Verastem Successfully Completes Phase 1 Stage of Combination Trial of Cancer Stem Cell Inhibitor VS-6063 and Paclitaxel

June 27, 2013

--Four of six patients continuing on study and significant reduction in CA-125 observed--

--Phase 1b portion of the study is now open for enrollment--

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 27, 2013-- Verastem, Inc., (NASDAQ: VSTM) focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, announced the completion of the dose escalation stage of VS-6063 and paclitaxel in a Phase 1/1b trial in patients with ovarian cancer.

The dose escalation reached the anticipated dose of 400mg BID in combination with weekly paclitaxel without any dose-limiting toxicities. 400mg BID is the recommended Phase 2 dose for VS-6063 as a single agent. In total, 6 patients with ovarian cancer have been treated in the first stage of the Phase 1/1b trial and four patients are continuing on study. A significant CA-125 reduction was observed in 3 patients. CA-125 is a marker that becomes elevated with disease progression in ovarian cancer.

“The combination of VS-6063 and weekly paclitaxel has been well tolerated, with no unexpected toxicity, and no worsening of the well understood side effects of paclitaxel," said Principal Investigator Manish Patel, M.D., Florida Cancer Specialists. "The observation that 3 of the 4 patients continuing on study have had a significant reduction in the CA-125 level is encouraging.”

“The initial results from this trial are promising,” said Dr. Joanna Horobin, Verastem Chief Medical Officer. “Importantly, the data from this study supports the use the cancer stem cell-targeting agent VS-6063 in combination with paclitaxel. We can now clinically test a strategy to target both the bulk tumor cells, and the cancer stem cells, simultaneously. It is our belief that to adequately treat many cancers it will be necessary to kill both cell populations in a tumor.”

VS-6063 targets cancer stem cells through potent inhibition of focal adhesion kinase (FAK). Research by Verastem and scientific cofounder and chair of the Scientific Advisory Board, Robert Weinberg, Ph.D., has demonstrated in preclinical models of multiple tumor types that the FAK pathway is critical for the growth and survival of cancer stem cells. Cancer stem cells are an underlying cause of tumor resistance to chemotherapy, recurrence and ultimate disease progression.

“We are very pleased with the initial results of the study and the potential of this combination for patients,” said Robert Forrester, Verastem President and Chief Operating Officer. “With the completion of the dose escalation stage we are now initiating an expansion cohort of patients with ovarian cancer to further evaluate the activity of the combination therapy. Importantly, the ability to combine VS-6063 with paclitaxel provides an opportunity to explore multiple additional indications where the tumors are driven by cancer stem cells and paclitaxel is the standard of care.”

The expansion stage of the trial will evaluate the single agent pharmacodynamic activity of VS-6063 on biomarkers and cancer stem cells in paired tumor biopsies collected prior to the addition of weekly paclitaxel. Anti-tumor activity will then be assessed by Response Evaluation Criteria for Solid Tumors (RECIST) and safety and pharmacokinetics will be monitored. Up to 15 additional patients will be enrolled in this second stage of the study at three U.S. locations.

“Our mission is to develop new medicines that target cancer stem cells in order to provide a meaningful improvement in cancer care,” said Christoph Westphal, M.D., Ph.D., Verastem Chairman and Chief Executive Officer. “In addition to the expansion study in ovarian cancer, we have multiple candidates in development. We recently began recruitment in a Phase 1 study of our second cancer stem cell selective agent, VS-4718, in patients with advanced cancers and we are shortly initiating a potentially pivotal, multinational, randomized, double-blind and placebo controlled study of VS-6063 in mesothelioma. The results from the initial dose escalation of VS-6063 and weekly paclitaxel are promising and we will continue to drive our candidates forward to develop new treatment options for our patients.”

For more information on the trial, please visit: http://www.clinicaltrials.gov/ct2/show/NCT01778803.

Upcoming events:

Verastem Annual Research and Development Day

Date: July 11, 2013
Time: 12:30pm to 3:30pm
Place: Harvard Club, 35 West 44th Street, New York, NY
RSVP: bsullivan@verastem.com
Conference Call Dial-in: 866-515-2907
Conference Call Passcode: 60920106
Webcast: http://phx.corporate-ir.net/phoenix.zhtml?c=250749&p=irol-EventDetails&EventId=4979610

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 and the structure of the Company's planned clinical trials. 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.

Source: Verastem, Inc.

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