Verastem Receives Orphan Medicinal Product Designation from the European Commission for VS-6063 in Mesothelioma

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 13, 2013-- Verastem, Inc., (NASDAQ: VSTM) focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, announced that VS-6063 has received orphan medicinal product designation from the European Commission for use in mesothelioma. The designation is to encourage the development of drugs which may provide significant benefit to patients suffering from rare diseases.

“We are pleased that the EMA recognizes the significant unmet medical need in mesothelioma,” said Christoph Westphal, M.D., Ph.D., Verastem Chairman and Chief Executive Officer. “This orphan drug designation provides us with a number of benefits in the development of VS-6063.”

VS-6063 is an orally-available, small molecule inhibitor of focal adhesion kinase (FAK). Research on the FAK signaling pathway has revealed a critical role for cancer stem cell survival and disease progression.

“Mesothelioma is a devastating disease with limited treatment options,” said Dr. Joanna Horobin, Verastem Chief Medical Officer. “We are working with investigators throughout Europe and internationally to bring a new treatment option for these patients.”

A biomarker test is being developed in conjunction with LabCorp (NYSE: LH) to identify a subgroup of mesothelioma patients low in a marker called Merlin. Approximately 40-50% of mesothelioma patients lack Merlin. Studies by Verastem and others have shown that Merlin-low mesothelioma cells and tumors appear to be particularly sensitive to FAK inhibition. Verastem’s clinical study is designed as an adaptive, double-blind, placebo-controlled trial to evaluate the effect of VS-6063 in both the overall patient population and also those whose tumors are Merlin-low.

“We are in discussions with regulatory agencies worldwide,” said Robert Forrester, Verastem President and Chief Operating Officer. “We plan to start the randomized, double-blind, placebo controlled trial of VS-6063 in mesothelioma later this summer.”

Under EMA guidelines, Orphan Medicinal Product Designation provides up to 10 years of potential market exclusivity if the product candidate is approved for marketing in the European Union and the orphan designation is maintained. Orphan status also permits EMA assistance in optimizing the candidate's clinical development through participation in designing the clinical protocol and preparing the marketing application. Additionally, a drug candidate designated by the EMA as an Orphan Medicinal Product may qualify for a reduction in regulatory fees as well as a European Union-funded research grant.

In addition to the upcoming mesothelioma study, VS-6063 is currently being evaluated in a Phase 1/1b trial in combination with paclitaxel in patients with ovarian cancer.

Verastem has multiple programs targeting cancer stem cells in or entering clinical development in 2013. FAK inhibitor VS-4718 has received allowance from the FDA to initiate a Phase 1 trial in advanced solid tumors and the dual PI3K/mTOR inhibitor VS-5584 is currently in IND-enabling studies and is expected to enter clinical development in the second half 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, VS-4718 and VS-5584, and the Company’s FAK, PI3K/mTOR and diagnostic programs generally, the timeline for clinical development and regulatory approval of the Company’s compounds including the impact of and any potential benefits from Orphan Medicinal Product Designation, and the structure of the Company’s planned clinical trials and estimates of 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 may not be predictive of the success of later clinical trials, that the Company will be unable to successfully complete the clinical development of its compounds, including VS-6063, VS-4718 and VS-5584, 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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