Scientific Data on PYK2 Inhibition by VS-4718 in Multiple Myeloma Published in the Journal Blood

November 7, 2014

Study Supports Development of VS-4718 as a Dual FAK/PYK2 Inhibitor

Additional data to be presented at the upcoming American Society of Hematology Annual Meeting in December

BOSTON--(BUSINESS WIRE)--Nov. 7, 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 a paper, titled "PYK2 Promotes Tumor Progression in Multiple Myeloma," has been published in *Blood* (2014 Oct 23;124(17):2675-86), a peer-reviewed medical journal published by the American Society of Hematology.

"These data demonstrate that PYK2 is a promising therapeutic target in multiple myeloma," said Jonathan Pachter, Ph.D., Verastem Head of Research. "These results build upon prior scientific findings demonstrating the activity of dual FAK/PYK2 inhibitors across multiple types of cancer and support the ongoing clinical development of VS-4718."

The paper describes the finding that patients with multiple myeloma have a higher expression of proline-rich tyrosine kinase 2 (PYK2), a member of the focal adhesion kinase (FAK) family, compared to healthy individuals, and that PYK2 plays a tumor-promoting role in myeloma progression. The FAK family is composed of just two members, FAK and PYK2, which are highly homologous. In the published study, it was demonstrated that inhibition of PYK2 led to reduction of myeloma tumor growth *in vivo* as well as decreased cell proliferation, cell cycle progression, and adhesion ability *in vitro.* In contrast, overexpression of PYK2 was shown to promote the malignant phenotype, as evidenced by enhanced tumor growth and reduced survival. The paper further describes how administration of Verastem's FAK/PYK2 inhibitor, VS-4718, effectively inhibited myeloma cell growth in both *in vitro* and *in vivo* models.

"In addition to this work recently published by our collaborators at the Dana Farber Cancer Institute, we have been conducting further research and collaborating with scientific leaders to understand the potential of cancer stem cell inhibitors in hematological malignancies", continued Dr. Pachter. "We believe that inhibitors of FAK and PYK2, such as VS-4718, may be useful in the treatment of many types of cancer, particularly where there is minimal residual disease with enrichment of cancer stem cells following chemotherapy. We and our collaborators will be presenting additional research at the upcoming American Society of Hematology in December."

"VS-4718 is currently being evaluated in a Phase 1 dose escalation clinical trial in patients with advanced solid tumors," said Robert Forrester, President and Chief Executive Officer of Verastem. "A new Phase 1 trial of VS-4718 in hematological malignancies is currently planned to begin in the first quarter of 2015."

The full publication can be accessed online here.

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 solid tumors.

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-4718 and the Company's FAK inhibition and PI3K/mTOR programs generally, the timeline for clinical development of the Company's compounds, including the new Phase 1 trial of VS-4718 in hematological malignancies, 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 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 the Company will be unable to successfully complete the clinical development of its compounds, including VS-4718, 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 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.
Brian Sullivan, 781-292-4214
bsullivan@verastem.com