Verastem Presents New Data on Mesothelioma Programs at the 12th International Mesothelioma Interest Group Meeting

October 24, 2014

-Results from a Window of Opportunity study in surgically-eligible patients demonstrate a reduction in pFAK, cancer stem cell markers and tumor size following 12 days of treatment with VS-6063-

BOSTON--(BUSINESS WIRE)--Oct. 24, 2014-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, announced the presentation of clinical and preclinical data in oral presentation and discussion sessions at the 12th International Mesothelioma Interest Group (iMig) Conference being held October 22-24, 2014, at the Cape Town International Conference Centre in Cape Town, South Africa.

Professor Raphael Bueno, M.D., Chief of Thoracic Surgery, Brigham and Women's Hospital (BWH), Boston and Principal Investigator of Verastem's ongoing Window of Opportunity study presented preliminary clinical results in an oral presentation. The study is evaluating the biomarker response to VS-6063 (defactinib), an oral small molecule that targets cancer stem cells through the inhibition of focal adhesion kinase (FAK) in patients with resectable pleural mesothelioma. Biopsies of the tumor are taken before and after oral administration of VS-6063 (400mg BID) for 12 days. The study also measures tumor size using CT/PET scans before and after the 12-day administration of VS-6063. All study patients were enrolled at the BWH clinical site.

An analysis of the preliminary data (n=10) showed that VS-6063 reduced FAK activity (pFAK-Y397) by an average of 70% in the patients evaluated to date, and reduced the presence of cancer stem cell markers in the post-treatment biopsies in 5 out of the 7 patients with evaluable paired biopsies. Measurement of tumor size by CT/PET using RECIST modified for mesothelioma confirmed that there was no progression of disease while on the 12 day treatment with single agent VS-6063 in any of the 10 patients. Moreover, in 2 patients, tumor shrinkage consistent with a partial response (-30%, -49%) was noted. VS-6063 was well tolerated with no apparent negative impact on surgical outcome.

"These initial biopsy data show signs of activity against biomarkers of cancer stem cells as well as intriguing reductions in tumor size, after short-term exposure to single agent VS-6063," said Dr. Bueno. "To follow up on these encouraging results, the protocol is being amended to explore more extended dosing in 10-15 additional patients with mesothelioma prior to surgery. The window of opportunity before elective surgery provides a novel platform for the clinical evaluation of promising new agents for the treatment of this devastating disease."

"This study was designed to evaluate potential biomarkers of response to VS-6063 treatment in patients with pleural mesothelioma," said Dr. Joanna Horobin, Verastem Chief Medical Officer. "We are encouraged by the biomarker response, and intrigued by the tumor shrinkage observed after 12 days of VS-6063 administration. As we expand our clinical program with VS-6063 in mesothelioma into other patient settings, we are encouraged by these interesting signs of clinical activity."

In addition to the Window of Opportunity study, Verastem is conducting the registration-directed COMMAND study as a maintenance treatment immediately following successful front-line therapy with Alimta and cisplatin in patients with malignant pleural mesothelioma. Verastem anticipates starting a study of VS-6063 and VS-5584 in combination for patients with relapsed or progressive malignant pleural mesothelioma in early 2015.

Verastem also presented preclinical data at the conference demonstrating the ability of VS-6063 and the combination of VS-6063 and VS-5584 to target and kill cancer stem cells in models of mesothelioma. In addition, Professor Robert Weinberg, Ph.D., Whitehead Institute/Massachusetts Institute of Technology, Verastem co-founder and Chair of the Scientific Advisory Board, gave a keynote address to open the symposium entitled: "Cancer Stem Cells as Target Pathways." These presentations support the ongoing and planned clinical trials that Verastem is conducting in patients with mesothelioma.

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

Oral Presentations and Discussions

iMig Special Keynote Lecture: "Cancer Stem Cells as Target Pathways"
Presenter: Robert Weinberg, Ph.D., *Whitehead Institute/Massachusetts Institute of Technology, Verastem scientific cofounder and chair of the Scientific Advisory Board*
Date and time: Wednesday, October 22, 2014, 8:50 AM (local time)
Link to presentation: http://bit.ly/12otlcV

Presentation Title: "FAK Inhibitor VS-6063 (defactinib) Targets Mesothelioma Cancer Stem Cells which are Enriched by Standard of Care Chemotherapy"
Presenter: Paul Baas, M.D., Ph.D., *Department of Thoracic Oncology, The Netherlands Cancer Institute*
Date and time: Thursday, October 23, 2014, 3:30 PM (local time)
Link to presentation: http://bit.ly/12otlcV

Presentation Title: "Determination of Biomarker Response in a Phase II Window of Opportunity Study of Defactinib (VS-6063), a Focal Adhesion Kinase (FAK) Inhibitor, in Subjects with Resectable MPM"
Presenter: Raphael Bueno, M.D., *Chief, Division of Thoracic Surgery, Brigham & Women's Hospital*
Date and time: Thursday, October 23, 2014, 3:45 PM (local time)
Link to presentation: http://bit.ly/12otlcV

Presentation Title: "The Cancer Stem Cell Inhibitors VS-6063 (defactinib) and VS-5584 Exhibit Synergistic Anticancer Activity in Preclinical Models of Mesothelioma"
Presenter: Mitchell Keegan, Ph.D., *Vice President, Development, Verastem*
Date and time: Friday, October 24, 2014, 10:30 AM (local time)
Link to presentation: http://bit.ly/12otlcV

Poster Presentation

Presentation Title: "COMMAND: A Phase 2 Randomized, Double-Blind, Placebo-Controlled, Multicenter Study of Defactinib as Maintenance Therapy in Subjects with Malignant Pleural Mesothelioma which has not Progressed on at Least 4 cycles of Pemetrexed/Platinum Therapy"
Presenter: Mitchell Keegan, Ph.D., *Vice President, Development, Verastem*
Date and time: Thursday, October 23, 2014, 10:30AM (local time)
Link to poster: http://bit.ly/12otlcV

Several of the above-mentioned studies were selected to be featured in the "Best of iMig" series, which highlighted the most relevant studies from the conference. Video of the presentations can be found at http://bit.ly/12d3OU1

About Malignant Pleural Mesothelioma

Malignant pleural mesothelioma is an aggressive form of cancer that occurs in the mesothelium, the thin layer of tissue that covers the lungs. Mesothelioma is associated with exposure to asbestos in most cases. According to the World Health Organization, there are a total of 59,000 cases of mesothelioma worldwide each year. Most mesotheliomas begin as one or more nodules that progressively grow to form a solid coating of tumor surrounding the lung leading to eventual suffocation and death. A high percentage of mesotheliomas contain cancer stem cells which are generally resistant to the currently available treatment options for mesothelioma.

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-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 Phase 1 dose escalation trial of VS-5584 in patients with advanced solid tumors and lymphomas.

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, or defactinib, andVS-5584 and the Company's FAK inhibition program and PI3K/mTOR program generally, the timeline for clinical development and regulatory approval of the Company's compounds, the expected timing for the reporting of data from ongoing trials, 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 compounds and preliminary or interim data from clinical trials, including the Window of Opportunity study, 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 the Company will be unable to successfully complete the clinical development of its compounds, including VS-6063 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, 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.

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