



Verastem Announces New Research Published in the Journal Cell That Highlights the Potential of FAK Inhibition to Enhance the Efficacy of Anti-Tumor Immunotherapy

September 24, 2015

BOSTON--(BUSINESS WIRE)--Sep. 24, 2015-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today announced that researchers from the University of Edinburgh have published a study in the journal *Cell* which highlights the potential of FAK inhibition to enable the body's immune system to fight cancer.

The paper discusses results from preclinical research showing that focal adhesion kinase (FAK), a protein which is often overproduced in tumors, enables cancer cells to evade attack by the immune system. In this study, researchers discovered that FAK inhibition can modulate the balance of immune cells in the tumor enabling an immune response to destroy the cancer cells.

Dr. Alan Serrels, one of the lead authors, at the Edinburgh Cancer Research UK Centre at the University of Edinburgh, said: "FAK is hi-jacked by cancer cells to protect them from the immune system. This exciting research reveals that by blocking FAK, we've now found a promising new way to help the immune system recognise the cancer and fight it. FAK inhibitors are already in clinical trials and have generally been well tolerated, so could potentially be an excellent complement to existing immunotherapy treatments."

The research, which was carried out in mice with squamous cell carcinoma, showed complete T-cell mediated tumor regression when the mice were administered the FAK inhibitor. This research is the first to demonstrate that FAK inhibition increases the presence of cytotoxic T cells in the tumor and decreases the presence of immunosuppressive T regulatory cells.

"This is a ground-breaking paper which shows that FAK inhibitors can induce tumor regression through a T cell-mediated mechanism," said Jonathan Pachter, Ph.D., Verastem Head of Research and co-author of the study. "These early data are encouraging and provide important support for the thesis that FAK inhibitors such as defactinib may be useful in combination with immuno-oncology agents with the goal of yielding more durable responses for a greater number of cancer patients."

The research was funded by Cancer Research UK, European Research Council, and Medical Research Council.

The full press release from Cancer Research UK can be accessed here: <http://bit.ly/1gRLBC0>

The paper, titled "Nuclear FAK controls chemokine transcription, Tregs and evasion of anti-tumor immunity," can be accessed at <http://bit.ly/1KTb3mW>.

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 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, a trial in patients with KRAS-mutated non-small cell lung cancer and a trial evaluating the combination of VS-6063 and VS-5584 in patients with relapsed mesothelioma. VS-6063 has been granted orphan drug designation for use in mesothelioma in the U.S. and EU.

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 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 and PI3K/mTOR. 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 and activity of the Company's product candidates, VS-6063 and VS-4718, and the Company's FAK program generally, and the potential for combination of FAK inhibitors with immuno-oncology agents. 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 product candidates and preliminary or interim 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 our product candidates will cause unexpected safety events, that the Company will be unable to successfully initiate or complete the clinical development of its product candidates, that the development of the Company's product candidates will take longer or cost more than planned, and that the Company's product candidates 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, 2014 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.

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