Verastem Announces the Presentation of Clinical Data at iMig 2016

May 3, 2016

- VS-6063 generally well tolerated; early signs of tumor reduction observed -

- Results from Cohort 2 continue encouraging observations from Cohort 1 for single-agent VS-6063 in chemotherapy-naïve patients as a neoadjuvant therapy -

BOSTON--(BUSINESS WIRE)--May 3, 2016-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer, today announced the presentation of clinical data by Professor Raphael Bueno, M.D., Chief of Thoracic Surgery, Brigham and Women's Hospital (BWH), Boston, and Principal Investigator of the ongoing Window of Opportunity study, in an oral presentation at the 13th International Mesothelioma Interest Group (iMig) Conference being held May 1-4, 2016 in Birmingham, UK.

The primary objective from this ongoing open-label, single-center, neoadjuvant Window of Opportunity study is to evaluate tolerability, along with biomarker and tumor volume response to Verastem's focal adhesion kinase (FAK) inhibitor VS-6063 (400mg BID) following either 12 days (Cohort 1) or 35 days (Cohort 2) of treatment in surgically-eligible patients with malignant pleural mesothelioma (MPM).

"Results from this early-stage study show that single-agent VS-6063 was generally well tolerated as a neoadjuvant therapy in chemotherapy-naïve patients," said Dr. Bueno. "In addition to evidence of a favorable safety profile, we also observed reductions in tumor size and favorable potential immunomodulatory effects. These Cohort 2 results continue the findings from Cohort 1 for the effects of single-agent VS-6063 in surgically-eligible mesothelioma patients."

Data analysis from Cohort 1 and Cohort 2 (n=20) showed that VS-6063 was generally well tolerated with no apparent negative impact on surgical outcome. Six of the twenty patients demonstrated an encouraging tumor reduction after brief treatment with VS-6063 (12 or 35 days). Using expression data, a positive correlation between tumor reduction and an ESTIMATE Score (Estimation of STromal and Immune cells in Malignant Tumours; Yoshihara et al., Nature Commun. 2013) were observed. Notably, increased CD8+ T-cell infiltration and significant decreases in IL-10, an immunosuppressive cytokine, were observed with VS-6063 treatment ($p = 0.0186$).

"These data are early but encouraging and support the growing body of research supporting the thesis that FAK inhibition produces favorable changes within the tumor microenvironment," said Gregory Berk, MD, Chief Medical Officer of Verastem.

Details for the presentation at iMig 2016 are as follows:

Oral Presentation

Title: Phase 2 Neoadjuvant Study of VS-6063, a FAK Inhibitor, in Subjects with Surgically Resectable Malignant Pleural Mesothelioma
Session: Mini Symposium 10: Novel Targets – Entering in the Clinic (Track: Early Phase Clinical Trials)
Date and time: Tuesday, May 3, 2016 at 14:45 BST

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

About Focal Adhesion Kinase

Focal Adhesion Kinase (FAK) is a non-receptor tyrosine kinase encoded by the PTK-2 gene that is involved in cellular adhesion and, in cancer, metastatic capability. VS-6063 (defactinib) and VS-4718 are orally available compounds that are potent inhibitors of FAK. VS-6063 and VS-4718 utilize a multi-faceted approach to treat cancer by reducing cancer stem cells, enhancing anti-tumor immunity, and modulating the local tumor microenvironment. VS-6063 and VS-4718 are currently being studied in multiple clinical trials for patients with cancer.

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 and VS-4718, and Verastem's FAK and diagnostics programs generally, the utility of FAK inhibitors for the treatment of cancer including in combination with other cancer treatments, 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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