Verastem Presents Updated Clinical Data from VS-6063 and Paclitaxel Combination Phase 1/1b Study in Patients with Ovarian Cancer at the 2014 ASCO Annual Meeting

June 2, 2014

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 2, 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 data in a poster presentation and discussion at the American Society of Clinical Oncology Annual Meeting being held May 30 – June 3, 2014, at McCormick Place in Chicago, IL.

Verastem presented interim data from the ongoing Phase 1/1b trial of its lead product candidate, VS-6063 (defactinib), an oral small molecule that targets cancer stem cells through the inhibition of focal adhesion kinase (FAK), in combination with paclitaxel in patients with ovarian cancer. The study, which has completed enrollment, is evaluating 22 patients at three sites in the US.

“These data continue to show that the combination of cancer stem cell-targeting agent VS-6063 and paclitaxel is well tolerated, with no unexpected toxicity or worsening of the well understood side effects of paclitaxel, and support the further study of this combination in the clinic,” said Dr. Joanna Horobin, Verastem Chief Medical Officer. “In addition to the demonstration of safety, we continue to see interesting signs of early clinical activity where 14 of 22 (64%) patients enrolled in this study achieved a best overall response of at least stable disease including two partial responses and two complete responses to date.”

“We believe that to change the way cancer is treated it will be necessary to kill both the cancer stem cells, and the bulk tumor cells, in order to improve patient outcomes for many types of cancer,” said Robert Forrester, Verastem President and Chief Executive Officer. “The ability to combine VS-6063 with paclitaxel provides an opportunity to explore additional indications where the tumors are driven by cancer stem cells and paclitaxel is the standard of care. We are encouraged by these initial data and will update on the progress of the 9 patients that remain on study later this year.”

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

Poster Presentation and Discussion

Date & Time: Monday, June 2, 2014, from 8:00 a.m. to 12:45 p.m. CT

Poster Title: Phase 1/1b Study of the FAK Inhibitor Defactinib (VS-6063) in Combination with Weekly Paclitaxel for Advanced Ovarian Cancer

Abstract Number: 5521

Session ID: Poster Highlights Session: Gynecologic Cancer

Location: E354b & E354a

Summary: Defactinib (VS-6063) is an oral small molecule that targets cancer stem cells through the inhibition of focal adhesion kinase (FAK). FAK is a protein kinase that is critical for cancer stem cell survival. Tumor initiation by cancer stem cells requires attachment, proliferation and survival which are all mediated by FAK. Standard of care (SOC) agents such as paclitaxel have been shown in preclinical models to enrich for chemoresistant and tumor initiating cancer stem cells, while preclinical studies with VS-6063 have shown that it can selectively target cancer stem cells and attenuates the enrichment of cancer stem cells typically seen with standard of care agents. This multicenter study evaluated the safety and efficacy of the combination of VS-6063 and weekly paclitaxel in advanced ovarian cancer.

The study results demonstrated that combination therapy was generally well tolerated with no dose limiting toxicities or exacerbation of paclitaxel related toxicities observed at either dose level (200mg or 400mg BID). The recommended Phase 2 dose was determined to be VS-6063 400mg BID with weekly paclitaxel (80 mg/m^2) administered on Day 1, 8 and 15 of a 28 day cycle. Interesting signs of clinical activity have been observed in this ongoing trial with nine patients remaining on study. To date, four patients have exhibited objective responses: Two complete responses and two partial responses. Treatment with VS-6063 resulted in substantial decreases in FAK expression in four of the five paired biopsies. The successful administration of VS-6063 with weekly paclitaxel may enable the utilization of this combination in other clinical settings where paclitaxel is used.

Other Poster Presentations

In addition, investigators presented posters describing the trial designs from two of Verastem’s other ongoing clinical studies: one for the registration-directed COMMAND study of VS-6063 for patients with malignant pleural mesothelioma, and the other for the Phase 2 study of VS-6063 for patients with non-small cell lung cancer.

Analyst Event and Webcast

The company hosted an analyst and investor event during ASCO where a scientific update on the COMMAND study and the rationale for targeting cancer stem cells in mesothelioma was discussed. Professor Dean Fennell, Ph.D., FRCP, Chair of Thoracic Oncology at the University of Leicester and Incoming President of the International Mesothelioma Interest Group was a guest speaker.

A replay of the event webcast can be accessed here or by visiting the Verastem website.

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 the FAK pathway 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 Phase 1/1b study in combination with paclitaxel for patients with ovarian cancer and a Phase 2 trial in patients with Kras-mutated non-small cell lung cancer. VS-6063 has been granted orphan drug designation in the U.S. and E.U. for use in mesothelioma.

About COMMAND

COMMAND is a registration-directed, double-blind, placebo-controlled trial of VS-6063 with Progression Free Survival (PFS) and Overall Survival (OS) as the primary endpoints. VS-6063 targets cancer stem cells. Cancer stem cells are an underlying cause of tumor progression and recurrence. The design of COMMAND allows the opportunity to enrich for patients with tumors low in the biomarker, merlin. Preclinical and early clinical research has demonstrated that low merlin levels may be predictive of increased effectiveness of FAK inhibitors such as VS-6063. The COMMAND study stratifies patients to evaluate the effect of VS-6063 in both the overall patient population and the subgroup of patients whose tumors are low in merlin.

COMMAND is expected to enroll approximately 350-400 patients at clinical sites in 12 countries, including the US, UK, Japan, Australia, Canada, South Africa, New Zealand and countries in mainland Europe. Eligible patients who had a partial response or stable disease following standard first-line therapy with platinum/pemetrexed will be stratified to merlin low or high and then randomized to receive either placebo or 400 mg of defactinib. For more information visit www.COMMANDmeso.com

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, and the Company's FAK inhibition program, 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, and potential indications for clinical development. 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 data from clinical trials may not be predictive of the results or success of ongoing or later clinical trials, including the ongoing Phase 1/1b ovarian cancer study, 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, 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 presentation 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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