Epizyme Provides Business Update and Reports First Quarter 2019 Financial Results

May 6, 2019

Tazemetostat NDA Submissions for Epithelioid Sarcoma and Follicular Lymphoma on Track for Second Quarter and Fourth Quarter 2019, Respectively

Updated Data from Tazemetostat Development Program to be Presented in the Second Quarter

First Research Milestone Achieved in Worldwide Collaboration with Boehringer Ingelheim to Develop Novel Epigenetic Oncology Therapies

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 6, 2019-- Epizyme, Inc. (Nasdaq: EPZM), a late-stage biopharmaceutical company developing novel epigenetic therapies, today provided business and pipeline updates and reported first quarter 2019 financial results.

"This is an incredibly exciting time for our company, and 2019 is slated to be one of the most important years in Epizyme's evolution," said Robert Bazemore, president and chief executive officer of Epizyme. "Our team is on track to submit two NDAs for tazemetostat this year, first for epithelioid sarcoma in the second quarter, which, if successful, would make tazemetostat the first commercially available EZH2 inhibitor, and then a second submission for follicular lymphoma in the fourth quarter. We are preparing for multiple clinical trials starting mid-year designed to expand the utility of tazemetostat, and to initiate clinical development of our novel G9a inhibitor in the second half of 2019. In addition, we have achieved the first milestone in our partnership with Boehringer Ingelheim, which further validates our research expertise and our collaboration strategy in epigenetic target discovery and drug development. With each milestone accomplished, we are one step closer to fulfilling our mission of bringing new treatments to patients with cancer and other serious diseases."

Tazemetostat Program Updates and Progress

- **NDA Submission for Epithelioid Sarcoma (ES) on Track for Second Quarter:**Epizyme is well underway with its preparations to submit its first New Drug Application (NDA) for accelerated approval to the U.S. Food and Drug Administration (FDA) in the second quarter of 2019 for tazemetostat in patients with ES. This is an ultra-rare and difficult-to-treat sarcoma, and if approved, tazemetostat would be the first treatment specifically indicated for patients with ES. Updated data from the company's ongoing Phase 2 trial in ES will be presented at the 2019 American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago; details will be disclosed at a future date.
- **NDA Submission for All-Comer Follicular Lymphoma (FL) Population Planned for Fourth Quarter:**Epizyme plans to submit an NDA for accelerated approval of tazemetostat for patients with FL, regardless of their EZH2 mutational status, who have been previously treated with two or more systemic therapies. The ongoing Phase 2 study has completed enrollment, and the company is working toward the submission of an NDA for accelerated approval in the fourth quarter of 2019 for this patient population. Updated data from the Phase 2 trial will be presented at a medical meeting in the second quarter of 2019. Details will be disclosed at a future date.

Planned Tazemetostat Clinical Studies in 2019

Epizyme is planning for multiple clinical trials designed to expand the benefit of tazemetostat into earlier treatment lines in follicular lymphoma, and to explore new combinations and potential indications in both FL and solid tumors. Planned clinical trials include:

- a combination study of tazemetostat with the chemo-free treatment regimen "R^2" (Revlimid® plus Rituxan®) for patients with relapsed/refractory FL who have received at least one prior therapy;
- a combination study of tazemetostat with Rituxan for patients with relapsed/refractory FL;
- a combination study of tazemetostat with R-CHOP for front-line patients with FL in collaboration with the Lymphoma Study Association (LYSA);
- a combination study of tazemetostat with the standards-of-care for patients with castration-resistant prostate cancer; and
- a combination study of tazemetostat with a PARP inhibitor for patients with platinum-resistant solid tumors, such as small-cell lung cancer, triple-negative breast cancer and ovarian cancer.

Initiation of Clinical Development of EZM8266 for Sickle Cell Disease

- Upon approval of an Investigational New Application (IND) for EZM8266 for the treatment of patients with sickle cell disease, Epizyme anticipates beginning clinical development in the second half of 2019 with a Phase 1 trial of EZM8266, a novel, first-in-class G9a inhibitor.

Business Updates

- Epizyme recently earned a $5.5 million milestone payment from Boehringer Ingelheim, following the selection of a lead optimization candidate for the shared program targeting an enzyme within the helicase family. The companies are jointly researching and developing this program, with both parties sharing U.S. commercialization responsibilities and Boehringer Ingelheim assuming responsibility for commercialization outside the U.S. The companies will also share research responsibilities for a histone acetyltransferase (HAT) program that is under development.
- In March of this year, Epizyme raised $172.50 million in aggregate gross proceeds, before deducting underwriting discounts and offering expenses, from two concurrent underwritten public offerings.

First Quarter 2019 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $371.1 million as of March 31, 2019, as compared to $247.9 million as of March 31, 2018.
- **Revenue:** Collaboration revenue for the first quarter of 2019 was $7.9 million. There was no collaboration revenue recognized for the first quarter of 2018. The increase in collaboration revenue is due to the company's collaboration with Boehringer Ingelheim, which was initiated in November 2018.
- **R&D Expenses:** Research and development (R&D) expenses were $26.9 million for the first quarter of 2019, compared to $25.6 million for the first quarter of 2018. The increase is primarily due to greater tazemetostat manufacturing costs and costs incurred in preparation for two NDA submissions offset by decreases in clinical trial expenses.
- **G&A Expenses:** General and administrative (G&A) expenses were $12.0 million for the first quarter of 2019, compared to $9.4 million for the first quarter of 2018. The increase is primarily due to a rise in medical affairs and commercial costs as a result of organizational development in preparation for tazemetostat commercialization.
- **Net Loss Attributed to Common Stockholders:** Net loss attributable to common stockholders was $32.3 million, or $0.39 per share, for the first quarter of 2019, compared to $34.1 million, or $0.49 per share, for the first quarter of 2018.

Financial Guidance

Following its March financing, and based on its current operating plan, Epizyme expects its cash runway to extend into at least the first quarter of 2021.

The company will not hold a conference call in conjunction with these results.

About the Epizyme-Boehringer Ingelheim Collaboration

Epizyme and Boehringer Ingelheim established a worldwide collaboration agreement in November 2018 to develop novel epigenetic oncology therapies. Under the terms of the agreement, Boehringer Ingelheim and Epizyme will jointly research and develop a helicase program, with both parties sharing U.S. commercialization responsibilities and Boehringer Ingelheim assuming responsibility for commercialization outside the U.S. Epizyme and Boehringer Ingelheim will also share research responsibilities for a histone acetyltransferase (HAT) program, with Boehringer Ingelheim assuming responsibility for worldwide development and commercialization. Epizyme received an upfront payment of $15 million and will receive an additional $5 million in research funding in 2019, and is eligible to receive up to $280.5 million in research, development and commercialization milestones. For the helicase program, Epizyme will fund a portion of the global development costs, retain a share of U.S. profits and receive tiered royalties on ex-U.S. sales. For the HAT program, Epizyme is eligible to receive tiered royalties on worldwide sales.

About Epizyme, Inc.

Epizyme, Inc. is a late-stage biopharmaceutical company committed to rewriting treatment for cancer and other serious diseases through novel epigenetic medicines. Epizyme is broadly developing its lead product candidate, tazemetostat, a first-in-class EZH2 inhibitor, with studies underway in both solid tumors and hematological malignancies, as a monotherapy and combination therapy in relapsed and front-line disease. The company also is developing a novel G9a program with its next development candidate, EZM8266, which is targeting sickle cell disease. By focusing on the genetic drivers of disease, Epizyme's science seeks to match targeted medicines with the patients who need them. For more information, visit www.epizyme.com.

Cautionary Note on Forward-Looking Statements

Any statements in this press release about future expectations, plans and prospects for Epizyme, Inc. and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: uncertainties inherent in the initiation of future clinical studies and in the availability and timing of data from ongoing clinical studies; whether interim results from a clinical trial will be predictive of the final results of the trial; whether results from preclinical studies or earlier clinical studies will be predictive of the results of future trials; whether results from clinical studies will warrant meetings with regulatory authorities, submissions for regulatory approval or review by governmental authorities under the accelerated approval process; whether Fast Track Designation and Orphan Drug Designations will provide the benefits for which tazemetostat is eligible; expectations for regulatory approvals, including accelerated approval, to conduct trials or to market products; whether the company's cash resources will be sufficient to fund the company's foreseeable and unforeseeable operating expenses

and capital expenditure requirements; other matters that could affect the availability or commercial potential of the company's therapeutic candidates; and other factors discussed in the "Risk Factors" section of the company's most recent Form 10-Q filed with the SEC and in the company's other filings from time to time with the SEC. In addition, the forward-looking statements included in this press release represent the company's views as of the date hereof and should not be relied upon as representing the company's views as of any date subsequent to the date hereof. The company anticipates that subsequent events and developments will cause the company's views to change. However, while the company may elect to update these forward-looking statements at some point in the future, the company specifically disclaims any obligation to do so.

EPIZYME, INC.
CONDENSED CONSOLIDATED BALANCE SHEET DATA (UNAUDITED)

(Amounts in thousands)

	March 31, 2019	December 31, 2018
Consolidated Balance Sheet Data:		
Cash, cash equivalents, and marketable securities	$ 371,146	$ 240,304
Total assets	412,722	275,501
Current portion of deferred revenue	5,409	13,300
Deferred revenue, net of current portion	3,806	3,806
Total stockholders' equity	368,353	233,009

EPIZYME, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (UNAUDITED)
(Amounts in thousands except per share data)

	Three Months Ended March 31,	
	2019	2018
Collaboration revenue	$ 7,891	$ —
Operating expenses:		
Research and development	26,896	25,622
General and administrative	11,986	9,360
Total operating expenses	38,882	34,982
Operating loss	(30,991)	(34,982)
Other income, net:		
Interest income, net	1,658	899
Other (expense) income, net	(6)	18
Other income, net	1,652	917
Net loss	$ (29,339)	$ (34,065)
Reconciliation of net loss to net loss attributable to common stockholders:		
Net loss	$ (29,339)	$ (34,065)
Accretion of convertible preferred stock	(2,940)	-
Net loss attributable to common stockholders	$ (32,279)	$ (34,065)
Net loss per share attributable to common stockholders - basic and diluted	$ (0.39)	$ (0.49)
Weighted-average common shares outstanding used in net loss per share attributable to common stockholders - basic and diluted	82,424	69,386

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