounts in thousands except per share data)

	March 31, 2018	December 31, 2017
ASSETS		
Current assets:		
Cash and cash equivalents	$130,289	$ 226,664
Marketable securities	117,634	49,775
Accounts receivable	24	382
Prepaid expenses and other current assets	8,915	8,983
Total current assets	256,862	285,804
Property and equipment, net	2,275	2,527
Restricted cash and other assets	1,064	1,028
Total assets	$260,201	$ 289,359
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$7,227	$ 7,001
Accrued expenses	17,402	17,549
Current portion of capital lease obligation	—	110
Other current liabilities	4	4
Total current liabilities	24,633	24,664
Deferred revenue	3,806	28,809
Other long-term liabilities	630	515
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.0001 par value; 5,000 shares authorized; no shares issued and outstanding	—	—
Common stock, $0.0001 par value; 125,000 shares authorized; 69,486 shares and 69,302 shares issued and outstanding, respectively	7	7
Additional paid-in capital	728,356	723,510
Accumulated other comprehensive loss	(72)	(49)
Accumulated deficit	(497,159)	(488,097)
Total stockholders' equity	231,132	235,371
Total liabilities and stockholders' equity	$260,201	$ 289,359

See notes to condensed consolidated financial statements.

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EPIZYME, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(UNAUDITED)

(Amounts in thousands except per share data)

	Three Months Ended March 31,	
	2018	2017
Collaboration revenue	$—	$—
Operating expenses:		
Research and development	25,622	24,695
General and administrative	9,360	8,269
Total operating expenses	34,982	32,964
Operating loss	(34,982)	(32,964)
Other income, net:		
Interest income, net	899	438
Other income	18	4
Other income, net	917	442
Net loss	$(34,065)	$(32,522)
Other comprehensive income (loss):		
Unrealized (loss) gain on available-for-sale securities	(23)	12
Comprehensive loss	$(34,088)	$(32,510)
Loss per share allocable to common stockholders:		
Basic	$(0.49)	$(0.56)
Diluted	$(0.49)	$(0.56)
Weighted average shares outstanding:		
Basic	69,386	58,219
Diluted	69,386	58,219

See notes to condensed consolidated financial statements.

EPIZYME, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS (UNAUDITED)

(Amounts in thousands)

	Three Months Ended,	
	March 31, 2018	2017 (as revised)*
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$(34,065)	$(32,522)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	361	411
Stock-based compensation	2,887	2,757
Amortization of discount on investments	(106)	(18)
Changes in operating assets and liabilities:		
Accounts receivable	358	15
Prepaid expenses and other current assets	68	(2,230)
Accounts payable	226	1,913
Accrued expenses	(147)	(3,577)
Other assets	(36)	(4)
Other liabilities	113	20
Net cash used in operating activities	(30,341)	(33,235)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of available-for-sale securities	(100,763)	(40,785)
Maturities of available-for-sale securities	32,988	58,200
Purchases of property and equipment	(108)	(118)
Net cash (used in) provided by investing activities	(67,883)	17,297
CASH FLOWS FROM FINANCING ACTIVITIES:		
Payments under capital lease obligation	(110)	(150)
Proceeds from public offering, net of commissions	—	1,587
Proceeds from stock options exercised	1,500	641
Issuance of shares under employee stock purchase plan	459	346
Net cash provided by financing activities	1,849	2,424
Net (decrease) in cash, cash equivalents and restricted cash	(96,375)	(13,514)
Cash, cash equivalents and restricted cash, beginning of period	227,126	78,357
Cash, cash equivalents and restricted cash, end of period	$130,751	$64,843
SUPPLEMENTAL CASH FLOW INFORMATION:		
Purchases of property and equipment unpaid at period end	$44	$438
Cumulative catch up related to the adoption of ASU 2016-09	$—	$115

* Revised as a result of the adoption of ASU 2016-18

See notes to condensed consolidated financial statements.

EPIZYME, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (UNAUDITED)

1. Overview

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 is committed to rewriting treatment for cancer and other serious diseases through discovering, developing, and commercializing novel epigenetic medicines. By focusing on the genetic drivers of disease, the Company's science seeks to match targeted medicines with the patients who need them. The Company is broadly developing its lead product candidate, tazemetostat, an oral, first-in-class selective inhibitor of the EZH2 histone methyltransferase, or HMT, in a range of cancer types and settings, and developing the lead development candidate in the Company's novel G9a program, EZM8266, for the treatment of sickle cell disease, or SCD.

Through March 31, 2018, the Company has raised, including amounts received under collaboration agreements, an aggregate of $891.6 million to fund its operations, of which $217.8 million was non-equity funding through its collaboration agreements, $597.8 million was from the sale of common stock in the Company's public offerings and $76.0 million was from the sale of redeemable convertible preferred stock in private financings prior to the Company's initial public offering in May 2013. As of March 31, 2018, the Company had $247.9 million in cash, cash equivalents and marketable securities.

The Company commenced active operations in early 2008. Since its inception, the Company has generated an accumulated deficit of $497.2 million through March 31, 2018, and will require substantial additional capital to fund its research and development. The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, risks of failure of clinical trials and preclinical studies, the need to obtain additional financing to fund the future development and commercialization of tazemetostat and the rest of its pipeline, the need to obtain marketing approval for its product candidates, the need to successfully commercialize and gain market acceptance of its product candidates, dependence on key personnel, protection of proprietary technology, compliance with government regulations, development by competitors of technological innovations and ability to transition from clinical-stage manufacturing to commercial-stage production of products.

2. Summary of Significant Accounting Policies

Basis of Presentation

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, or the 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, 2017, or the Annual Report.

The unaudited condensed consolidated financial statements include the accounts of Epizyme, Inc. and its wholly owned, controlled subsidiary, Epizyme Securities Corporation. 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 condensed consolidated financial statements are issued to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. The three months ended March 31, 2018 and 2017

are referred to as the first quarter of 2018 and 2017, 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.

Significant Accounting Policies

During the quarter ended March 31, 2018, the Company adopted Accounting Standards Codification, or ASC, Topic 606, Revenue from Contracts with Customers, or ASC 606, using the modified retrospective transition method, resulting in a significant change in the Company's revenue recognition policy, as referenced below in this Note 2 under Recently Adopted Accounting Pronouncements. In addition, the Company adopted ASC 2016-18, Restricted Cash, resulting in a change to the beginning and ending cash balances for the periods presented in the condensed consolidated statements of cash flows, also referenced below in this Note 2. There have been no other material changes to the Company's significant accounting policies during the three months ended March 31, 2018, as compared to the significant accounting policies disclosed in Note 2, Summary of Significant Accounting Policies, of the Company's financial statements included in the Annual Report.

Going Concern

At each reporting period, the Company evaluates whether there are conditions or events that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the financial statements are issued. The Company is required to make certain additional disclosures if it concludes substantial doubt exists and it is not alleviated by the Company's plans or when its plans alleviate substantial doubt about the Company's ability to continue as a going concern.

The Company's evaluation entails analyzing prospective operating budgets and forecasts for expectations of the Company's cash needs, and comparing those needs to the current cash, cash equivalent and marketable security balances. After considering the Company's current research and development plans and the timing expectations related to the progress of its programs, and after considering its existing cash, cash equivalents and marketable securities as of March 31, 2018, the Company did not identify conditions or events that raise substantial doubt about the Company's ability to continue as a going concern within one year from the date these financial statements were issued.

Revenue Recognition

Effective January 1, 2018, the Company adopted Accounting Standards Codification, or ASC, Topic 606, Revenue from Contracts with Customers, or ASC 606, using the modified retrospective transition method. Under this method, results for reporting periods beginning after January 1, 2018 are presented pursuant to ASC 606, while prior period amounts are not adjusted and continue to be reported in accordance with ASC 605. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations, and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

The Company has entered into collaboration and license agreements, which are within the scope of ASC 606, to discover, develop, manufacture and commercialize product candidates. The terms of these agreements typically contain multiple promises or obligations, which may include: (i) licenses, or options to obtain licenses, to compounds directed to specific HMT targets (referred to as "exclusive licenses") and (ii) research and development activities to be performed on behalf of the collaboration partner related to the licensed HMT targets. Payments to the Company under these agreements may include non-refundable license fees, customer option exercise fees, payments for research activities, reimbursement of certain costs, payments based upon the achievement of certain milestones and royalties on any resulting net product sales.

The Company first evaluates license and/or collaboration arrangements to determine whether the arrangement (or part of the arrangement) represents a collaborative arrangement pursuant to ASC Topic 808, Collaborative Arrangements, based on the risks and rewards and activities of the parties pursuant to the contractual arrangement. The Company accounts for collaborative arrangements (or elements within the contract that are deemed part of a collaborative arrangement), which represent a collaborative relationship and not a customer relationship, outside the scope of ASC 606. The Company's collaborations primarily represent revenue arrangements. For the arrangements or arrangement components that are subject to revenue accounting guidance, in determining the appropriate amount of revenue to be recognized as it fulfills its obligations under each of its agreements, the Company performs the following steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. As part of the accounting for these arrangements, the Company must use significant judgment to determine: a) the number of performance obligations based on the determination under step (ii) above and whether those performance obligations are distinct from other performance obligations in the contract; b) the transaction price under step (iii) above; and c) the stand-alone selling price for each performance obligation identified in the contract for the allocation of transaction price in step (iv) above. The Company uses judgment to determine whether milestones or other variable consideration, except for royalties, should be included in the transaction price as described further below. The transaction price is allocated to each performance obligation on a relative stand-alone selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied. In determining the stand-alone selling price of a license to the Company's proprietary technology or a material right provided by a customer option, the Company considers market conditions as well as entity-specific factors, including those factors contemplated in negotiating the agreements as well as

internally developed estimates that include assumptions related to the market opportunity, estimated development costs, probability of success and the time needed to commercialize a product candidate pursuant to the license. In validating its estimated stand-alone selling price, the Company evaluates whether changes in the key assumptions used to determine its estimated stand-alone selling price will have a significant effect on the allocation of arrangement consideration between performance obligations.

Amounts received prior to revenue recognition are recorded as deferred revenue. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as current portion of deferred revenue in the accompanying consolidated balance sheets. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion. Amounts recognized as revenue, but not yet received or invoiced are generally recognized as contract assets.

Exclusive Licenses – If the license to the Company's intellectual property is determined to be distinct from the other promises or performance obligations identified in the arrangement, which generally include research and development services, the Company recognizes revenue from non-refundable, upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license. In assessing whether a license is distinct from the other promises, the Company considers relevant facts and circumstances of each arrangement, including the research and development capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can benefit from the license for its intended purpose without the receipt of the remaining promise, whether the value of the license is dependent on the unsatisfied promise, whether there are other vendors that could provide the remaining promise, and whether it is separately identifiable from the remaining promise. For licenses that are combined with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. The measure of progress, and thereby periods over which revenue should be recognized, are subject to estimates by management and may change over the course of the research and development and licensing agreement.

Research and Development Services – The promises under the Company's collaboration and license agreements generally include research and development services to be performed by the Company on behalf of the collaboration partner. For performance obligations that include research and development services, the Company generally recognizes revenue allocated to such performance obligations based on an appropriate measure of progress. The Company utilizes judgment to determine the appropriate method of measuring progress for purposes of recognizing revenue, which is generally an input measure such as costs incurred. The Company evaluates the measure of progress each reporting period as described under Exclusive Licenses above. Reimbursements from the partner that are the result of a collaborative relationship with the partner, instead of a customer relationship, such as co-development activities, are recorded as a reduction to research and development expense.

Customer Options – The Company's arrangements may provide a collaborator with the right to select a target for licensing either at the inception of the arrangement or within an initial pre-defined selection period, which may, in certain cases, include the right of the collaborator to extend the selection period. Under these agreements, fees may be due to the Company (i) at the inception of the arrangement as an upfront fee or payment, (ii) upon the exercise of an option to acquire a license or (iii) upon extending the selection period as an extension fee or payment. If an arrangement is determined to contain customer options that allow the customer to acquire additional goods or services, the goods and services underlying the customer options are not considered to be performance obligations at the outset

of the arrangement, as they are contingent upon option exercise. The Company evaluates the customer options for material rights, or options to acquire additional goods or services for free or at a discount. If the customer options are determined to represent a material right, the material right is recognized as a separate performance obligation at the inception of the arrangement. The Company allocates the transaction price to material rights based on the relative stand-alone selling price, which is determined based on the identified discount and the probability that the customer will exercise the option. Amounts allocated to a material right are not recognized as revenue until, at the earliest, the option is exercised or expires.

Milestone Payments – At the inception of each arrangement that includes development milestone payments, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. The Company evaluates factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the particular milestone in making this assessment. There is considerable judgment involved in determining whether it is probable that a significant revenue reversal would not occur. At the end of each subsequent reporting period, the Company reevaluates the probability of achievement of all milestones subject to constraint and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment. If a milestone or other variable consideration relates specifically to the Company's efforts to satisfy a single performance obligation or to a

specific outcome from satisfying the performance obligation, the Company generally allocates the milestone amount entirely to that performance obligation once it is probable that a significant revenue reversal would not occur.

Royalties – For arrangements that include sales-based royalties, including milestone payments based on a level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of its licensing arrangements.

For a complete discussion of accounting for collaboration revenues, see Note 8, Collaborations.

Pending Accounting Pronouncements

In February 2016, the Financial Accounting Standards Board, or FASB, issued Accounting Standards Update, or ASU, 2016-02, Leases (Topic 842), which requires lessees to recognize a right-of-use asset and lease liability for most lease arrangements. The new standard is effective for annual reporting periods beginning after December 15, 2018. Although early adoption is permitted, the Company does not plan to early adopt the new standard. The Company is currently evaluating the potential changes to the Company's future financial reporting and disclosures that may result from adopting this ASU, but expects that all of its lease commitments will be subject to the new standard.

Recently Adopted Accounting Pronouncements

Revenue Recognition

In May 2014, the FASB, issued ASU, 2014-09, Revenue From Contracts With Customers. ASU 2014-09 amends Accounting Standards Codification, or ASC, 605, Revenue Recognition ("ASC 605"), by outlining a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers. In addition, the FASB issued ASUs 2016-10 and 2016-12, which provide clarifying amendments to ASU 2014-09. ASU 2014-09 and its related amendments will be effective for the Company for interim and annual periods beginning after December 15, 2017. The new standards are codified under ASC 606, Revenue From Contracts with Customers ("ASC 606"). The Company adopted this new standard on January 1, 2018 using the modified retrospective approach. The Company has elected to use the following practical expedient that is permitted under the rules of the adoption, which has been applied consistently to all contracts: the Company has not retrospectively restated its contracts that have been amended at each amendment date as is generally required under ASC 606. Instead, upon adoption, an entity may reflect the aggregate effect of all modifications that occurred before the beginning of the earliest period presented when identifying the satisfied and unsatisfied performance obligations; determining the transaction price; and allocating the transaction price to the satisfied and unsatisfied performance obligations.

As a result of adopting ASC 606 on January 1, 2018, the Company recorded a cumulative-effect credit to opening accumulated deficit of $25.0 million as of January 1, 2018 and a corresponding decrease to deferred revenue, net of current portion. No revenue for the three months ended March 31, 2018 was recognized in accordance with ASC 606. There was no impact to revenue recognized for the three months ended March 31, 2018 as a result of the adoption of

ASC 606. Deferred revenue as of March 31, 2018 was $3.8 million under ASC 606, as compared to a balance of $28.9 million, which would have resulted under ASC 605.

The cumulative-effect change relates principally to the Company's treatment of option rights under its agreement with Celgene Corporation, or Celgene, and the identification of more performance obligations under ASC 606 in comparison with identified units of accounting under ASC 605. The adoption did not impact the previous accounting for the Company's agreements with Glaxo Group Limited, or GSK and Eisai Co. Ltd., or Eisai. Pursuant to ASC 605, the Company had deemed Celgene's options to license the three small molecule HMT inhibitors targeting three predefined targets, or the Option Targets, as non-substantive and therefore included the services that it would be required to perform upon option exercise as deliverables. ASC 606 provides that only options that are deemed to be material rights are a performance obligation and that any goods or services required upon exercise of the option be excluded from the evaluation of performance obligations until the option is exercised. As a result of this change to the guidance, (1) the pre-IND research services performed by the Company for each of the three Option Targets were deemed to be distinct performance obligations whereas each had previously been combined into one unit of accounting with the respective license that is subject to the exercise of the option and (2) a lesser amount of transaction price was allocated to the options. For further discussion of the change and the adoption of this standard, see Note 8, Collaborations.

Cash

As of January 1, 2018, the Company adopted ASU 2016-15, Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments. The new standard clarifies certain aspects of the statement of cash flows, including the classification of debt prepayment or debt extinguishment costs, settlement of zero-coupon debt instruments or other debt instruments with coupon

interest rates that are insignificant in relation to the effective interest rate of the borrowing, contingent consideration payments made after a business combination, proceeds from the settlement of insurance claims, proceeds from the settlement of corporate-owned life insurance policies, distributions received from equity method investees and beneficial interests in securitization transactions. The new standard also clarifies that an entity should determine each separately identifiable source or use within the cash receipts and cash payments on the basis of the nature of the underlying cash flows. In situations in which cash receipts and payments have aspects of more than one class of cash flows and cannot be separated by source or use, the appropriate classification should depend on the activity that is likely to be the predominant source or use of cash flows for the item. The adoption of this standard did not have a material impact on the Company's condensed consolidated statements of cash flows.

As of January 1, 2018, the Company adopted ASU 2016-18, Restricted Cash, or ASU 2016-18, which requires an entity to reconcile and explain the period-over-period change in total cash, cash equivalents and restricted cash within its statements of cash flows. The Company adopted the standard using the retrospective approach. The adoption of the standard did not have a material impact on the Company's condensed consolidated financial statements or disclosures; however, prior period restricted cash was added to beginning and ending cash and cash equivalents in the consolidated statements of cash flows to conform to the current presentation.

A reconciliation of cash, cash equivalents, and restricted cash reported within the condensed consolidated balance sheets that sum to the total of the same such amounts shown in the condensed consolidated statements of cash flows, is as follows:

	As of March 31,	
	2018	2017
	(In thousands)	
Cash and cash equivalents	$130,289	$64,381
Restricted cash, as part of other assets	462	462
Total cash, cash equivalents, and restricted cash shown in the consolidated statements of cash flows	$130,751	$64,843

The $0.5 million relates to a letter of credit as a security deposit for the office and laboratory lease at Technology Square in Cambridge, Massachusetts. The Company has recorded cash held to secure this letter of credit as restricted cash in restricted cash and other assets on the consolidated balance sheet. There were no other material changes to the Company's consolidated financial statements or disclosures.

Share-Based Payment

As of January 1, 2018, the Company adopted ASU 2017-09, Compensation-Stock Compensation (Topic 718): Scope of Modification Accounting, which clarifies when to account for a change to the terms or conditions of a share-based payment award as a modification. The new standard does not change the accounting for modifications but clarifies that modification accounting guidance should only be applied if the fair value, vesting conditions, or classification of the award changes as a result of the change in terms or conditions. The adoption of this standard did not materially impact the Company's stock-based compensation expense as no awards were modified during the three months ended March 31, 2018.

3. Marketable Securities

The following table summarizes the available-for-sale securities held at March 31, 2018 (in thousands):

Description	Amortized	Unrealized	Unrealized	Fair Value

	Cost	Gains	Losses	
Commercial paper	$ 57,949	$ —	$ (26)	$ 57,923
Corporate notes	59,757	—	(46)	59,711
U.S. government agency securities and U.S. Treasuries	—	—	—	—
Total	$ 117,706	$ —	$ (72)	$ 117,634

The following table summarizes the available-for-sale securities held at December 31, 2017 (in thousands):

Description	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Commercial paper	$ 16,964	$ —	$ (6)	$ 16,958
Corporate notes	31,610	—	(43)	31,567
U.S. government agency securities and U.S. Treasuries	1,250	—	—	1,250
Total	$ 49,824	$ —	$ (49)	$ 49,775

The amortized cost of available-for-sale securities is adjusted for amortization of premiums and accretion of discounts to maturity. At March 31, 2018, the balance in the Company's accumulated other comprehensive loss was composed solely of activity related to the Company's available-for-sale marketable securities. There were no realized gains or losses recognized on the sale or maturity of available-for-sale securities during the three months ended March 31, 2018, and as a result, the Company did not reclassify any amounts out of accumulated other comprehensive loss for the same period.

The aggregate fair value of available-for-sale securities held by the Company in an unrealized loss position for less than twelve months as of March 31, 2018 was $73.6 million, which consisted of 8 commercial paper securities and 14 corporate notes securities. The aggregate unrealized loss for those securities in an unrealized loss position for less than twelve months as of March 31, 2018 was less than $0.1 million. The aggregate fair value of available-for-sale securities held by the Company in an unrealized gain position for less than twelve months as of March 31, 2018 was $44.0 million, which consisted of 3 commercial paper securities and 7 corporate notes securities. The aggregate unrealized gain for those securities in an unrealized gain position for less than twelve months as of March 31, 2018 was less than $0.1 million.

The Company does not intend to sell and it is unlikely that the Company will be required to sell the above investments before recovery of their amortized cost bases, which may be maturity. The Company determined that there was no material change in the credit risk of any of its investments. As a result, the Company determined it did not hold any investments with any other-than-temporary impairment as of March 31, 2018. The weighted-average maturity of the Company's portfolio was approximately three months at March 31, 2018.

4. Fair Value Measurements

The Company's financial instruments as of March 31, 2018 and December 31, 2017 consisted primarily of cash and cash equivalents, marketable securities and accounts receivable and accounts payable. As of March 31, 2018 and December 31, 2017, the Company's financial assets recognized at fair value consisted of the following:

	Fair Value as of March 31, 2018			
	Total	Level 1	Level 2	Level 3
	(In thousands)			
Cash equivalents	$114,926	$64,853	$50,073	$ —
Marketable securities:				
Commercial paper	57,923	—	57,923	—
Corporate notes	59,711	—	59,711	—

U.S. government agency securities and treasuries	—	—	—	—
Total	$232,560	$64,853	$167,707	$ —

	Fair Value as of December 31, 2017			
	Total	Level 1	Level 2	Level 3
	(In thousands)			
Cash equivalents	$207,251	$207,251	$—	$ —
Marketable securities:				
Commercial paper	16,958	—	16,958	—
Corporate notes	31,567	—	31,567	—
U.S. government agency securities and treasuries	1,250	—	1,250	—
Total	$257,026	$207,251	$49,775	$ —

Cash equivalents and marketable securities have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, utilizing third-party pricing services or other market observable data.

The Company measures its cash equivalents at fair value on a recurring basis. The Company classifies the majority of its cash equivalents within Level 1 of the fair value hierarchy because they are valued using observable inputs that reflect quoted prices for identical assets in active markets. The Company measures its marketable securities at fair value on a recurring basis and classifies those instruments and some cash equivalents within Level 2 of the fair value hierarchy. The pricing services used by management utilize industry standard valuation models, including both income and market based approaches and observable market inputs to determine the fair value of marketable securities and those cash equivalents classified within Level 2 of the fair value hierarchy.

5. Supplemental Balance Sheet Information

Accrued expenses consisted of the following:

	March 31, 2018	December 31, 2017
	(In thousands)	
Employee compensation and benefits	$2,179	$4,628
Research and development expenses	12,792	11,658
Professional services and other	2,431	1,263
Accrued expenses	$17,402	$17,549

6. Income Taxes

The Company did not record a federal or state income tax provision or benefit for the three months ended March 31, 2018 and 2017 due to the expected and known loss before income taxes to be incurred, or incurred, as applicable, for the years ended December 31, 2018 and 2017, as well as the Company’s continued maintenance of a full valuation allowance against its net deferred tax assets, with the exception of the deferred tax asset related to alternative minimum tax credit.

In accordance with SAB 118, the Company's preliminary estimate of the effects of the Tax Cuts and Jobs Act, or the TCJA, including the remeasurement of deferred tax assets and liabilities, is subject to the finalization of management’s analysis related to certain matters, such as developing interpretations of the provisions of the TCJA and the filing of its tax returns. U.S. Treasury regulations, administrative interpretations or court decisions interpreting the TCJA may require further adjustments and changes in its estimates. The final determination of the effects of the TCJA will be completed as additional information becomes available, but no later than one year from the enactment of the TCJA. In all cases, the Company will continue to make and refine its calculations as additional analysis is completed. In addition, the Company's estimates may also be affected as it gains a more thorough understanding of the tax law. The Company notes that as of March 31, 2018, no adjustments were made to the provisional amounts.

7. Commitments and Contingencies

There have been no significant changes to the Company's commitments and contingencies in the three months ended March 31, 2018, as compared to those disclosed in Note 7, Commitments and Contingencies, included in its Annual Report.

8. Collaborations

Celgene

In April 2012, the Company entered into a collaboration and license agreement with Celgene. On July 8, 2015, the Company entered into an amendment and restatement of the collaboration and license agreement with Celgene.

Original 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 $7.0 million of global development co-funding through March 31, 2018. 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 to which Celgene had the right to exercise its option during an initial option period that would have ended in July 2015 but was extended pursuant to the amended and restated agreement as discussed below under "Amended and Restated Agreement Structure" (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 was 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 were to equally co-fund global development and each party was to solely fund territory-specific development costs for its territory.

Amended and Restated Agreement Structure

Under the amended and restated collaboration and license agreement:

- Celgene retained its exclusive license to small molecule HMT inhibitors targeting DOT1L, including pinometostat,
- Celgene's other option rights were narrowed to small molecule 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 were expanded to include the United States, with the exclusive license to HMT inhibitors targeting the third Option Target continuing to be for all countries other than the United States,
- Celgene's option period was extended for each of the Option Targets and Celgene's option is exercisable at the time of the Company's investigational new drug application, or 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 and restated agreement were 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 were 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 and restated 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 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. The Company is also 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. 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 Celgene. Due to the varying stages of development of each target, the Company is not able to determine the next milestone that might be earned, if any.

The amended and restated 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.

Accounting Considerations of the Amended and Restated Agreement

The Company assessed the amended arrangement in accordance with ASC 606 and concluded that the contract counterparty, Celgene, is a customer based on the arrangement structure, through the satisfaction of each target's performance obligations. As of the amendment, the Company identified the following performance obligations under the arrangement, whether satisfied or not:

- an exclusive license to small molecule HMT inhibitors targeting DOT1L, including pinometostat, combined with pre-IND research services for DOT1L;
- post-IND research and development services for DOT1L through a Phase 1 clinical trial;
- pre-IND research services for each Option Target; and
- material rights related to each of Celgene's options at the time of an IND filing to license HMT inhibitors targeting each Option Target.

The Company determined that the DOT1L license and pre-IND research and development activities for DOT1L were not distinct from one another, due to the limited economic benefit that Celgene would derive from the DOT1L license if it did not obtain the research services. After IND effectiveness, the Company concluded that the DOT1L license would be distinct apart from any remaining research and development services because Celgene, or other market participants, would have the ability to execute human clinical trials on the identified compound. Accordingly, the DOT1L license and pre-IND research services for DOT1L were accounted for as a combined performance obligation. The post-IND research and development services for DOT1L have been accounted for as a separate performance obligation.

The pre-IND research services for each Option Target were the only performance obligations not subject to the exercise of a customer option at the time of the amendment for each Option Target and therefore represent three separate performance obligations (one for each Option Target).

The Company evaluated the option rights at the time of an IND filing to determine whether they provide Celgene with material rights. The Company concluded that the options were issued at a discount, and therefore provide material rights. As such, the option rights at the time of an IND filing for each Option Target represent three separate performance obligations (one for each Option Target) as of the amendment of the arrangement. The license to each HMT inhibitor targeting each respective Option Target, the Company's research and development obligations through the completion of a Phase 1 clinical trial for each Option Target, and the option to maintain the license beyond the end of Phase 1 clinical development for each Option Target are all subject to Celgene's exercise of the option rights at the

time of an IND filing and, therefore, are not considered performance obligations as of the amendment.

Under the agreement, the Company determined that the total transaction price was $103.0 million as of the amendment of the arrangement, comprised the following:

- $68.0 million total upfront payment received under the original agreement, as described above;
- $25.0 million clinical development milestone payment for DOT1L; and
- $10.0 million upfront payment under the amended and restated agreement.

The option exercise fees of $75.0 million in the aggregate, for the options at the time of IND and completion of Phase 1, that may be received are excluded from the transaction price until each customer option is exercised. The future potential milestone payments were excluded from the transaction price, as all milestone amounts were fully constrained. The Company will reevaluate the transaction price at the end of each reporting period and as uncertain events are resolved or other changes in circumstances occur, and, if necessary, adjust its estimate of the transaction price.

The transaction price was allocated to the performance obligations based on the estimated stand-alone selling prices at the time of the amendment. For the DOT1L performance obligation that includes the license and pre-IND research services, the stand-alone selling price was determined considering the stage and status of the program and the technology involved and the level of development

expected, as well as the expected cost and margin for the research services. For the post-IND research and development services for DOT1L and the pre-IND research services for each Option Target, the stand-alone selling price was determined considering the expected cost and a reasonable margin for the respective services. The material rights from the option rights at the time of an IND filing for each Option Target were valued based on the estimated discount at which the option is priced and the Company's estimated probability of the options' exercise as of the time of the amendment. The Company believes that a change in the assumptions used to determine its stand-alone selling price for the performance obligations most likely would not have a significant effect on the allocation of consideration received (or receivable) to the performance obligations that were not satisfied as of the adoption of ASC 606.

The Company allocated the following amounts of the total transaction price to the performance obligations as of the amendment date:

- $65.1 million, including the $25.0 million clinical development milestone payment for DOT1L, to the two DOT1L performance obligations, which were satisfied prior to the ASC 606 adoption date;
- $34.1 million to the three Pre-IND research services performance obligations related to the Option Targets, which were substantially satisfied as of the ASC 606 adoption date; and
- $3.8 million to the three material rights related to Celgene's option rights at the time of an IND filing for each Option Target, which shall not be satisfied until the option is exercised or one of the parties opts out of the arrangement.

All performance obligations, except for the three material rights were substantially satisfied as of the adoption of ASC 606 and therefore all of the transaction price allocated to those performance obligations has been recognized as revenue under ASC 606. Through March 31, 2018, the Company had recognized revenue of $99.2 million under the agreement as collaboration revenue in the Company's consolidated statements of operations and comprehensive loss and in accumulated deficit as a result of the cumulative-effect recognition upon adoption of ASC 606. The amounts received that have not yet been recognized as revenue, related to the material rights, are recorded in deferred revenue on the Company's condensed consolidated balance sheet. Deferred revenue related to the agreement amounted to $3.8 million as of March 31, 2018, all of which is included in noncurrent liabilities.

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 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. Subsequent to a GSK strategic portfolio prioritization, the Company received notice in October 2017 that GSK terminated the agreement with respect to the third target, effective December 31, 2017, which returned all rights to that target to the Company. The two other targets continue to be subject to the agreement and were not impacted by the termination with respect 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 has received and recognized as collaboration revenue 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, $9.0 million for research and development services and $31.0 million of preclinical research and development milestone payments. The preclinical and research and development milestone payments total includes a $10.0 million milestone payment earned in May 2017 related to the second target in the collaboration, upon GSK's initiation of good laboratory practices toxicology studies, as well as a $6.0 million clinical milestone following GSK's initiation of patient dosing in a Phase 1 clinical trial of a PRMT5 inhibitor that the Company discovered and licensed to GSK. As of March 31, 2018, for the two remaining targets, the Company is eligible to receive up to $70.0 million in clinical development milestone payments, up to $197.0 million in regulatory milestone payments and up to $128.0 million in sales-based milestone payments. As a result of the termination of the agreement as it relates to the third target, the Company will receive no additional payments related to that target. 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. The Company determined the next milestone that might be achieved under this agreement is for $8.0 million due at the first dosing of a patient for an undisclosed target under a Phase 1 study. 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. 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 March 31, 2018, the Company has earned a total of $69.0 million under the GSK agreement, which the Company recognized as collaboration revenue in the condensed consolidated statements of operations and comprehensive loss, including $10.0 million of milestone revenue in the year ended December 31, 2017. The Company did not have any deferred revenue related to this agreement as of March 31, 2018 or December 31, 2017 and any future revenues will relate to any milestone payments and royalties received under the agreement with respect to the two remaining targets, if any.

The future potential milestone payments were excluded from the transaction price for the GSK agreement, as all future milestone amounts were fully constrained. The Company will reevaluate the likelihood of achieving future milestones at the end of each reporting period. The remaining future milestone payments are related to performance obligations that have been satisfied. Therefore, if the risk of significant reversal is resolved, any future milestone revenue from the arrangement will be recognized as revenue in the period the risk is relieved.

Eisai

In April 2011, the Company entered into a collaboration and license agreement with Eisai Co. Ltd, or 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.

As of December 31, 2014, the Company had completed its performance obligations under 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 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 and waived the right of first negotiation for the rest of Asia.

Under the original agreement, Eisai was solely responsible for funding all research, development and commercialization costs for EZH2 compounds. Under the amended and restated agreement, the Company is solely responsible for funding global development, manufacturing and commercialization costs for EZH2 compounds outside of Japan, including the remaining development costs due under a Roche Molecular 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 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, had not achieved regulatory approval for marketing and, absent obtaining such approval, had 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 consolidated statements of operations and comprehensive loss. The Company has also agreed to pay Eisai up to $20.0 million in clinical development milestone payments, including a $10.0 million milestone upon the earlier of initiation of a first phase 3 clinical trial of any EZH2 product or the first submission of an NDA or MAA, up to $50.0 million in regulatory milestone payments, including a $25.0 million milestone payment upon regulatory approval of the first NDA or MAA, 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.

Companion Diagnostics

Roche Molecular

In December 2012, Eisai and the Company entered into an agreement with Roche Molecular under which Eisai and the Company engaged Roche Molecular to develop a companion diagnostic to identify patients who possess certain activating mutations of EZH2. In October 2013, this agreement was amended to include additional mutations in EZH2. The development costs due under the amended agreement with Roche Molecular were the responsibility of Eisai until the execution of the amended and restated collaboration and license agreement with Eisai in March 2015, at which time the Company assumed responsibility for the remaining development costs due under the agreement. In December 2015, the Company entered into a second amendment to the companion diagnostic agreement with Roche Molecular. The agreement was further amended in March 2018. Before the additional amendment, the Company was responsible for the remaining development costs of $10.5 million due under the agreement. Under the amended agreement, the Company is responsible for remaining development costs of $10.4 million due under the agreement and Eisai has

agreed to reimburse the Company $0.9 million of this amount related to a regulatory milestone for Japan. The Company expects the remaining development costs under the amended agreement to be incurred and paid through 2019.

Under the agreement with Roche Molecular, Roche Molecular is obligated to use commercially reasonable efforts to develop and to make commercially available the companion diagnostic. Roche Molecular has exclusive rights to commercialize the companion diagnostic.

The agreement with Roche Molecular will expire when the Company is no longer developing or commercializing tazemetostat. The Company may terminate the agreement by giving Roche Molecular 90 days' written notice if the Company discontinues development and commercialization of tazemetostat or determines, in conjunction with Roche Molecular, that the companion diagnostic is not needed for use with tazemetostat. Either the Company or Roche Molecular may also terminate the agreement in the event of a material breach by the other party, in the event of material changes in circumstances that are contrary to key assumptions specified in the agreement or in the event of specified bankruptcy or similar circumstances. Under specified termination circumstances, Roche Molecular may become entitled to specified termination fees.

9. Stock-Based Compensation

Total stock-based compensation expense related to stock options, restricted stock units and shares issued under the employee stock purchase plan was $2.9 million and $2.8 million for the three months ended March 31, 2018 and 2017, respectively.

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

	Three Months Ended March 31,	
	2018	2017
	(In thousands)	
Research and development	$1,125	$1,392
General and administrative	1,762	1,365
Total	$2,887	$2,757

Stock Options

The weighted-average grant date fair value of options, estimated as of the grant date using the Black-Scholes option pricing model, was $10.39 and $8.20 per option for those options granted during the three months ended March 31, 2018 and 2017, respectively. Key assumptions used to apply this pricing model were as follows:

	Three Months Ended March 31

	2018	2017
Risk-free interest rate	2.5 %	1.8 %
Expected life of options	6.0 years	6.0 years
Expected volatility of underlying stock	71.4%	74.5 %
Expected dividend yield	0.0 %	0.0 %

The following is a summary of stock option activity for the three months ended March 31, 2018:

	Number of Options (In thousands)	Weighted Average Exercise Price per Share	Weighted Average Remaining Contractual Term (In years)	Aggregate Intrinsic Value (In thousands)
Outstanding at December 31, 2017	4,576	$ 14.57		
Granted	1,625	16.06		
Exercised	(153)	9.82		
Forfeited or expired	(334)	19.42		
Outstanding at March 31, 2018	5,714	$ 14.84	8.46	$ 22,348
Exercisable at March 31, 2018	1,761	$ 15.71	7.05	$ 8,488

As of March 31, 2018, there was $34.7 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 2.97 years.

Restricted Stock Units

As of March 31, 2018, there were no restricted stock units outstanding.

10. Loss Per Share

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

	Three Months Ended	
	March 31,	
	2018	2017
	(In thousands except per share data)	
Net loss	$(34,065)	$(32,522)
Weighted average shares outstanding	69,386	58,219
Basic and diluted loss per share allocable to common stockholders	$(0.49)	$(0.56)

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	
	March 31,	
	2018	2017
	(In thousands)	
Stock options	5,714	5,186
Unvested restricted stock units	—	57
Shares issuable under employee stock purchase plan	7	16
	5,721	5,259

11. Related Party Transactions

Celgene has made a series of equity investments in the Company, owning 3,674,640 shares of common stock representing 5.3% of the Company’s outstanding common stock as of March 31, 2018. Refer to Note 8, Collaborations, for additional information regarding the Company’s original agreement with Celgene entered into in April 2012 and the amended and restated agreement with Celgene entered into in July 2015.

The Company has received consulting and advisory services from a director. The Company paid this director approximately $190,000 and $0 for these services during the three months ended March 31, 2018 and March 31, 2017, respectively. Of these amounts, $72,000 and $48,000 of amounts due to the director were included in accrued expenses at March 31, 2018 and December 31, 2017, respectively.

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

Our management's discussion and analysis of our financial condition and results of operations are based upon our 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, or GAAP, and with Regulation S-X promulgated under the Securities Exchange Act of 1934, as amended. This discussion and analysis should be read in conjunction with these condensed consolidated financial statements and the notes thereto included elsewhere in this Quarterly Report on Form 10-Q. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in Part II, Item 1A. Risk Factors of this Quarterly Report on Form 10-Q, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage biopharmaceutical company that is committed to rewriting treatment for cancer and other serious diseases through discovering, developing, and commercializing novel epigenetic medicines. By focusing on the genetic drivers of disease, our science seeks to match targeted medicines with the patients who need them. We are broadly developing our lead product candidate, tazemetostat, an oral, first-in-class selective inhibitor of the EZH2 histone methyltransferase, or HMT, in a range of cancer types and settings, and developing the lead development candidate in our novel G9a program, EZM8266, for the treatment of sickle cell disease, or SCD.

We have taken a "pipeline in a product" approach to developing tazemetostat with a broad clinical development program through company-sponsored studies and collaborations that are evaluating tazemetostat as both a monotherapy and combination treatment in both hematological malignancies and solid tumors. Tazemetostat has shown meaningful clinical activity as a monotherapy in indications in both disease areas and has been generally well tolerated across clinical trials to date.

In April 2018, following a safety report of a pediatric patient who developed a secondary T-cell lymphoma in our ongoing Phase 1 clinical trial of tazemetostat in pediatric patients, the U.S. Food and Drug Administration, or FDA, issued a partial clinical hold on new enrollment of patients with genetically defined solid tumors and hematologic malignancies in our ongoing clinical trials of tazemetostat. In May 2018, the French National Agency for Medicines and Health Products Safety took a similar action. Under these regulatory actions, patients on study who have not experienced disease progression may be able to continue to receive tazemetostat. We are working with investigators and experts in the field to better understand this single case of secondary lymphoma in the context of the clinical activity and tolerability seen more broadly with tazemetostat in this and other studies in which more than 750 patients have been treated. We are also working diligently to address the hold on new patient enrollment, including updating the patient informed consent form, investigator's brochure and study protocols, and will need to confirm alignment with U.S. and French regulators in order to resume enrollment. In light of the partial clinical hold, we are assessing our timelines for completing patient enrollment in trials not fully enrolled at the time of the partial clinical hold, including our lymphoma trials of tazemetostat.

In our hematological program, we are conducting global Phase 2 studies evaluating tazemetostat's treatment potential in patients with relapsed or refractory follicular lymphoma, or FL, or diffuse large B-cell lymphoma, or DLBCL, the two most prevalent forms of non-Hodgkin lymphoma, or NHL, both with and without EZH2 activating mutations. Subject to the holds on enrollment in the United States and France, we are continuing to enroll FL and DLBCL patients with EZH2 mutations in our ongoing global Phase 2 monotherapy relapsed or refractory FL and DLBCL study. Based on initial engagement with the FDA, we believe we have the opportunity to submit for accelerated approval for tazemetostat as a monotherapy in FL, subject to the results of the FL cohorts of our Phase 2 study and additional regulatory engagement. We plan to continue to engage with the FDA in 2018 in regard to the partial clinical hold and to further refine our registration strategy for FL, and are assessing our timeline for submitting to the FDA a

New Drug Application, or NDA, for tazemetostat for FL. In addition, we also have multiple combination studies underway with both targeted agents and chemotherapy in DLBCL in both relapsed or refractory and first-line treatment settings, including a global Phase 1b trial combining tazemetostat with atezolizumab, a PD-L1 inhibitor, being conducted by Genentech, Inc., or Genentech, pursuant to a collaboration agreement. Enrollment was recently completed in both the Phase 1b atezolizumab combination and our prednisolone combination. We also plan to commence a combination study of tazemetostat in FL.

In our solid tumor program, we are evaluating tazemetostat's treatment potential in adults and children with molecularly defined solid tumors, including INI1- and SMARCA4-negative tumors, which we collectively refer to as INI1-negative tumors. We are conducting a global Phase 2 trial of tazemetostat in adults with INI1-negative tumors, including epithelioid sarcoma, malignant rhabdoid tumors, or MRT, other INI1-negative tumors, and chordoma. Based on positive data that we observed in the epithelioid sarcoma cohort in the ongoing study, we engaged with the FDA in May 2017. Based on this FDA interaction, we have identified a potential path to submission for accelerated approval in this indication and we are targeting submission of our first NDA to the FDA for tazemetostat

for epithelioid sarcoma in the fourth quarter of 2018. In connection with this submission, we plan to commence a clinical trial of tazemetostat for epithelioid sarcoma that could serve as the confirmatory trial required in connection with any accelerated approval.

We are also evaluating tazemetostat in the dose-expansion portion of a Phase 1 study in pediatric patients with INI1-negative tumors and in a Phase 2 clinical trial in pediatric patients with solid tumors and lymphoma under a Cooperative Research and Development Agreement, or CRADA, with the NCI.

In April 2018, we voluntarily paused enrollment worldwide in this Phase 1 pediatric trial following the receipt of the safety report regarding the patient who developed a secondary T-cell lymphoma. At the time of the safety report, the patient, who had advanced poorly differentiated chordoma, had been on study for approximately 15 months and had achieved a confirmed partial response. This patient has now discontinued tazemetostat and is being treated for T-cell lymphoma. The Pediatric MATCH trial is also subject to the partial clinical hold imposed by the FDA. We are assessing appropriate next steps for all pediatric studies.

In addition, we are evaluating tazemetostat in a Phase 2 study in adults with mesothelioma characterized by BAP1 loss-of-function. Data from this study are being presented at the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting.

Subject to the resolution of the holds on enrollment, Genentech has agreed that, as a part of its MORPHEUS study, it will evaluate tazemetostat in combination with atezolizumab (TECENTRIQ®), a PD-L1 inhibitor, in patients with non-small cell lung cancer. Subject to the resolution of the holds on enrollment, we also plan to evaluate tazemetostat in a Phase 2 clinical trial in adult patients with ovarian cancer under a Cooperative Research and Development Agreement, or CRADA, with the NCI.

We own the global development and commercialization rights to tazemetostat outside of Japan. Eisai Co. Ltd, or Eisai, holds the rights to develop and commercialize tazemetostat in Japan. Tazemetostat is covered by claims of U.S. and European composition of matter patents, which are expected to expire in 2032, exclusive of any extensions.

We have announced that the FDA has granted tazemetostat Fast Track designation in patients with relapsed or refractory FL, with or without activating EZH2 mutations, and relapsed or refractory DLBCL with EZH2 activating mutations. We have also announced that the FDA has granted tazemetostat orphan drug designation for the treatment of patients with FL, malignant rhabdoid tumors, or MRT, soft tissue sarcoma and mesothelioma. The orphan drug designation for the treatment of MRT applies to INI1-negative MRT as well as SMARCA4-negative malignant rhabdoid tumor of ovary, or MRTO. In addition, the European Commission has granted orphan drug designation to tazemetostat for the treatment of patients with FL, DLBCL and malignant mesothelioma. We also own global rights to EZM8266 targeting G9a.

We have collaboration agreements with Celgene Corporation and Celgene RIVOT Ltd., an affiliate of Celgene Corporation, which we collectively refer to as Celgene, Glaxo Group Limited (an affiliate of GlaxoSmithKline), or GSK, and Eisai. We also have a collaboration with Roche Molecular Systems, Inc., or Roche Molecular, to develop a companion diagnostic for use with tazemetostat to identify NHL patients with EZH2 activating mutations. These collaborations provide us with access to considerable scientific, development, regulatory and commercial capabilities. As of March 31, 2018, we had received $217.8 million in non-equity funding under these collaborations.

We have retained commercialization rights in the United States to all of our other programs, except two programs that are the subject of our collaboration with GSK and two of the preclinical programs that are the subject of our collaboration with Celgene. We plan to retain commercialization rights in the United States and possibly in select foreign jurisdictions in connection with any future collaborations. We intend to build a focused field presence and marketing capabilities to commercialize any of our product candidates that receive regulatory approval in the United States, as well as the capabilities to lead global commercial strategy. We have recently begun building the

infrastructure necessary to support the successful launch and marketing of tazemetostat and other product candidates that may receive marketing approval.

Through March 31, 2018, we have raised an aggregate of $891.6 million to fund our operations, of which $217.8 million was non-equity funding through our collaboration agreements, $597.8 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 March 31, 2018, we had $247.9 million in cash, cash equivalents and marketable securities.

We commenced active operations in early 2008, and since inception, have incurred significant operating losses. As of March 31, 2018, our accumulated deficit totaled $497.2 million. As a clinical stage company, we expect to continue to incur significant expenses and operating losses over the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. We expect our expenses to increase in connection with our ongoing activities, including our continued execution on our clinical development and commercialization plans for tazemetostat.

Collaborations

Refer to Note 8, Collaborations, of the notes to our condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for a description of the key terms of our arrangements with Celgene, GSK and Eisai.

As of March 31, 2018, we had recognized revenue of $99.2 million under the Celgene agreement as collaboration revenue in our condensed consolidated statements of operations and comprehensive loss and in accumulated deficit as a result of the cumulative-effect recognition upon adoption of Accounting Standards Codification Topic 606, Revenue from Contracts with Customers, or ASC 606. Deferred revenue related to the Celgene agreement amounted to $3.8 million as of March 31, 2018, all of which is included in noncurrent liabilities.

There have been no updates to the accounting for our GSK and Eisai collaboration agreements as a result of the adoption of ASC 606.

Results of Operations

Collaboration Revenue

The following is a comparison of collaboration revenue for the three months ended March 31, 2018 and 2017:

	Three Months Ended		
	March 31,		
	2018	2017	Change
	(In millions)		
Collaboration revenue	$ —	$ —	$ —

We did not recognize any collaboration revenue in the three months ended March 31, 2018 and 2017.

Research and Development

The following is a comparison of research and development expenses for the three months ended March 31, 2018 and 2017:

	Three Months Ended		
	March 31,		
	2018	2017	Change
	(In millions)		
Research and development	$25.6	$24.7	$ 0.9

During the three months ended March 31, 2018, total research and development expenses increased by $0.9 million compared to the three months ended March 31, 2017. The increase in the three months ended March 31, 2018 is primarily due to increased enrollment and activities associated with tazemetostat clinical development, expanded use of consulting and specialized services to support accelerated drug development efforts, and increased headcount to support development activities, as well as research related to advancing the company's preclinical G9a inhibitor program.

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

	Three Months Ended March 31,	
Product Program	2018	2017
	(In millions)	
External research and development expenses:		
Tazemetostat and related EZH2 programs	$ 11.2	$ 11.3
Pinometostat and related DOT1L programs	0.0	0.3
Discovery and preclinical stage product programs, collectively	3.9	3.4
Unallocated personnel and other expenses	10.5	9.7
Total research and development expenses	$ 25.6	$ 24.7

External research and development expenses for tazemetostat and related EZH2 programs remained consistent during the three months ended March 31, 2018, compared to the three months ended March 31, 2017. External research and development costs include external manufacturing costs related to the acquisition of active pharmaceutical ingredient and manufacturing of clinical drug supply, ongoing clinical trial costs, discovery and preclinical research in support of the tazemetostat program and expenses associated with our companion diagnostic program.

External research and development expenses for pinometostat and related DOT1L programs for the three months ended March 31, 2018 decreased $0.3 million when compared to the three months ended March 31, 2017. The decline in program spending reflects our completion of the pinometostat Phase 1 clinical trials during the fourth quarter of 2016 and the associated reduction in costs. The costs incurred related to pinometostat in the three months ended March 31, 2018 are primarily associated with costs attributed to the CRADA with the NCI.

External research and development expenses for discovery and preclinical stage product programs increased $0.5 million for the three months ended March 31, 2018 compared to the three months ended March 31, 2017, primarily related to increased development activities related to our novel G9a program for the potential treatment of sickle cell disease and expansion of activities related to our platform and other new target families.

Unallocated personnel and other expenses are comprised of compensation expenses for our full-time research and development employees and other general research and development expenses. Unallocated personnel and other expenses for the three months ended March 31, 2018 increased $0.8 million compared to the three months ended March 31, 2017. The increase in unallocated personnel and other expenses in the three months ended March 31, 2018 was primarily due to growth in our clinical, regulatory, and manufacturing functions to support continued development of tazemetostat.

We expect research and development expenses to increase significantly during the remainder of 2018, as we progress and expand our development program for tazemetostat, expand our regulatory activities, and advance a preclinical G9a program into later stage preclinical testing.

General and Administrative

The following is a comparison of general and administrative expenses for the three months ended March 31, 2018 and 2017:

	Three Months Ended March 31,		
	2018	2017	Change
	(In millions)		
General and administrative	$ 9.4	$ 8.3	$ 1.1

For the three months ended March 31, 2018, our general and administrative expenses increased $1.1 million compared to the three months ended March 31, 2017, primarily due to increased headcount and pre-commercial activities, including the build out of our medical affairs and commercial organizations.

We expect that general and administrative expenses will increase during the remainder of 2018, as we plan to increase our pre-commercial activities for tazemetostat.

Other Income, Net

The following is a comparison of other income, net for the three months ended March 31, 2018 and 2017:

	Three Months Ended
	March 31, 2018