Epizyme Receives Orphan Drug Designation for EPZ-5676

Small Molecule Inhibitor of the DOT1L Histone Methyltransferase (HMT) designated for Acute Leukemias with MLL Rearrangements (MLL-r)

Cambridge, Mass. – August 16, 2013 – Epizyme, Inc., (NASDAQ: EPZM) a clinical stage biopharmaceutical company creating innovative personalized therapeutics for patients with genetically defined cancers, announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for EPZ-5676. Criteria for orphan drug designation requires that the product be intended for treatment of a rare disease or condition, classified as affecting fewer than 200,000 people in the United States.

Orphan drug designation allows special incentives for sponsors planning to test a product for use in a rare disease or condition. These incentives include tax credits, research and development grant funding and reduced filing fees during development or at the time of application for marketing approval. Once approved, the product may qualify for seven years of marketing exclusivity independent of any other intellectual property.

About EPZ-5676

Epizyme is developing EPZ-5676, a small molecule inhibitor of DOT1L created with Epizyme’s proprietary product platform, for the treatment of acute leukemias in which the MLL gene is rearranged due to a chromosomal translocation (MLL-r). Due to the translocation, DOT1L is recruited to specific locations in the chromosome where it would not normally be present. As a result, DOT1L causes inappropriate methylation at these locations, which results in the increased expression of genes causing leukemia.

In September 2012, Epizyme initiated a Phase 1 clinical trial for EPZ-5676. The company believes EPZ-5676 is the first HMTi to enter human clinical development. As of August 2013, this program is in the dose escalation phase and is expected to initiate an expansion phase in the second half of 2013 that will exclusively enroll MLL-r patients.

Epizyme retains all U.S. rights to EPZ-5676 and has granted Celgene an exclusive license to EPZ-5676 outside of the United States. Epizyme has partnered with Abbott to develop a companion diagnostic to identify MLL-r patients. Additional information about these partnerships may be found here: http://www.epizyme.com/about-us/partnerships.asp

About MLL-r

MLL-r is an aggressive subtype of two of the most common forms of acute leukemia, acute myelogenous leukemia (AML) and acute lymphoblastic leukemia (ALL). The disease predominantly occurs in two different age ranges, an adult population and a pediatric population. Although both age ranges share a common genetic driver, the adult disease is frequently a secondary leukemia resulting from prior chemotherapy for a different, unrelated cancer, while the pediatric disease is sporadic in nature. According to research published in the journal Blood in December 2002, the five-year overall survival rate for adult patients with the MLL-r subtype of AML ranges from approximately 5 to 24 percent. The
five-year event-free survival rate in pediatric patients with the most common MLL-r subtype of ALL is approximately 27 percent, as identified in research published in the *New England Journal of Medicine* in 2004. The total annual incidence of MLL-r in all patients (adult and pediatric, AML and ALL) in major pharmaceutical markets is approximately 4,900 patients, according to a report by Clarion Healthcare commissioned by Epizyme. Patients with MLL-r are routinely diagnosed today with existing tests that are commonly used in clinical settings, and there is high awareness of MLL-r among oncologists.

**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.

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