October 22, 2013

Epizyme, Inc. Provides Third Quarter 2013 Financial Results and Corporate Update

- Top-line data for Phase 1 study of EPZ-5676 expected in fourth quarter -
- Significant clinical expansion planned for DOT1L and EZH2 programs in 2014 -

CAMBRIDGE, Mass., Oct. 22, 2013 /PRNewswire/ -- Epizyme, Inc. (NASDAQ: EPZM), a clinical stage biopharmaceutical company creating innovative personalized therapeutics for patients with genetically defined cancers, today provided a corporate update and reported financial results for the quarter ended September 30, 2013.

"2013 has been an important year for Epizyme as we translate the science of epigenetics into personalized therapeutics," said Robert Gould, Ph.D., chief executive officer. "We currently have two ongoing clinical programs, which to our knowledge are the only histone methyltransferase (HMT) inhibitor programs to have entered human clinical development. EPZ-5676, an inhibitor of the HMT DOT1L, is being developed as a treatment for patients with acute leukemias with rearrangements of the MLL gene (MLL-r). The dose escalation stage of the Phase 1 study of EPZ-5676 is proceeding and no dose-limiting toxicities have been observed to date. We have been highly encouraged by the tolerability thus far and believe this represents a significant step forward for this first-in-class epigenetic program and for the entire field. We anticipate completion of this stage by the end of the year. In the fourth quarter of 2013, we will share top-line dose escalation data and, based on data from the dose escalation stage, plan to initiate an expansion cohort stage that will be limited to patients with MLL-r. This expansion cohort is expected to provide an initial assessment of therapeutic effect in this population in 2014."

Dr. Gould continued, "In addition, for EPZ-6438, an inhibitor of the HMT EZH2 being developed as a treatment for non-Hodgkin lymphoma patients with oncogenic point mutations in EZH2, Epizyme, with our partner Eisai, initiated a Phase 1/2 clinical trial in June 2013. This Phase 1 dose escalation study is ongoing and no dose-limiting toxicities have been observed to date."

"Looking ahead to 2014, we plan to pursue additional clinical studies for both candidates in genetically defined cancers beyond the primary indications, including MLL-PTD for EPZ-5676 and synovial sarcoma and other INI1-deficient tumors for EPZ-6438," said Gould. "We are in a strong position to invest in Epizyme's internally generated pipeline and look forward to continued progress with multiple proof of concept studies in 2014."

EPZ-5676 Clinical Development — Progress and Plans

- **Top-line Dose Escalation Data Expected in Fourth Quarter of 2013:** The dose escalation stage of the ongoing Phase 1 study of EPZ-5676 is nearing completion, and Epizyme plans to disclose top-line dose escalation data in the fourth quarter of 2013.
- **Clinical Sites Added:** Five clinical sites have been added this year, bringing the total number of clinical sites participating in this study to six.
- **Granted Orphan Drug Designation:** EPZ-5676 was granted orphan drug designation by the U.S. FDA in August 2013.
- **MLL-r Restricted Expansion Cohort Stage to Begin in Fourth Quarter of 2013:** Based on data from the dose escalation stage, Epizyme plans to initiate the expansion cohort stage of the ongoing Phase 1 study in the fourth quarter of 2013. The expansion cohort will be limited to patients with MLL-r and is expected to provide an initial assessment of therapeutic effect in MLL-r patients in 2014. It will include as many as 12 sites in both the United States and Europe. Abbott Molecular Inc., under its collaboration with Epizyme, is developing a molecular companion diagnostic to identify eligible MLL-r patients for EPZ-5676, and the Investigational Use Only (IUO) diagnostic will be available for use in the expansion cohort.
- **Initiation of Pediatric MLL-r Phase 1 Study in 2014:** Based on the findings to date in the Phase 1 study of EPZ-5676 in adults, Epizyme plans to initiate a Phase 1 trial of EPZ-5676 in pediatric patients with MLL-r leukemia in the first half of 2014. The study will initially be open in approximately 5-6 investigational sites in the U.S.
- **Broadening Program into AML with MLL-PTD in 2014:** Epizyme plans to initiate a clinical study of EPZ-5676 in patients with AML with a genetic alteration called MLL-PTD. MLL-PTD cell lines and animal models exhibit similar sensitivity to DOT1L inhibition as seen in MLL-r pre-clinical studies. This data was presented on October 21, 2013, at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics. MLL-PTD accounts for an estimated 5-7% of adult AML cases with an estimated annual incidence of 2,300 patients in the major markets (U.S., EU and Japan) and represents a meaningful potential expansion of the clinical opportunity for EPZ-5676.

EPZ-6438 Clinical Development — Progress and Plans

- **Initiated Dose Escalation Study:** In June 2013 Epizyme announced the enrollment of the first patient in a Phase 1/2 study of EPZ-6438 (referred to as E7438 by Eisai). The Phase 1 dose escalation study is ongoing at two sites in France, and no dose-limiting toxicities have been observed to date. The companies also plan to significantly increase the number of clinical sites in 2014, and to submit an IND in the U.S., in anticipation of the Phase 2 initiation.
- **Phase 2 Initiation Expected in 2014:** With partner Eisai, Epizyme plans to initiate the Phase 2 portion of this study in 2014 after completion of the dose escalation phase. This phase will only enroll patients with non-Hodgkin lymphoma with oncogenic point mutations in EZH2 and is expected to provide an initial assessment of therapeutic effect.
- **Broadening Program into INI1-Deficient Tumors in 2014:** Epizyme plans to initiate clinical trials in patients with tumors in which there is genetically altered INI1 after completion of the ongoing Phase 1 study. EZH2 plays a driving oncogenic role in INI1-deficient tumors, as exemplified in a paper published in the April 2013 *Proceedings of the National Academy of Sciences* in which Epizyme scientists demonstrated sustained tumor regressions in pre-clinical studies of malignant rhabdoid tumors (MRT) and in an October 21, 2013 presentation at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics that describes the oncogenic role of EZH2 in synovial sarcomas, a malignancy in which INI1 function is altered as a result of a specific chromosomal translocation (SS18-SSX). In the major markets, synovial sarcoma has an annual incidence of 1,700 patients, and other INI1-deficient tumors together have an annual incidence of 700 patients.

Additional Business Highlights

- **Continued Pipeline Development of Personalized Therapeutics:** Pre-clinical studies are ongoing to identify additional genetically defined cancers for potential treatment with Epizyme's therapeutic candidates. Planned clinical studies in MLL-PTD and INI1-deficient tumors, focusing on synovial sarcoma, are examples of the novel therapeutic insights provided by Epizyme's product platform. Epizyme also continues to advance the HMT targets in the GSK collaboration as well as other non-partnered HMT targets.
- **Strengthened Intellectual Property Portfolio:** In August 2013, the U.S. Patent and Trademark Office (USPTO) issued a Notice of Allowance for U.S. Patent Application No. 13/310,157 "SUBSTITUTED PURINE AND 7-DEAZAPURINE COMPOUNDS" with claims covering the novel compound EPZ-5676. This is the first composition of matter patent for EPZ-5676 and, once issued, this patent will expire in 2032. Also in September 2013, the USPTO issued U.S. Patent No. 8,524,467 titled "Diagnostic and Therapeutic Targets for Leukemia," with claims directed to methods of identifying DOT1L inhibitor compounds for the treatment of leukemia. This patent expires in 2030.

Third Quarter 2013 Financial Results & Financial Guidance

- **Cash Position:** Cash and cash equivalents as of September 30, 2013, were $139.6 million, compared to $98.0 million as of December 31, 2012. The cash increase was driven by net proceeds of $82.5 million from Epizyme's initial public offering in June 2013 and a $6 million milestone achieved in June 2013 from the Eisai collaboration for the initiation of the EPZ-6438 Phase 1/2 study.
- **Revenue:** Collaboration revenue was $8.4 million for the third quarter of 2013 and $32.2 million for the nine months ended September 30, 2013, compared to $15.3 million and $36.3 million for the comparable periods in 2012. Collaboration revenue includes deferred revenue from payments received in previous periods as well as payments received and recognized during the respective periods.
- **R&D Expenses:** Research and development expenses were $14.6 million for the third quarter of 2013 and $41.9 million for the nine months ended September 30, 2013, compared to $9.3 million and $27.4 million for the comparable periods in 2012. The increase was largely driven by the expansion of Epizyme's product platform and costs for the clinical studies of EPZ-5676 and EPZ-6438.
- **G&A Expenses:** General and administrative expenses were $3.6 million for the third quarter of 2013 and $9.7 million for the nine months ended September 30, 2013, compared to $1.6 million and $5.2 million for the comparable periods in 2012. The increase was largely driven by incremental expenses to support public company operations as well as increased stock-based compensation expense and other costs to support Epizyme's growth.
- **Net (Loss) Income:** Net loss was $9.7 million for the third quarter of 2013 and $19.4 million for the nine months ended September 30, 2013, compared to net income of $4.4 million and $3.8 million for the comparable periods in 2012. The 2012 net income was largely driven by the revenue recognition of a portion of the $68.0 million upfront payment received from Celgene in April 2012.
- **Shares Outstanding:** Shares outstanding as of September 30, 2013 were 28.4 million, following the sale of 5.9 million shares of common stock in the Company's initial public offering and the resulting automatic conversion of the Company's redeemable convertible preferred stock into 20.6 million shares of common stock. This compares to 1.7 million shares outstanding as of September 30, 2012, which did not include the Company's redeemable convertible preferred stock. Basic weighted average shares outstanding for the nine months ended September 30, 2013 were 13.2 million. Epizyme expects basic weighted average shares outstanding to be approximately 17 million shares for the full-year 2013.
- **End of Year Guidance:** Epizyme expects full-year 2013 net cash used in operating activities of approximately $60 million, full-year 2013 GAAP revenue of approximately $40 million, and to end the year with more than $115 million in cash and cash equivalents.

Conference Call Information

Epizyme will host a conference call and live audio webcast today at 4:30 p.m. EDT to discuss the quarter and provide a corporate update. To participate in the conference call, please dial 1-877-303-9053 (domestic) or 1-970-315-0464 (international) and refer to conference ID 85846372. The live webcast can be accessed under "Events and Presentations" in the Investor Relations section of the Company's website at www.epizyme.com.

The archived webcast will be available on the Company's website beginning approximately two hours after the event.

About Epizyme, Inc.

Epizyme, Inc. is a clinical stage biopharmaceutical company creating personalized therapeutics for patients with genetically defined cancers. Epizyme has built a proprietary product platform that the company uses to create small molecule inhibitors of a 96-member class of enzymes known as histone methyltransferases, or HMTs. HMTs are part of the system of gene regulation, referred to as epigenetics, that controls gene expression. Genetic alterations can result in changes to the activity of HMTs, making them oncogenic (cancer-causing). By focusing on the genetic drivers of cancers, Epizyme's targeted science seeks to match the right medicines with the right patients for a personalized approach to cancer treatment.

For more information, visit www.epizyme.com and connect with us on Twitter at @EpizymeRx.

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

	September 30, 2013	December 31, 2012
Cash and cash equivalents	$ 139,575	$ 97,981
Total assets	147,350	103,511
Deferred revenue	50,706	69,445
Redeemable convertible preferred stock (Series A, B and C)	-	76,156
Stockholders' equity (deficit)	87,283	(51,126)

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

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

Basic	28,406	1,651	13,212	1,637
Diluted	28,406	3,017	13,212	2,641

Cautionary Note on Forward-Looking Statements

Any statements in this press release about future expectations, plans and prospects for the Company, including statements about the Company's strategy, future operations, clinical development of the Company's therapeutic candidates, expectations regarding the sufficiency of the Company's cash balance to fund operating expenses and capital expenditures, milestone or royalty payments from the Company's collaborators, the Company's anticipated milestones and future expectations and plans and prospects for the Company 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: the uncertainties inherent in the initiation of future clinical trials, expectations of expanding ongoing clinical trials, availability and timing of data from ongoing clinical trials, expectations for regulatory approvals, development progress of the Company's companion diagnostics, availability of funding sufficient for 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 or companion diagnostics and other factors discussed in the "Risk Factors" section of the Company's 10-Q filed with the Securities and Exchange Commission on August 1, 2013. In addition, the forward-looking statements included in this press release represent the Company's views as of 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. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date hereof

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