Verastem Initiates Trial of Defactinib in Japan

September 24, 2013

Study Represents First-in-Asia Use of Lead FAK Inhibitor to Target Cancer Stem Cells

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 24, 2013-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today announced the initiation of a Phase 1 trial of defactinib (VS-6063), a potent inhibitor of focal adhesion kinase (FAK), in Japanese patients with advanced solid tumors. FAK is a pathway critical for the growth and survival of cancer stem cells and the study marks the launch of Verastem's development efforts in Japan.

The Phase 1 open-label, dose-escalation study, which will be conducted at Kinki University in Osaka, is designed to assess the safety, pharmacokinetics and pharmacodynamics of single agent defactinib. The study will enroll 12-18 patients. Pending a successful outcome to the Phase 1 study, Verastem may add additional clinical sites in Japan to the recently initiated COMMAND trial, a global registration-directed study of defactinib in patients with malignant pleural mesothelioma, and may pursue additional development efforts in Japan.

"Initiation of this trial is another important clinical milestone for Verastem and for defactinib, a drug candidate which is now being studied at sites in 12 countries over five continents," said Dr. Joanna Horobin, Verastem Chief Medical Officer. "In the US Phase 1 study, defactinib demonstrated good tolerability, as well as early signs of activity. With this study we hope to take the first steps toward a path to registration in Japan in parallel to our efforts in Europe and the US."

"The simultaneous Japanese development of defactinib with the ongoing efforts in the United States and Europe is highly exciting," said Professor Kazuhiko Nakagawa, M.D., Ph.D., Director, Department of Medical Oncology, Kinki University Faculty of Medicine and Coordinating Investigator for defactinib development in Japan. "At Kinki University, we are dedicated to accelerating the development of novel compounds such as defactinib that have the potential to make a significant difference in the treatment of cancer for our patients in Japan."

Historically, the initiation of development in Japan for most drugs has been delayed in comparison to efforts in the US and EU. Verastem is pursuing a rapid expansion of the geographical development of defactinib in an attempt to rapidly make defactinib available for patients worldwide.

"We are pleased to take part in this novel Phase 1 trial of defactinib that targets cancer stem cells," said Toshio Shimizu, M.D., Principal Investigator of the Phase 1 study. "Led by scientific breakthroughs from Professor Robert Weinberg on the role of focal adhesion kinase in cancer stem cells, Verastem is taking a new approach to the treatment of cancer in an attempt to address the underlying cause of disease progression and tumor recurrence."

"With a dramatic rise in the incidence of mesothelioma in Japan, it is imperative that new treatments with the potential to address this deadly disease are developed in parallel with other major markets," said Christoph Westphal, M.D., Ph.D., Verastem Executive Chairman. "Initiation of this study in Japan serves to underscore our passion for carrying out Verastem's mission, which is to deliver new medicines that will make a meaningful difference in the lives of cancer patients around the world."

About Defactinib

Defactinib (VS-6063) is an oral compound designed to target cancer stem cells through the potent inhibition of focal adhesion kinase (FAK). Cancer stem cells are an underlying cause of tumor resistance to chemotherapy, recurrence and ultimate disease progression. Research by Robert Weinberg, Ph.D., scientific cofounder and chair of Verastem's Scientific Advisory Board, and Verastem has demonstrated that the FAK pathway is critical for the growth and survival of cancer stem cells. Defactinib is currently being studied in the registration-directed COMMAND trial in mesothelioma, a Phase 1/1b study in ovarian cancer and a Phase 1 study in Japan. A Phase 2 trial in KRAS-mutated Non-Small Cell Lung Cancer is expected to begin in the third quarter of 2013. Defactinib has been granted orphan drug designation in the U.S. and E.U. for use in mesothelioma.

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, or defactinib, and the Company's FAK and diagnostic programs generally, the timeline for clinical development and regulatory approval of the Company's compounds, the expected timing for the reporting of data from ongoing trials, the structure of the Company's planned or pending 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 and preliminary data from clinical trials may not be predictive of the results or success of later clinical trials, that data may not be available when we expect it to be, that the Company will be unable to successfully complete the clinical development of its compounds, including defactinib, 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.

Source: Verastem, Inc.

Verastem, Inc.
Brian Sullivan, 617-252-9314
bsullivan@verastem.com