Verastem Presents Clinical and Preclinical Data at the 16th World Conference on Lung Cancer

September 10, 2015

BOSTON--(BUSINESS WIRE)--Sep. 10, 2015-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today announced oral and poster presentations at the 16th World Conference on Lung Cancer (WCLC) which took place September 6 - 9, 2015, in Denver, CO.

Four oral presentations included results from a Phase 2 clinical trial of VS-6063 (defactinib) in advanced refractory KRAS-Mutated Non-Small Cell Lung Cancer (NSCLC) and presentations on preclinical studies of VS-6063 and VS-5584 in Small-Cell Lung Cancer (SCLC) and Mesothelioma. Two poster presentations detailed trial designs for Verastem's ongoing COMMAND trial in mesothelioma and Phase 1 dose-escalation study of VS-5584 in advanced solid tumors.

Verastem presented these clinical and preclinical data at WCLC in support of its programs targeting cancer stem cells (CSCs) through inhibition of the focal adhesion kinase (FAK; VS-6063) and PI3K/mTOR (VS-5584) signaling pathways. Research on the FAK and PI3K/mTOR signaling pathways has revealed critical roles for each in determining 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.

Details and links to the data presentations at WCLC are below:

Oral Presentations
Title: Phase 2 study of defactinib, VS-6063, a focal adhesion kinase (FAK) inhibitor, in patients with KRAS mutant non-small cell lung cancer (NSCLC)
Session info: Mini oral 30: New Kinase Targets; Track: Treatment of Advanced Diseases – NSCLC
Link to slides from the oral presentation: http://bit.ly/R3M6wc

Title: The cancer stem cell inhibitors VS-6063 (defactinib) and VS-5584 exhibit synergistic anticancer activity in pre-clinical models of mesothelioma
Session info: Oral session 40: Biology 1; Track: Thymoma, Mesothelioma and Other Thoracic Malignancies
Link to slides from the oral presentation: http://bit.ly/R3M6wc

Title: Targeting Cancer Stem Cells in Small Cell Lung Cancer
Session info: Mini oral 27: Biology and other issues in SCLC; Track: Small Cell Lung Cancer
Link to slides from the oral presentation: http://bit.ly/R3M6wc

Title: FAK inhibitor VS-6063 targets mesothelioma cancer stem cells: Rationale for maintenance therapy after conventional chemotherapy
Session info: Mini oral 38: Biology and Prognosis; Track: Thymoma, Mesothelioma and Other Thoracic Malignancies
Link to slides from the oral presentation: http://bit.ly/R3M6wc

Poster Presentations

Title: COMMAND: A Phase 2 randomized, double-blind, study of defactinib (VS-6063) as maintenance therapy in malignant pleural mesothelioma
Poster #: P3.08.014
Session info: Thymoma, Mesothelioma and Other Thoracic Malignancies – Mesothelioma
Link to copy of the poster presentation: http://bit.ly/R3M6wc

Title: A Phase 1 dose escalation study of VS-5584, a PI3K/mTOR inhibitor, administered with VS-6063, a focal adhesion kinase inhibitor, in mesothelioma
Poster #: P2.08.008
Session info: Thymoma, Mesothelioma and Other Thoracic Malignancies – Mesothelioma
Link to copy of the poster presentation: 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 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, a trial in patients with Kras-mutated non-small cell lung cancer and a trial evaluating the combination of VS-6063 and VS-5584 in patients with relapsed mesothelioma. VS-6063 has been granted orphan drug designation for use in mesothelioma in the U.S. and EU.

About VS-5584

VS-5584 is an orally available compound that has demonstrated potent and highly selective activity against class 1 PI3K enzymes and dual inhibitory actions against mTORC1 and mTORC2. In preclinical studies, VS-5584 has been shown to reduce the percentage of cancer stem cells and induce tumor regression in chemotherapy-resistant models. Verastem is currently conducting a dose escalation trial of VS-5584 in patients with advanced solid tumors as a single agent and a combination trial of VS-5584 and VS-6063 in patients with relapsed mesothelioma. VS-5584 has been granted orphan drug designation for use in mesothelioma in the U.S. and EU.

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 and PI3K/mTOR. 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, VS-6063and VS-5584, and the Company's FAK, PI3K/mTOR and diagnostics programs generally, the structure of our planned or pending clinical trials and additional planned studies. 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 enrollment of clinical trials may take longer than expected, that our product candidates will cause unexpected safety events, that the Company will be unable to successfully initiate or complete the clinical development of its product candidates, 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, 2014 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.

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