



Epizyme Presents Data from a Phase 1 Trial of Tazemetostat in Children with Relapsed or Refractory INI1-Negative Solid Tumors at the 2017 AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics

First presentation of pediatric dose escalation data in tazemetostat, showing favorable safety and tolerability with promising anti-tumor activity

CAMBRIDGE, Mass., Oct. 27, 2017 – Epizyme, Inc. (NASDAQ: EPZM), a clinical-stage biopharmaceutical company creating novel epigenetic therapies, today announced encouraging data from the dose escalation portion of the company's ongoing Phase 1 clinical trial of tazemetostat, a first-in-class, oral EZH2 inhibitor, in pediatric patients with relapsed or refractory INI1-negative molecularly defined solid tumors. These data will be presented today during the Spotlight on Proffered Papers oral presentation session starting at 10:50 a.m. ET at the 2017 AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics, which is being held Oct. 26-30, 2017 in Philadelphia. The study results will be further detailed in a poster presentation on Saturday, Oct. 28, 2017 at 12:30 p.m. ET.

The data, which are being presented by principal investigator Susan N. Chi, M.D., Director, Pediatric Brain Tumor Clinical Trials Program, Dana-Farber/Boston Children's Cancer and Blood Disorders Center, and Assistant Professor of Pediatrics, Harvard Medical School, are the first presentation of tazemetostat results in pediatric patients, providing continued evidence for tazemetostat's activity in INI1-negative tumors, as seen in adult patients.

“We currently have little to offer children with relapsed or refractory INI1-negative tumors because of the limited efficacy and significant side effects of available treatments,” said Dr. Chi. “In this study, it’s promising to see the tolerability profile and anti-tumor activity of this novel agent among our youngest of patients.”

The open-label, multi-dose, multi-center Phase 1 dose escalation study was conducted in 46 patients aged six months to 21 years with INI1-negative tumors including epithelioid sarcoma, poorly differentiated chordoma, atypical teratoid rhabdoid tumors, malignant rhabdoid tumors, renal medullary carcinoma or relapsed/refractory synovial sarcoma. The oral suspension of tazemetostat was administered twice daily in continuous 28-day cycles in the following cohorts:



240mg/m², 300mg/m², 400mg/m², 520mg/m², 700mg/m², 900mg/m², 1200mg/m².

Tazemetostat was generally well-tolerated at all explored doses, including the highest dose tested. Adverse events (AEs) reported, regardless of attribution, were mostly mild to moderate, the most common of which were vomiting (41%), pyrexia (28%), headache (24%) and nausea (24%). Only one patient experienced a dose-limiting toxicity (DLT) event at the dose level of 300mg/m² (Grade 4 dyspnea and Grade 3 hypoxia); however, no other DLTs were observed at higher doses. One other patient discontinued the study due to a treatment-related AE, and five patients had dose reductions.

Tazemetostat showed encouraging anti-tumor activity across a range of INI1-negative cancers in pediatric patients. Complete or partial responses were observed in patients at dose levels ranging from 520 to 900 mg/m² twice daily, as follows:

- Complete responses in epithelioid sarcoma (n=1), chordoma (n=1), atypical teratoid rhabdoid tumor (n=1)
- Partial response in chordoma (n=1)

The recommended Phase 2 dose of 1200 mg/m² twice daily was established based on safety, pharmacokinetics, pharmacodynamics and activity. The study is now enrolling patients into four dose expansion cohorts.

“The data from this study, specifically the favorable safety profile and the objective responses in three different cancers, are further evidence of the progress of our tazemetostat program,” said Peter Ho, M.D., Ph.D., chief medical officer of Epizyme. “The results from the dose escalation portion of this study support the potential of tazemetostat to treat INI1-negative cancers, in both the pediatric and adult treatment settings. We are grateful to the children and families who have participated in this study and are encouraged by the potential of tazemetostat to benefit a patient population with such a significant unmet medical need.”

About the Tazemetostat Clinical Trial Program

Tazemetostat, a first-in-class EZH2 inhibitor, is currently being studied in ongoing Phase 2 programs in both follicular lymphoma (FL) and diffuse large B-cell lymphoma (DLBCL) forms of non-Hodgkin lymphoma; certain molecularly defined solid tumors, including epithelioid sarcoma and other INI1-negative tumors; and mesothelioma, as well as in 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 and for DLBCL with EZH2-activating mutations, as well as Orphan Drug designation for malignant rhabdoid tumors, soft tissue sarcoma, and mesothelioma.

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 and 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.

Cautionary Note on Forward-Looking Statements

Any statements in this press release about future expectations, plans and prospects for Epizyme, Inc., including statements about the Company's anticipated use of proceeds from the offering, future operations, clinical development of the Company's therapeutic candidates, expectations regarding future clinical trials 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: uncertainties related to market conditions, uncertainties inherent in the initiation of future clinical trials, and other factors discussed in the "Risk Factors" section of the preliminary prospectus filed with the SEC on September 13, 2017, the Company's most recent Form 10-Q filed with the SEC and in other filings the Company makes 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, except as required by law.

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