Verastem Reports Second Quarter 2016 Financial Results

August 8, 2016

BOSTON--(BUSINESS WIRE)--Aug. 8, 2016-- Verastem, Inc. (NASDAQ: VSTM), focused on discovering and developing drugs to treat cancer, today reported financial results for the second quarter ended June 30, 2016, and also provided an overview of certain corporate developments.

"We continue to execute on the research and development of our two clinical-stage oncology programs targeting several high unmet need tumor types," said Robert Forrester, President and Chief Executive Officer of Verastem. "The scientific evidence of the importance of focal adhesion kinase in maintaining the tumor microenvironment that leads to immunosuppression and aggressive cancer continues to mount as described in the recent *Nature Medicine* publication from our collaborators at The Washington University in Saint Louis. Enrollment and dosing continues in the Phase 1 dose-escalation study evaluating our lead focal adhesion kinase inhibitor VS-6063 in combination with Merck's PD-1 inhibitor pembrolizumab and gemcitabine in patients with pancreatic cancer. We are looking forward to the commencement of a clinical collaboration trial evaluating VS-6063 in combination with Merck-KGaA and Pfizer's PD-L1 inhibitor avelumab in ovarian cancer during the second half of the year. We closed the quarter with a strong balance sheet totaling $92.9 million in cash, cash equivalents and short-term investments."

Second Quarter 2016 and Recent Highlights:

Focal Adhesion Kinase (FAK) Inhibition Program

- ***Published Preclinical Research in Nature Medicine*** – In July 2016, the Company announced the publication of preclinical research conducted by our scientific collaborator, David G. DeNardo, PhD, Assistant Professor of Medicine, Division of Oncology, Department of Immunology, Washington University School of Medicine in St. Louis. In the published study, Dr. DeNardo demonstrates that FAK inhibition decreases fibrosis and immunosuppressive cell populations in pancreatic ductal adenocarcinoma, rendering previously unresponsive tumors sensitive to chemo- and immunotherapy. These findings provide important support and rationale for the ongoing Phase 1 dose-escalation clinical studies evaluating Verastem's FAK inhibitors in combination with pembrolizumab and gemcitabine, and, gemcitabine and Abraxane® in patients with pancreatic cancer.

- ***Presented Clinical Data from the Window of Opportunity Study at iMig 2016*** – In May 2016, the Company announced results from the ongoing open-label, single-center, neoadjuvant Window of Opportunity study evaluating tolerability, along with biomarker and tumor volume response to VS-6063 (400mg BID) following either 12 days (Cohort 1) or 35 days (Cohort 2) of treatment in surgically-eligible patients with malignant pleural mesothelioma. Data analysis from Cohort 1 and Cohort 2 showed that VS-6063 was generally well tolerated with early signs of tumor reduction observed, with six of the twenty patients demonstrating an encouraging tumor reduction after brief treatment with VS-6063.
- ***Development of VS-6063 in Combination with Immunotherapy Continues in Pancreatic Cancer*** – Dosing continues in a Washington University-sponsored Phase 1 dose-escalation study evaluating VS-6063 in combination with pembrolizumab and gemcitabine in patients with pancreatic cancer. This is the first clinical trial to evaluate FAK inhibition in combination with an immuno-oncology agent.
- ***Development of VS-4718 Continues in Solid Tumors*** – Clinical testing of VS-4718 continues in both a Phase 1 single agent dose escalation study in patients with solid tumors and in a Phase 1/1b combination study with gemcitabine and Abraxane® for the treatment of patients with newly diagnosed pancreatic cancer.

Dual PI3K and mTORC1/2 Inhibition Program

- ***Recommended Phase 2 Dose of VS-5584*** – The maximum tolerated dose of single-agent VS-5584 has been reached in a Phase 1 study, and the recommended Phase 2 dose (RP2D) is being confirmed. Reductions in pharmacodynamic markers of PI3K and mTOR activity and clinical activity have been observed in several tumor types.

Corporate

- ***New Appointments to the Board of Directors*** – In June 2016, the Company announced that Michael Kauffman, MD, PhD, who has served as a director since November 2012, became Lead Director and Bruce J. Wendel joined the Board as an independent director. Mr. Wendel is an industry veteran with a long history of building companies and bringing oncology drugs to market having served in executive roles at Abraxis, American Pharmaceutical Partners, IVAX Corporation and Bristol-Myers Squibb. He currently serves as Chief Strategic Officer at Hepalink USA and as a director at ProMetic Life Sciences Inc.
- ***Gregory I. Berk, MD Named Chief Medical Officer*** – In April 2016, the Company announced the appointment of Gregory I. Berk, MD as Chief Medical Officer. Dr. Berk, a medical oncologist with 25 years of both industry and academic

experience, will be responsible for leading the Company's global clinical development strategy and clinical operations.

Second Quarter 2016 Financial Results

Net loss for the second quarter ended June 30, 2016 (2016 Quarter) was $8.6 million, or $0.23 per share, as compared to a net loss of $15.4 million, or $0.42 per share, for the second quarter ended June 30, 2015 (2015 Quarter). Net loss includes non-cash stock-based compensation expense of $1.7 million and $2.6 million for the 2016 Quarter and 2015 Quarter, respectively.

Research and development expense for the 2016 Quarter was $4.5 million compared to $11.0 million for the 2015 Quarter. The $6.5 million decrease from the 2015 Quarter to the 2016 Quarter was primarily related to a decrease of $4.9 million in contract research organization expense for outsourced biology, chemistry, development and clinical services, which includes our clinical trial costs, a decrease in personnel related costs of $1.2 million, a decrease of approximately $584,000 in stock-based compensation, and a net decrease of approximately $193,000 in travel, facilities and other costs. These decreases were partially offset by an increase of approximately $343,000 in consulting fees.

General and administrative expense for the 2016 Quarter was $4.2 million compared to $4.4 million for the 2015 Quarter. The decrease of approximately $200,000 from the 2015 Quarter to the 2016 Quarter primarily resulted from approximate decreases in stock-based compensation expense of $330,000 and $230,000 in consulting fees. These decreases were offset by a net increase of approximately $360,000 in personnel costs, professional fees, and other costs.

As of June 30, 2016, Verastem had cash, cash equivalents and investments of $92.9 million compared to $110.3 million as of December 31, 2015. Verastem used $6.7 million for operating activities during 2016 Quarter.

The number of outstanding common shares as of June 30, 2016, was 36,992,418.

Financial Guidance

Based on current operating plans, we expect to have sufficient cash, cash equivalents and short-term investments to fund our research and development programs and operations into 2018.

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 that are potent inhibitors of FAK. VS-6063 and VS-4718 utilize a multi-faceted approach to treat cancer by reducing cancer stem cells, enhancing anti-tumor immunity, and modulating the local tumor microenvironment. VS-6063 and VS-4718 are currently being studied in multiple clinical trials for patients with cancer.

About PI3K and mTOR

PI3K and mTOR are components of a central proliferative signaling pathway in multiple types of human cancer. 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. In preclinical studies, VS-5584 has been shown to reduce the percentage of cancer stem cells and induce tumor regression in chemotherapy-resistant models. Verastem is currently conducting a dose escalation trial of VS-5584 in patients with non-hodgkin's lymphoma and chronic lymphocytic leukemia.

About Verastem, Inc.

Verastem, Inc. (NASDAQ:VSTM) is a biopharmaceutical company focused on discovering and developing drugs to improve outcomes for patients with cancer. Our product candidates utilize a multi-faceted approach to treat cancer by reducing cancer stem cells, enhancing anti-tumor immunity, and modulating the local tumor microenvironment. Our most advanced clinical product candidates are the Focal Adhesion Kinase inhibitors, VS-6063 and VS-4718, and the dual PI3K/mTOR inhibitor, VS-5584. 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 structure of our planned and pending clinical trials and the timeline 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," "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, 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.

Verastem, Inc.

Unaudited Selected Consolidated Balance Sheets Information

(in thousands)

	June 30, 2016	December 31, 2015
Cash, cash equivalents and investments	$ 92,866	$ 110,258
Prepaid expenses and other current assets	667	585
Property and equipment, net	1,728	2,048
Other assets	162	203
Total assets	**$ 95,423**	**$ 113,094**
Accounts payable and accrued expenses	$ 5,925	$ 10,040
Other liabilities	430	585
Stockholders' equity	89,068	102,469
Total liabilities and stockholders' equity	**$ 95,423**	**$ 113,094**

Verastem, Inc.

Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended June 30,		Six months ended June 30,	
	2016	2015	2016	2015
Operating expenses:				
Research and development	$ 4,492	$ 11,045	$ 8,671	$ 21,573
General and administrative	4,217	4,417	8,472	9,131
Total operating expenses	8,709	15,462	17,143	30,704
Loss from operations	(8,709)	(15,462)	(17,143)	(30,704)
Interest income	140	85	280	147
Net loss	**$ (8,569)**	**$ (15,377)**	**$ (16,863)**	**$ (30,557)**
Net loss per share—basic and diluted	**$ (0.23)**	**$ (0.42)**	**$ (0.46)**	**$ (0.87)**
Weighted-average number of common shares used in net loss per share-basic and diluted	**36,992**	**36,522**	**36,983**	**34,931**

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