Epizyme Reports Third Quarter 2017 Operating Results and Company Updates

November 1, 2017

Company Scheduled for First FDA Interaction for Tazemetostat in NHL in 4Q17

Chief Medical Officer, Dr. Peter Ho, to Transition from Epizyme at the End of 2017; Will Continue to Support Upcoming Clinical and Regulatory Milestones through Transition

Recent Financing Extends Operating Runway into at Least 3Q19

Conference Call to be Held Today at 4:30 p.m. ET

CAMBRIDGE, Mass., Nov. 01, 2017 (GLOBE NEWSWIRE) -- Epizyme, Inc. (NASDAQ:EPZM) today reported operating results for the third quarter 2017, recent progress in its clinical development programs for tazemetostat in non-Hodgkin's lymphoma (NHL) and solid tumors, and company updates.

"2017 has been a year of tremendous progress, with additional important milestones remaining this quarter, including our scheduled interaction with the FDA to begin discussing the registration strategy for tazemetostat in NHL," said Robert Bazemore, president and chief executive officer of Epizyme. "Our experienced management team is executing well on the drivers for both short and long-term Epizyme growth. Our clinical organization is delivering on a comprehensive tazemetostat clinical program according to our plans; our research team has named G9a as the target for the next program in our pipeline; and our business operations team completed a capital raise that meaningfully extended our runway. I am confident in our ability to build on this momentum as we begin the transition into a commercial company, with our first NDA submission targeted for 2018 and the potential launch of tazemetostat to follow."

Due to the company's transition, Peter T.C. Ho, M.D., Ph.D., chief medical officer, has decided to leave the company at the end of 2017. Throughout the rest of this year, Dr. Ho will continue to oversee all clinical programs, upcoming clinical milestones and preparation of medical congress presentations, as well as support the scheduled interaction with the U.S. Food and Drug Administration (FDA) in the fourth quarter. Epizyme is conducting an active search for a seasoned clinical executive and is in the process of securing an interim CMO to serve in a consultative capacity during the transition.

Bazemore added, "Based on our planned transformation in 2018 and his passion for early-stage drug development, Peter will be transitioning from Epizyme at the end of the year. Since joining in 2014, Peter has been instrumental in building our clinical team and bringing the tazemetostat program to where it is today. We are very thankful to Peter for his many contributions and wish him all the best in the future. This transition allows us the opportunity to add new strength to the team with a seasoned clinical executive with extensive late-stage drug development experience and a track record of driving new treatments to approval."

Tazemetostat NHL Program Updates

- Epizyme is on-track to hold the first interaction with the FDA in the fourth quarter of 2017 to discuss its NHL program, and plans to provide an update in early 2018.
- The ongoing Phase 2 study in patients with follicular lymphoma and diffuse large B-cell lymphoma continues to enroll patients with EZH2 mutations. As a result of Epizyme's efforts to accelerate enrollment of these patients, which includes the recently initiated collaboration with US Oncology, August and September were the highest screening months to date for the study.

Tazemetostat Solid Tumor Program Updates

- **Phase 2 Mesothelioma Study:** Epizyme's ongoing Phase 2 study designed to evaluate tazemetostat as a treatment for adults with mesothelioma characterized by BAP1 loss-of-function has surpassed the futility assessment and achieved the primary endpoint of at least a 30 percent disease control rate at 12 weeks. The company expects to report data from this study in 2018. In addition, the FDA has granted Orphan Drug designation to tazemetostat for the treatment of patients with mesothelioma.
- **Pediatric Phase 1 INI1-Negative Tumor Study**: Data from the completed dose-escalation portion of Epizyme's Phase 1 study of tazemetostat in pediatric patients with INI1-negative solid tumors were presented at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics. Objective responses were observed in patients with epithelioid sarcoma (n=1), poorly differentiated chordoma (n=2) and atypical teratoid rhabdoid tumors (n=1) at dose levels ranging from 520 to 900 mg/m^2 twice daily. Enrollment in the dose-expansion portion of the study is ongoing.
- **Adult Phase 2 INI1-Negative Tumor Study:** The malignant rhabdoid tumor cohort and other INI1-negative tumor cohort of the company's ongoing Phase 2 study in molecularly defined solid tumors have both surpassed their futility assessment with objective responses observed in both populations. Epizyme is continuing to enroll and monitor patients in these arms, and plans to present updated data from this study in 2018. The company has also added a separate cohort to enroll adults with chordoma, due to the high rate of enrollment of these patients in the other INI1-negative cohort and the observed clinical activity with tazemetostat in this tumor type in both adults and children so far.

Upcoming Data Presentations

- Previously announced data on adult and pediatric epithelioid sarcoma patients in the company's ongoing INI1-negative solid tumor studies will be presented during a plenary session at the Connective Tissue Oncology Society (CTOS) Annual Meeting at 1:00 p.m. HAST on Thursday, Nov. 9, 2017. The adult data were originally presented at the American Society of Clinical Oncology (ASCO) 2017 Annual Meeting and the pediatric data at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics.
- Epizyme will introduce the next development program in its pipeline, which addresses G9a, a novel target for the potential treatment of sickle cell disease (SCD), during a plenary presentation at the 2017 American Society of Hematology (ASH) Annual Meeting at 7:30 a.m. ET on Monday, Dec. 11, 2017. This is the first of three internally discovered programs expected to enter the clinic by the end of 2020.
- New patient data from the company's 62-gene panel biomarker study of tazemetostat in patients with NHL will also be presented in a poster session at the ASH Annual Meeting.

Third Quarter 2017 Financial Results

- **Cash Position**: Cash, cash equivalents and marketable securities were $307.2 million as of September 30, 2017, as compared to $263.3 million as of September 30, 2016. The increase is mainly driven by the $151.3 million in net proceeds following the company's underwritten public offering of 10,557,000 shares of common stock.
- **Revenue:** There was no revenue recognized in the third quarter of 2017, compared to $6.6 million for the third quarter of 2016, as there were no collaboration milestones during the quarter.
- **R&D Expenses:** Research and development (R&D) expenses were $28.7 million for the third quarter of 2017, compared to $23.9 million for the third quarter of 2016. The increase is primarily due to increased tazemetostat manufacturing activities, tazemetostat clinical development and research activities related to advancing the company's next development program.
- **G&A Expenses**: General and administrative (G&A) expenses were $9.3 million for the third quarter of 2017, compared to $7.5 million for the third quarter of 2016. The increase is primarily due to increased personnel-related expenses to support the company's growth and pre-commercial activities.
- **Net Loss:** Net loss was $37.6 million for the quarter ended September 30, 2017, compared to $24.3 million for the quarter ended September 30, 2016.

Financial Guidance
Epizyme provides financial guidance that the company believes that its existing cash, cash equivalents and marketable securities as of September 30, 2017 will be sufficient to fund its planned operations into at least the third quarter of 2019.

About the Tazemetostat Clinical Trial Program
Tazemetostat, a first-in-class EZH2 inhibitor, is currently being studied as a monotherapy in ongoing Phase 2 programs in both follicular lymphoma (FL) and diffuse large B-cell lymphoma (DLBCL) forms of non-Hodgkin lymphoma (NHL); certain molecularly defined solid tumors, including epithelioid sarcoma and other INI1-negative tumors; mesothelioma; and combination studies in DLBCL. Tazemetostat has been granted Fast Track designation by the U.S. Food and Drug Administration for FL regardless of EZH2 mutation status and for DLBCL with EZH2-activating mutations, as well as Orphan Drug designation for mesothelioma, soft tissue sarcoma and malignant rhabdoid tumors.

About Orphan Drug Designation
The FDA Orphan Drug designation program provides a special status to drugs and biologics intended to treat, diagnose or prevent so-called orphan diseases and disorders that affect fewer than 200,000 people in the U.S. This designation provides for a seven-year marketing exclusivity period against competition, as well as certain incentives, including federal grants, tax credits and a waiver of PDUFA filing fees.

About Epizyme, Inc.
Epizyme, Inc. is a clinical-stage biopharmaceutical company committed to rewriting cancer treatment 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. Using the Company's proprietary platform, Epizyme has pioneered the identification and development of small molecule inhibitors of chromatin modifying proteins (CMPs), such as tazemetostat. CMPs are part of the system of gene regulation, referred to as epigenetics, that controls gene expression. Genetic alterations can result in changes to the activity of CMPs, which can allow cancer cells to grow and proliferate. By focusing on the genetic drivers of cancers, Epizyme's science seeks to match targeted medicines with the specific patients that need it. For more information, visit www.epizyme.com and connect with us on Twitter at @EpizymeRx.

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 and orphan drug designations will provide the benefits for which tazemetostat is eligible; expectations for regulatory approvals 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.

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EPIZYME, INC.
CONSOLIDATED BALANCE SHEET DATA (UNAUDITED)
(Amounts in thousands)

	September 30, 2017	December 31, 2016
Consolidated Balance Sheets Data :		
Cash, cash equivalents and marketable securities	$ 307,228	$ 242,192
Total assets	320,546	252,441
Deferred revenue	28,809	28,809
Total stockholders' equity	268,189	201,700

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Collaboration revenue	$ -	$ 6,584	$ 10,000	$ 7,529
Operating expenses:				
Research and development	28,741	23,888	80,728	63,078
General and administrative	9,311	7,522	28,750	20,792
Total operating expenses	38,052	31,410	109,478	83,870
Loss from operations	(38,052)	(24,826)	(99,478)	(76,341)
Other income, net	455	490	1,335	1,145
Net loss	$ (37,597)	$ (24,336)	$ (98,143)	$ (75,196)
Loss per share allocable to common stockholders:				
Basic	$ (0.63)	$ (0.42)	$ (1.67)	$ (1.32)
Diluted	$ (0.63)	$ (0.42)	$ (1.67)	$ (1.32)
Weighted average shares outstanding:				
Basic	59,899	57,970	58,837	56,828
Diluted	59,899	57,970	58,837	56,828

Source: Epizyme, Inc.