



Verastem Presents Data at the 2014 San Antonio Breast Cancer Symposium

December 11, 2014

BOSTON--(BUSINESS WIRE)--Dec. 11, 2014-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today announced a poster presentation at the 2014 San Antonio Breast Cancer Symposium (SABCS) taking place December 9 - 13, 2014.

"The clinical relevance of cancer stem cells (CSCs) has perhaps been best documented in breast cancer," said Jonathan Pachter, Ph.D., Verastem Head of Research. "Our new data demonstrate the functional relevance of the effective knock down of cancer stem cells by our FAK inhibitors, VS-6063 and VS-4718, in preclinical models of triple negative breast cancer. As single agents in these models, both FAK inhibitors effectively extended anti-tumor activity after cessation of chemotherapy, and induced regression of metastatic lesions. This work builds upon the existing body of preclinical proof-of-concept data supporting the functional relevance of cancer stem cells and supports the planned clinical development of a FAK inhibitor for breast cancer."

Verastem is presenting these preclinical data in support of its VS-6063 and VS-4718 development programs targeting cancer stem cells through inhibition of the focal adhesion kinase (FAK) signaling pathway. Research on the FAK signaling pathway has revealed critical roles for each in CSC survival and disease progression. CSCs represent a subpopulation of cancer cells that have tumor-initiating capability, are particularly resistant to chemotherapy and can mediate tumor recurrence both locally and at metastatic sites.

A summary of the data presented at the conference is below:

Title: VS-6063 (defactinib) and VS-4718 Reduce Cancer Stem Cells in Models of Breast Cancer: Implications for Clinical Trials in the Neoadjuvant Setting

Abstract #: 1696

Session/Poster: Poster Session 6; Poster #P6-11-06

Date and Time: Friday, December 12, 2014; 3:15 – 5:00 PM CT

Summary: In breast cancer, CSCs can be identified by Aldehyde Dehydrogenase 1 (ALDH) or CD44-high/CD24-low expression. Neoadjuvant chemotherapy has been shown to lead to an increase in CSCs in locally advanced breast cancer. In addition, the presence of CSCs in residual axillary disease is associated with a significantly worse prognosis following neoadjuvant chemotherapy and surgery. Currently, there are no approved therapies that effectively target and kill CSCs. VS-6063 and VS-4718 are orally bioavailable small molecules that kill cancer stem cells through the inhibition of FAK, and both compounds have demonstrated preferential targeting of CSCs in preclinical models. In this study, the effects of VS-6063 and VS-4718 were evaluated in multiple breast cancer models.

The research results demonstrated that CSCs are readily detectable in primary breast cancers at surgery, and VS-6063 and VS-4718 diminish the CSC subpopulation in vitro, ex vivo and in xenograft models, as measured by a number of functional and biomarker assays. This critical subpopulation of CSCs is also detectable in residual tumor following neoadjuvant therapy. These results provide scientific rationale for the clinical development of VS-6063 in combination with chemotherapy for the neoadjuvant treatment of TNBC and support the clinical investigation of CSC-targeted agents such as VS-6063 or VS-4718 in the neoadjuvant setting to potentially delay time to relapse and improve patient outcomes.

A copy of the poster presentation is available at <http://bit.ly/R3M6wc>.

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 FAK activity 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 "Window of Opportunity" study in patients with mesothelioma prior to surgery, a Phase 1/1b study in combination with paclitaxel in patients with ovarian cancer, and a trial in patients with Kras-mutated non-small cell lung cancer. VS-6063 has been granted orphan drug designation in the U.S. and EU for use in mesothelioma.

About VS-4718

VS-4718 is an orally available compound designed to target cancer stem cells through the potent inhibition of focal adhesion kinase (FAK). VS-4718 is currently being studied in a Phase 1 dose escalation study in patients with advanced cancers.

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 and activity of the Company's product candidates, including VS-6063, or defactinib, and VS-4718, and the Company's FAK and PI3K/mTOR programs generally, the timeline for clinical development and regulatory approval of the Company's product candidates, including the

clinical development of VS-6063 for the neoadjuvant treatment of TNBC, and the structure of the Company's planned or pending 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 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 the Company will be unable to successfully complete the clinical development of its product candidates, including VS-6063 and VS-4718, that the development of the Company's product candidates will take longer or cost more than planned, and that the Company'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 the Company's Annual Report on Form 10-K for the year ended December 31, 2013 and in any subsequent SEC filings. The forward-looking statements contained in this press release 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, 781-292-4214

bsullivan@verastem.com