Washington University in St. Louis Initiates Clinical Study of Verastem's VS-6063 in Combination with Merck's Pembrolizumab and Gemcitabine in Pancreatic Cancer

January 6, 2016

BOSTON--(BUSINESS WIRE)--Jan. 6, 2016-- Verastem, Inc. (NASDAQ: VSTM), focused on discovering and developing drugs to treat cancer, today announced the initiation of a Phase 1 dose-escalation study at Washington University to evaluate Verastem's FAK inhibitor VS-6063 in combination with Merck's PD-1 inhibitor pembrolizumab and gemcitabine in patients with pancreatic cancer. This is the first of several combination clinical trials the Company expects to initiate this year.

This Phase 1 clinical trial is anticipated to enroll approximately 50 patients and is being conducted at the Washington University School of Medicine's Division of Oncology under the direction of Andrea Wang-Gillam, MD, PhD, Clinical Director of the Gastrointestinal Oncology Program.

"As recently published in *Cell,* preclinical studies strongly suggest that the inhibition of focal adhesion kinase combined with checkpoint inhibition may potentiate a more significant tumor immune response leading to both tumor shrinkage and durable disease control," said Dr. Wang-Gillam. "This trial is primarily designed to evaluate the safety of the combination and may also provide a greater understanding of how FAK inhibition in combination with immunotherapies could improve outcomes for patients with pancreatic cancer, one of the most deadly of all cancer types."

This clinical study is supported by a growing body of preclinical research suggesting that focal adhesion kinase (FAK) inhibition, when combined with PD-1 inhibitors, increases the anti-tumor activity of these immunotherapeutic agents. As published in the September 24th, 2015 issue of the journal *Cell*, and presented at the recent NCI/AACR/EORTC and SITC conferences, FAK inhibition has been shown to increase cytotoxic (CD8+) T cells in tumors, decrease T cell exhaustion, decrease immunosuppressive cell populations, enhance T cell killing of tumor cells, and create a generally more favorable tumor microenvironment, which allows for enhanced efficacy of immuno-oncology therapeutics.

In addition to other "cold" tumors like glioblastoma and prostate, pancreatic cancer is a tumor type in which immunotherapeutics have achieved limited clinical benefit, possibly due to the dense desmoplastic stroma and the presence of high numbers of immunosuppressive cells. Pre-clinical research has demonstrated that high stromal density prevents anti-cancer agents and T cells from entering pancreatic tumors thereby limiting efficacy. In addition, FAK inhibition has been shown to reduce stromal density and allow cytotoxic T cells to better penetrate the tumor and kill the cancer cells. Collectively, these data provide strong rationale for combining Verastem's FAK inhibitors with checkpoint inhibitors in the clinic for pancreatic cancer.

"This combination trial marks the launch of a new clinical development program at Verastem which is focused on advancing our FAK inhibition program in combination with immuno-oncology agents and other current and emerging standard of care treatments," said Robert Forrester, Verastem President and Chief Executive Officer. "We anticipate initiating additional clinical trials this year to evaluate our FAK inhibitors in combination with other treatments, and the data generated will further inform the continued development of our innovative anti-cancer therapeutics."

About Focal Adhesion Kinase

Focal Adhesion Kinase (FAK) is a non-receptor tyrosine kinase encoded by the PTK-2 gene that is involved in cellular adhesion and, in cancer, metastatic capability. VS-6063 (defactinib) and VS-4718 are orally available compounds designed to target cancer stem cells through the potent inhibition of 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 and VS-4718 are currently being studied in multiple clinical trials for their ability to improve patient survival through the targeting of cancer stem cells, potentiation of an immune response against cancer cells and reduction in the stromal density encapsulating a tumor.

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.

Verastem forward-looking statements notice:

This press release includes forward-looking statements about Verastem's strategy, future plans and prospects, including statements regarding the development and activity of Verastem's product candidates, VS-6063, VS-4718 and VS-5584, and Verastem's FAK, PI3K/mTOR and diagnostics programs generally, the utility of FAK inhibitors for the treatment of cancer, the timeline for clinical development and regulatory approval of our product candidates, the structure of our planned or pending clinical trials, our rights to develop or commercialize our product candidates and our ability to finance contemplated development activities and fund operations for a specified period. 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 Verastem'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 Verastem will be unable to successfully initiate or complete the clinical development of its product candidates, that the development of Verastem's product candidates will take longer or cost more than planned, and that Verastem'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 Verastem's Annual Report on Form 10-K for the year ended December 31, 2014, Verastem's Quarterly Report on Form 10-Q for the quarter ended September 30, 2015 and in any

subsequent SEC filings. The forward-looking statements contained in this press release reflect Verastem's current views with respect to future events, and Verastem does not undertake and specifically disclaims any obligation to update any forward-looking statements.

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Source: Verastem, Inc.

Verastem, Inc.
Brian Sullivan, 781-292-4214
bsullivan@verastem.com