



Verastem Issued Japanese Patent on Dual mTORC1/2 and PI3K Inhibitor VS-5584

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 4, 2014-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today announced that it has been issued a patent by the Japanese Patent Office (JPO) for the company's small molecule dual inhibitor of mTORC1/2 and PI3K VS-5584. The granted patent is titled "Pyrimidine Substituted Purine Compounds As Kinase(s) Inhibitors," and has claims covering the composition of matter for VS-5584 and VS-5584's ability to inhibit and regulate cellular metabolism, growth, and proliferation. VS-5584 is currently in a Phase 1 dose escalation study in patients with solid tumors and lymphomas.

"This latest patent milestone underscores Verastem's continued interest in pursuing oncology-based development programs in Japan," said Robert Forrester, Verastem President and Chief Executive Officer. "There is a significant unmet medical need in cancer worldwide. We are committed to pursuing the development of new therapeutic options targeting cancer stem cells concurrently in the major markets."

The PI3K/mTOR signaling pathway is a key regulator in cancer progression and the survival of cancer stem cells. VS-5584 is a highly potent dual inhibitor of mTORC1/2 and PI3K with relative equipotency against all four human Class I PI3K isoforms and the mTOR kinase. Verastem identified that the dual inhibition of both mTORC1/2 and PI3K preferentially kills cancer stem cells through the use of the company's in vitro cancer stem cell screening platform and multiple in vivo models of human cancer. In data presented at the 2013 AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics, VS-5584 decreased cancer stem cells across multiple models of triple negative breast cancer, small cell lung cancer and ovarian cancer in contrast to standard chemotherapy which increased the relative proportion of cancer stem cells in these models.

In addition to the patent issuance for VS-5584, Verastem is also pursuing development of lead focal adhesion kinase (FAK) inhibitor in Japan. VS-6063 is currently in a Phase 1 study for Japanese patients with advanced solid tumors. This study is designed to establish the safety, pharmacokinetics and pharmacodynamics of VS-6063 in Japanese patients with the goal of opening Japanese clinical trial sites later this year in support of the COMMAND study. COMMAND is a registration-directed trial of VS-6063 in patients with mesothelioma currently ongoing in 11 countries worldwide.

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 pathways. In preclinical studies, VS-5584 has been shown to reduce the percentage of cancer stem cells and induce tumor regression in taxane-resistant models. Verastem is currently conducting a Phase 1 dose escalation trial of VS-5584 in patients with advanced solid tumors and lymphomas.

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, a Phase 1 study in Japan in patients with advanced solid tumors 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 VS-5584 and the Company's FAK, mTOR/PI3K and diagnostic programs generally, the timeline for clinical development including projected enrollment of trials, 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 data from clinical trials may not be predictive of the results or success of pending or later clinical trials, that data may not be available when we expect it to be, that the Company will not be able to enroll a sufficient number of patients in the expected timeframe, 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, 2012 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. Verastem, Inc.

Verastem, Inc.

Brian Sullivan, 617-252-9314

bsullivan@verastem.com