Verastem Reports Data from Phase 1 Study of VS-6063 (defactinib) in Japanese Patients

March 5, 2014

– Well Tolerated; Meets Study Objectives –

– Results Facilitate Potential Parallel Development of VS-6063 (defactinib) in Japan –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 5, 2014-- Verastem, Inc. (NASDAQ: VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today presented preliminary data from an ongoing First-in-Asia Phase 1 trial of VS-6063 in Japanese patients with advanced solid tumors, including one patient with mesothelioma. The Phase 1 study assessed the safety and pharmacokinetics of single agent VS-6063.

“We are encouraged that the safety and pharmacokinetic profile observed in Japanese patients in this Phase 1 study is consistent with that seen in the US Phase 1 study,” said Dr. Joanna Horobin, Verastem Chief Medical Officer. “With these data in hand, we are speaking with the Japanese regulatory authorities about the potential for opening COMMAND trial sites in Japan later this year.”

Verastem’s ongoing COMMAND trial is a randomized, double-blind, placebo controlled registration-directed study which is evaluating VS-6063 in patients with mesothelioma and is currently accruing patients in 8 countries.

The Japanese Phase 1 is an open-label, dose-escalation study that enrolled nine subjects who received single-agent VS-6063 (200, 400 or 600mg; n=3 in each dose cohort) BID. The study results demonstrated that VS-6063 was well tolerated at all dose levels. There were no serious adverse events or evidence of dose-limiting toxicity. Pharmacokinetic results from the recommended Phase 2 dose of 400mg BID were consistent with previously reported data in non-Japanese subjects. These safety and pharmacokinetic results support advancing the VS-6063 development program in Japanese patients.

“We have now completed full enrollment and VS-6063 was well tolerated at each dose level,” said Toshio Shimizu, M.D., Ph.D., Assistant Professor, Phase 1 Clinical Trials Program, Department of Medical Oncology, Kinki University Faculty of Medicine and Principal Investigator of the study. “The study was open to patients with all solid tumors and one of the patients has mesothelioma. This patient is enrolled in the 400mg BID dose cohort and is responding well to treatment, continues on trial and is currently in cycle 7 of treatment. At this point their disease remains stable and we have seen an improvement in clinical symptoms. These data are encouraging and we hope to continue development of VS-6063 in Japan in parallel with the United States and Europe.”

The details of the presentation and a downloadable link to the presentation are as follows:

Conference: 12th International Congress on Targeted Anticancer Therapies
Date: March 5, 2014
Location: Capitol Hilton in Washington, D.C.
Abstract Title: Addressing the Drug Lag in Japan: A Phase 1 Study of Defactinib in Japanese Subjects to Facilitate Multiregional Clinical Trials
Abstract Code: P6.1

About VS-6063

VS-6063 (defactinib) is an orally available 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. VS-6063 is currently being studied in the registration-directed COMMAND trial in mesothelioma (www.COMMANDmeso.com), a Phase 1/1b study in combination with paclitaxel for patients with ovarian cancer, a Phase 1 study in Japan in patients with advanced solid tumors and a Phase 2 trial in patients with Kras-mutated non-small cell lung cancer. VS-6063 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 inhibition program, the timeline for clinical development and regulatory approval of the Company’s compounds, including the potential for opening COMMAND trial sites in Japan, the expected timing for the reporting of data from ongoing trials, and the structure of the Company’s planned or pending clinical trials, and potential indications for clinical development. 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 ongoing or later clinical trials, that

data may not be available when we expect it to be, that the Japanese regulatory authorities will not approve the initiation of the COMMAND trial, 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.

Source: Verastem

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