Verastem Presents Results and Updated Development Plans at Annual Research and Development Day

July 10, 2014

-Lead cancer stem cell inhibitor VS-6063 continues to show promise in the clinic-

-An additional partial response reported since ASCO in the combination trial of VS-6063 and paclitaxel in ovarian cancer-

-64% stable disease or better as best clinical response to date, including five objective responses, in the ongoing combination trial of VS-6063 and paclitaxel in ovarian cancer-

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jul. 10, 2014-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today hosted its annual Research and Development Day at the Hudson Theatre in the Millennium Broadway Hotel in New York City.

“We continue to see promising clinical signals from the VS-6063 program,” said Dr. Joanna Horobin, Verastem Chief Medical Officer. “An additional partial response has been reported since our interim report at ASCO on the combination of VS-6063 and weekly paclitaxel in twenty-two patients with ovarian cancer. To date, best clinical response of at least stable disease has been observed in 14 of the 22 patients (64%). This comprises two complete responses, three partial responses and nine patients with stable disease, including four patients whose disease has been stable for 6 months or longer. Eight patients continue on study drug. Based on this encouraging activity, we are planning a controlled Phase 2 study in patients with platinum resistant ovarian cancer.”

Members of the Verastem leadership team provided in-depth reviews of the Company’s development programs targeting cancer stem cells with a focus on lead inhibitor VS-6063. VS-6063 targets cancer stem cells through the inhibition of focal adhesion kinase (FAK). The review of Verastem's clinical programs included a presentation of clinical and preclinical data obtained to date, updated development plans, guidance for key upcoming milestones and commentary by guest speakers.

“It is well known that in patients undergoing cancer treatment, tumors often become resistant to standard therapies resulting in disease progression. We believe that cancer stem cells are an underlying cause of this failure,” said Robert Forrester, Verastem President and Chief Executive Officer. “By targeting cancer stem cells, we want to change the way that cancer is treated. We have made significant progress in the past 12 months and have continued to further scientific understanding with all of our product candidates. Encouraged by these initial signs of clinical activity, we are adding additional studies to further test our hypothesis that the targeting of cancer stem cells may enable a more durable clinical response.”

Research and Development Day Program Highlights:

Focal Adhesion Kinase (FAK) Inhibition

- VS-6063
  - COMMAND (**C**ontrol **O**f **M**esothelioma with **MA**inte**N**ance **D**efactinib), a registration-directed, randomized, double-blind and placebo-controlled study in patients with malignant pleural mesothelioma
    - Open in 12 countries worldwide; expect to enroll 350-400 patients
    - Addition of Japanese sites to COMMAND to pursue simultaneous development in the US, EU, Japan and other major regions of mesothelioma incidence
    - Primary endpoints include Progression Free Survival (PFS) and Overall Survival (OS), with potential to seek accelerated approval on PFS
    - Interim analysis to define the primary patient population expected midyear 2015
  - Combination of VS-6063 with weekly paclitaxel in patients with ovarian cancer
    - 64% clinical best response of stable disease or better including two complete, and three partial, responses. Eight patients continue on study
    - Combination therapy was generally well-tolerated with no dose limiting toxicities
    - Treatment with VS-6063 resulted in decreased pFAK activity and a reduction in cancer stem cells in patient biopsies following 10 days of VS-6063 treatment
  - Mesothelioma “Window of Opportunity” study prior to surgery
    - Single agent VS-6063 for 12 days in patients with malignant pleural mesothelioma eligible for surgery
    - Data to be submitted to the International Mesothelioma Interest Group (iMIG) meeting in October 2014
  - Phase 2 study of VS-6063 in patients with Kras-mutated NSCLC
    - Single agent VS-6063 to evaluate potential clinical activity in this late-stage patient population
    - Expansion of the safety data base for a potential NDA filing for mesothelioma
    - An interim analysis is expected in H2 2014
  - Additional planned studies include:
    - *Ovarian cancer*

        - Phase 2, placebo-controlled study of VS-6063 and weekly paclitaxel in patients with platinum-resistant ovarian cancer
        - "Window of opportunity" study in patients with ovarian cancer who are undergoing surgical resection
    - *Triple negative breast cancer*
        - Phase 2, placebo-controlled study of VS-6063 added to standard of care neo-adjuvant chemotherapy, in patients with newly diagnosed triple negative breast cancer undergoing surgery
    - *Mesothelioma*
    - Phase 1, dose escalation trial of VS-5584 in combination with VS-6063 in patients with relapsed/refractory mesothelioma
- VS-4718
    - A Phase 1 dose escalation trial of VS-4718 in patients with advanced cancer is ongoing and interim data are expected in H2 2014

Dual PI3K/mTOR Inhibition

- VS-5584
    - A Phase 1 dose escalation trial of VS-5584 in patients with advanced cancer is ongoing and interim data are expected in H2 2014
    - Initiation of a Phase 1, dose escalation trial of VS-5584 in combination with VS-6063 in patients with relapsed/refractory mesothelioma

A replay of the webcast will be archived for 90 days following the presentation date.
Replay webcast: http://bit.ly/1vvh3sF
Replay Dial-in (U.S.): 888-799-6166
Replay Dial-in (International): 857-288-2550
Replay Passcode: 5946305

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 COMMAND

COMMAND is a registration-directed, double-blind, placebo-controlled trial of VS-6063 with PFS and 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 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 "Window of opportunity" study in patients with mesothelioma prior to surgery, 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 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 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, VS-4718 and VS-5584 and the Company's FAK inhibition program, PI3K/mTOR and diagnostics programs 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, the structure of the Company's planned or pending clinical trials, additional planned studies and potential indications for clinical development or 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 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, VS-4718 and VS-5584, that the development of the Company's compounds will take longer or cost more than planned, that the Company will be unable to start additional studies as 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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