Verastem Announces Oral Presentation of Data Supporting the Preferential Targeting of Ovarian Cancer Stem Cells at the Society of Gynecologic Oncology's 2016 Annual Meeting on Women's Cancer

March 22, 2016

BOSTON--(BUSINESS WIRE)--Mar. 22, 2016-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer, today announced the presentation of scientific data at the Society of Gynecologic Oncology's 2016 Annual Meeting on Women's Cancer being held March 19-22, 2016 in San Diego, CA.

"The data presented today at SGO 2016 are important because they provide further scientific evidence that chemotherapy can lead to an increase in ovarian cancer stem cells (CSCs), making the tumor more aggressive and resistant to further treatment," said Dr. Jonathan Pachter, Verastem Head of Research. "At Verastem, we believe that our compounds in development may be especially beneficial as therapeutics when used in combination with other agents, including current and emerging standard of care treatments and immunotherapies, and have the potential to create a more durable clinical response. We look forward to the initiation of a Phase 1/1b clinical trial of the combination of VS-6063 and avelumab, in collaboration with Pfizer and Merck KGaA, for patients with ovarian cancer in the second half of this year."

Verastem, and its collaborators, are presenting these scientific data in support of Verastem's development programs which utilize a multi-faceted approach to treat cancer by reducing cancer stem cells, enhancing anti-tumor immunity, and modulating the local tumor microenvironment. The Company's most advanced clinical product candidates are the Focal Adhesion Kinase inhibitors, VS-6063 and VS-4718, and the dual PI3K/mTOR inhibitor, VS-5584. Research on the FAK and PI3K/mTOR signaling pathways has revealed critical roles for each in cancer stem cell survival and disease progression.

Details for the SGO presentation are as follows:

Oral Presentation

Title: Standard chemotherapy for ovarian cancer increases expression of cancer stem cell biomarkers which is predictive of survival

Session: Scientific Plenary IX: Ovary

Date and time: Tuesday, March 22, 2016 at 8:30 - 10:05 AM

Location: Hall A

Summary: In ovarian cancer, certain molecular mediators are thought to possess CSC characteristics and the presence of these mediators, which is linked to earlier recurrence and shorter survival, is possibly brought about by chemotherapy. The aim of this study was to explore the effect of chemotherapy on ovarian cancer stem-like mediators and to determine if there was a relationship to survival. Researchers obtained matched pre- and post-chemotherapy tumor specimens from stage IIIC/IV ovarian cancer patients (n=22) who all underwent neoadjuvant chemotherapy with interval debulking surgery. Samples were then analyzed for expression of 27 CSC markers. CSC markers were then validated in tumorsphere model and *in vivo* tumor initiating studies.

All 27 CSC markers demonstrated a mean increase in gene expression after exposure to chemotherapy. A 3-fold or greater increase in gene expression after exposure to chemotherapy was seen in 8 of 27 (30%) markers: ABCG2, ALDH1A1, CTGF, DPP4, MYC, CD133, SOX2, and POSTN. Three markers demonstrated a significant fold increase that correlated with platinum resistance: POSTN (4.1-fold), ALDH1A1 (5-fold), and SOX2 (14.5-fold). When implanted into immunocompromised mice, SOX2(hi) cells exhibited significantly higher levels of tumorsphere forming potential than SOX2(lo) cells and were more tumorigenic. High gene expression in these 3 markers demonstrated shorter progression free survival, compared to low expression. These results support the further investigation of directed agents to inhibit these CSC markers to potentially extend survival for patients with ovarian cancer.

A copy of the oral presentation will be available at http://bit.ly/R3M6wc

About Verastem, Inc.

Verastem, Inc. (NASDAQ:VSTM) is a biopharmaceutical company focused on discovering and developing drugs to improve outcomes for patients with cancer. Our product candidates utilize a multi-faceted approach to treat cancer by reducing cancer stem cells, enhancing anti-tumor immunity, and modulating the local tumor microenvironment. Our most advanced clinical product candidates are the Focal Adhesion Kinase inhibitors, VS-6063 and VS-4718, and the dual PI3K/mTOR inhibitor, VS-5584. For more information, please visit www.verastem.com.

Verastem forward-looking statements notice:

This press release includes forward-looking statements about Verastem's strategy, future plans and prospects, including statements regarding the development and activity of Verastem's product candidates, VS-6063, VS-4718 and VS-5584, and Verastem's FAK, PI3K/mTOR and diagnostics programs generally, the utility of FAK inhibitors for the treatment of cancer, the timeline for clinical development and regulatory approval of our product candidates, the structure of our planned or pending clinical trials, our rights to develop or commercialize our product candidates and our ability to finance contemplated development activities and fund operations for a specified period. 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 Verastem's product candidates and preliminary or interim 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 enrollment of clinical trials may take longer than expected, that our product candidates will cause unexpected safety events, that Verastem will be unable to successfully initiate or complete the clinical development of its product candidates, that the development of Verastem's product candidates will take longer or cost more than planned, and that Verastem's product candidates will not receive regulatory approval or become commercially successful products. Other risks and uncertainties include those identified under the heading "Risk Factors" in Verastem's Annual Report on Form 10-K for the year ended December 31, 2015 and in any subsequent SEC filings. The forward-looking statements contained in this press release reflect Verastem's current views with respect to future events, and Verastem does not undertake and specifically disclaims any obligation to update any forward-looking statements.

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