Verastem Announces Initiation of COMMAND Study in Japan

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- *Japanese Investigative Sites Join International Trial of VS-6063 for Patients with Mesothelioma —*
- *Allowance by the PMDA Enables Verastem to Pursue Parallel Development in the Major Markets Worldwide –*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 12, 2014-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today announced the expansion of its ongoing COMMAND study to include clinical trial sites in Japan. COMMAND is a registration-directed trial evaluating the Company's lead candidate targeting cancer stem cells, VS-6063 (defactinib) a potent inhibitor of focal adhesion kinase (FAK), in patients with malignant pleural mesothelioma. Mesothelioma is a highly aggressive form of lung cancer and a high percentage of cases contain cancer stem cells. With the inclusion of Japan, COMMAND is now accruing patients and pursuing parallel clinical development in the major markets worldwide.

"The incidence of mesothelioma, among the most aggressive and lethal cancers, is rapidly increasing in Japan and the survival rate for these patients is extremely poor," said Professor Takashi Nakano, M.D., Ph.D., Chief Professor and Chairman, Department of Thoracic Oncology, Hyogo College of Medicine. "New treatment options for patients with mesothelioma are urgently needed."

"Typically the Japanese development of novel oncology agents is delayed compared to the rest of the world," said Professor Kazuhiko Nakagawa, M.D., Ph.D., Professor, Department of Medical Oncology, Kinki University Faculty of Medicine. "We were able to initiate and complete the Phase 1 assessment of VS-6063 in less than a year. By completing the trial so quickly, the ongoing COMMAND study can now include Japanese clinical sites in parallel with the other major countries participating in the trial."

Verastem recently reported the successful outcome of its Phase 1 trial of VS-6063 in Japanese patients with advanced solid tumors. The study results demonstrated that VS-6063 was well tolerated at all three dose levels tested. The data were consistent with the results from the US Phase 1 trial and there were no serious adverse events or evidence of dose-limiting toxicity. These results supported the Company's application to the Japanese PMDA for the initiation of clinical trial sites to evaluate VS-6063 in Japanese patients with mesothelioma.

"COMMAND is now open for patient enrollment at multiple sites in Japan," said Dr. Joanna Horobin, Verastem Chief Medical Officer. "An important element of our overall global development strategy for VS-6063 is to pursue a path toward registration in Japan in parallel with our development efforts worldwide. We are committed to developing new treatments that can potentially make a meaningful difference in the lives of patients suffering with this deadly disease."

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 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 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 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, 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.

Verastem, Inc.
Brian Sullivan (USA), 617-252-9314
bsullivan@verastem.com
or
Ambrose Lau (Japan), +81-3-5561-2915
verastemcommand@cosmopr.co.jp