Data on Verastem's Focal Adhesion Kinase Inhibitor Defactinib Presented at the 2017 American Association for Cancer Research Annual Meeting

April 2, 2017

BOSTON--(BUSINESS WIRE)--Apr. 2, 2017-- Verastem, Inc. (NASDAQ: VSTM), focused on discovering and developing drugs to treat cancer, today announced the oral presentation of data for its lead focal adhesion kinase (FAK) inhibitor, defactinib, by the Company's scientific collaborator David G. DeNardo, PhD, Assistant Professor of Medicine, Division of Oncology, Department of Immunology, Washington University School of Medicine in St. Louis, at the 2017 American Association for Cancer Research (AACR) annual meeting in Washington, DC.

In an oral presentation titled, "Reprogramming the tumor microenvironment to improve responses to therapy," Dr. DeNardo described data demonstrating that FAK inhibition can enable efficacy of PD-1 inhibition in preclinical models of pancreatic cancer that, like the clinical disease, are otherwise refractory to checkpoint inhibition. As described in Dr. DeNardo's presentation, a clinical trial is in progress, which combines Verastem's FAK inhibitor defactinib with Merck's PD-1 inhibitor pembrolizumab together with gemcitabine in patients with advanced pancreatic ductal adenocarcinoma (PDAC). Initial analysis of immune biomarkers from matched pairs of metastatic biopsies, taken either pre or post treatment, from patients with PDAC has been conducted in this study.

"Immunotherapeutic agents have shown little clinical benefit in pancreatic cancer and this is likely due, at least in part, to the presence of an immunosuppressive tumor microenvironment including a dense stroma which may prevent T cell entry into, or function within, tumor tissues," said Dr. DeNardo. "Our initial biomarker data, indicating an increase in activated proliferating cytotoxic T cells together with a reduction in tumor-associated macrophages (TAMs) appear promising. We are extremely interested to see whether these immune changes that may shift the balance from immunosuppressive to immunoreactive tumors, may translate into a clinical benefit for pancreatic cancer patients who have few effective treatment options."

Jonathan Pachter, PhD, Verastem's Chief Scientific Officer, added, "Pancreatic cancer has the highest mortality rate of all major cancers, and the disease is still considered largely incurable. The data presented today by Dr. DeNardo at AACR 2017 provide important support and rationale for the ongoing clinical study evaluating Verastem's lead FAK inhibitor defactinib in combination with pembrolizumab and gemcitabine in patients with pancreatic cancer."

The ongoing Phase 1 clinical trial is being conducted at the Washington University School of Medicine in St. Louis under the direction of Andrea Wang-Gillam, MD, PhD, Clinical Director of the Gastrointestinal Oncology Program with financial support from the Precision Medicine Research Associates and the BJH Foundation. The trial, which is expected to enroll approximately 50 patients, is currently completing its dose-escalation portion.

Details for the oral symposium presentation at AACR 2017 are:

Title: Reprogramming the Tumor Microenvironment to Facilitate Responses to Immunotherapy
Session: Major Symposium; SY14 – Myelomonocytic Cells and Stroma as Therapeutic Targets
Location: Room 206 – Level 2 Washington Convention Center
Date and time: Sunday, April 2, 2017 from 2:05-2:30pm ET

A copy of the oral presentation slides will be available here following the conclusion of Dr. DeNardo's presentation and a webcast will be available from AACR beginning on April 26, 2017.

About the Tumor Microenvironment

The tumor microenvironment encompasses multiple tumor and non-tumor cell populations and an extracellular matrix that support cancer cell survival. This includes immunosuppressive regulatory T cells, myeloid-derived suppressor cells, tumor-associated macrophages, cancer-associated fibroblasts and extracellular matrix proteins that can hamper the entry and therapeutic benefit of cytotoxic T cells and anti-cancer drugs. In addition to targeting the proliferative and survival signaling of cancer cells, Verastem's product candidates, including duvelisib and defactinib, also target the tumor microenvironment to potentially improve a patient's response to therapy.

About Defactinib

Defactinib is an investigational inhibitor of FAK, a non-receptor tyrosine kinase encoded by the PTK-2 gene that mediates oncogenic signaling in response to cellular adhesion and growth factors.[1] Based on the multi-faceted roles of FAK, defactinib is used to treat cancer through modulation of the tumor microenvironment, enhancement of anti-tumor immunity, and reduction of cancer stem cells.[2,3] Defactinib is currently being evaluated in combination with immunotherapy for the treatment of pancreatic cancer, ovarian cancer, non-small cell lung cancer, and mesothelioma, in three combination clinical trials with pembrolizumab or avelumab from Merck & Co. and Pfizer/Merck KGaA, respectively.[4,5,6] Information about these and additional clinical trials evaluating the safety and efficacy of defactinib can be found on www.clinicaltrials.gov.

About Verastem, Inc.

Verastem, Inc. (NASDAQ:VSTM) is a biopharmaceutical company focused on discovering and developing drugs to improve outcomes for patients with cancer. Verastem is currently developing duvelisib, a dual inhibitor of PI3K-delta and PI3K-gamma, which has successfully met its primary endpoint in a Phase 2 study in iNHL and is currently being evaluated in a Phase 3 clinical trial in patients with CLL. In addition, Verastem is developing the FAK inhibitor defactinib, which is currently being evaluated in three separate clinical collaborations in combination with immunotherapeutic agents for the

treatment of several different cancer types, including pancreatic cancer, ovarian cancer and non-small cell lung cancer, and mesothelioma. Verastem's product candidates seek to treat cancer by modulating the local tumor microenvironment, enhancing anti-tumor immunity and reducing cancer stem cells. 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 investigational product candidates, including duvelisib and defactinib (VS-6063), and Verastem's PI3K and FAK programs generally, the structure of our planned and pending clinical trials and the timeline and indications for clinical development, 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," "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 expected; that enrollment of clinical trials may take longer than expected; that our product candidates will cause unexpected safety events or result in an unmanageable safety profile as compared to their level of efficacy; 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; that Verastem may not have sufficient cash to fund its contemplated operations; that Verastem will not pursue or submit regulatory filings for its product candidates; and that Verastem's product candidates will not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients. 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, 2016 and in any subsequent filings with the U.S. Securities and Exchange Commission. The forward-looking statements contained in this press release reflect Verastem's views as of the date of this release, and Verastem does not undertake and specifically disclaims any obligation to update any forward-looking statements.

[1]Schaller MD and Parsons JT. Focal adhesion kinase: an integrin-linked protein tyrosine kinase. Trends Cell Biol. 1993 3: 258-62.

[2]Jiang H et al. Targeting focal adhesion kinase renders pancreatic cancers responsive to checkpoint immunotherapy. Nat Med 2016: Aug 22(8) 851-60.

[3]Sulzmaier FJ et al. FAK in cancer: mechanistic findings and clinical applications. Nature Rev Cancer. 2014 14: 598-610.

[4]www.clinicaltrials.gov, NCT02546531

[5]www.clinicaltrials.gov, NCT02943317

[6]www.clinicaltrials.gov, NCT02758587

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