Verastem Receives Orphan Drug Designation from the U.S. FDA for Defactinib in Mesothelioma

July 24, 2013

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jul. 24, 2013-- Verastem, Inc., (NASDAQ: VSTM) focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, announced that lead cancer stem cell inhibitor, VS-6063 (defactinib), has received orphan drug designation from the U.S. Food and Drug Administration (FDA) for use in the treatment of mesothelioma, a rare form of lung cancer. The designation is designed to encourage the development of drugs which may provide significant benefit to patients suffering from rare diseases.

“Mesothelioma is among the most aggressive and lethal cancers but has limited treatment options,” said Robert Forrester, Verastem President and Chief Executive Officer. “We are pleased that the FDA recognizes the significant unmet medical need in mesothelioma. We previously received orphan medicinal product status for defactinib in Europe and these two designations are an important component of our development strategy.”

Verastem recently outlined details of the registration-directed clinical study of defactinib in patients with malignant pleural mesothelioma. This study is designed as a double-blind, placebo-controlled trial with an expected enrollment of approximately 350-400 patients at clinical sites in 11 countries.

“We are in discussions with the regulatory agencies and clinical investigators worldwide,” said Dr. Joanna Horobin, Verastem Chief Medical Officer. “We recently held our investigator meetings for the physicians conducting the trial in the US and Australia and we are on track to begin enrolling patients in the third quarter. We plan to open sites worldwide on a rolling basis as we clear regulatory and clinical review in each country.”

Orphan drug designation is granted by the FDA Office of Orphan Drug Products to novel drugs or biologics that treat a rare disease or condition affecting fewer than 200,000 patients in the U.S. The designation provides eligibility for a seven-year period of market exclusivity in the U.S. after product approval, FDA assistance in clinical trial design, and an exemption from FDA user fees.

“Cancer stem cells play a central role in treatment resistance in many types of cancers,” said Christoph Westphal, M.D., Ph.D., Verastem Executive Chairman. “We believe new treatment options targeting cancer stem cells will be critical to achieve a durable clinical benefit for patients. This designation will provide us with many benefits as we pursue the development of defactinib for the treatment of mesothelioma.”

In addition to mesothelioma, Verastem recently announced the completion of the Phase 1 stage and initial data from an ongoing Phase 1/1b study of defactinib in combination with weekly paclitaxel for patients with ovarian cancer. Verastem also expects to initiate a Phase 1 study in Japan, and a Phase 2 trial in KRAS-mutated Non-Small Cell Lung Cancer, for defactinib during the third quarter of 2013.

About Verastem, Inc.

Verastem, Inc. (NASDAQ: VSTM) is discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells. Cancer stem cells are an underlying cause of tumor recurrence and metastasis. Verastem is developing small molecule inhibitors of signaling pathways that are critical to cancer stem cell survival and proliferation: FAK, PI3K/mTOR and Wnt. For more information, please visit www.verastem.com.

Forward-looking statements:

This press release includes forward-looking statements about the Company’s strategy, future plans and prospects, including statements regarding the development of the Company’s compounds, including VS-6063, and the Company’s FAK and diagnostic programs generally, the timeline for clinical development and regulatory approval of the Company’s compounds, the structure of the Company’s planned clinical trials and the Company’s ability to fund operations. The words “anticipate,” “appear,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “continue,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement. Applicable risks and uncertainties include the risks that the preclinical testing of the Company’s compounds and preliminary data from clinical trials may not be predictive of the results or success of later clinical trials, that the Company will be unable to successfully complete the clinical development of its compounds, including VS-6063, that the development of the Company’s compounds will take longer or cost more than planned, and that the Company’s compounds will not receive regulatory approval or become commercially successful products. Other risks and uncertainties include those identified under the heading “Risk Factors” in the Company’s Annual Report on Form 10-K for the year ended December 31, 2012 and in any subsequent SEC filings. The forward-looking statements contained in this presentation reflect the Company’s current views with respect to future events, and the Company does not undertake and specifically disclaims any obligation to update any forward-looking statements.

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