Verastem Reports First Quarter 2016 Financial Results

May 9, 2016

BOSTON--(BUSINESS WIRE)--May 9, 2016-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer, today reported financial results for the first quarter ended March 31, 2016, and also provided an overview of certain corporate developments.

"To date in 2016, Verastem has announced two new clinical collaborations with world-class organizations, including Merck KGaA and Pfizer, and Washington University in St. Louis and Merck & Co., to further elucidate the potential of FAK inhibition to enhance the efficacy of PD-(L)1 inhibitors in patients with pancreatic and ovarian cancer," said Robert Forrester, President and Chief Executive Officer of Verastem. "The data generated from these trials will continue to inform the ongoing development of our anti-cancer therapeutics which reduce cancer stem cells and modulate the local tumor microenvironment to allow both cancer treatments and the immune system to do their job more efficiently. We've had a strong start to 2016 with the announcement of these clinical collaborations in addition to attracting key strategic hires on the development team, including Dr. Greg Berk as Chief Medical Officer and Dr. Toyin Shonukan as Vice President of Clinical Development, to oversee and execute on our ongoing and future studies. We are well financed with approximately $100 million in available capital and we look forward to keeping you updated in the coming quarters on our progress."

First Quarter 2016 and Recent Highlights:

Focal Adhesion Kinase Inhibition Program

- ***Clinical Collaboration with Pfizer and Merck KGaA to Evaluate Combination of VS-6063 and Avelumab in Ovarian Cancer*** – In March 2016, the companies announced a clinical trial collaboration agreement to evaluate the combination of Verastem's focal adhesion kinase (FAK) inhibitor VS-6063 and Pfizer and Merck KGaA's anti-PD-L1 immunotherapy avelumab. Verastem has previously reported initial signs of clinical activity in patients with ovarian cancer when VS-6063 is used in combination with paclitaxel. Under the terms of the agreement, the parties will conduct a planned Phase 1/1b clinical trial evaluating escalating doses of the combination of VS-6063 and avelumab as a potential treatment option for patients with advanced ovarian cancer.
- ***Washington University in St. Louis Initiated a Clinical Study of VS-6063 in Combination with Merck & Co.'s Pembrolizumab and Gemcitabine in Pancreatic Cancer*** – In January 2016, Verastem announced the initiation of a Phase 1 dose-escalation study at Washington University to evaluate its FAK inhibitor VS-6063 in combination with Merck & Co.'s anti-PD-1 immunotherapy pembrolizumab and gemcitabine in patients with pancreatic cancer. The trial builds upon preclinical research conducted by Dr. David Denardo, presented at several conferences in late 2015 and early 2016, demonstrating the ability of FAK inhibition to increase the efficacy of checkpoint inhibition in the reduction of tumor volume and overall survival in models of pancreatic cancer. This Phase 1 clinical trial is currently enrolling and is anticipated to enroll approximately 50 patients with advanced pancreatic cancer.
- ***Presented Scientific Data Supporting FAK Inhibition in Combination with Immunotherapy at Key Medical Meetings*** – During the first quarter of 2016, Verastem presented data in support of its new development programs focused on advancing its FAK inhibitors in combination with immune-oncology agents and other current and emerging standard of care cancer treatments. Data were presented at several medical and scientific meetings, including the 2016 American Academy of Cancer Research (AACR), the Society for Gynecologic Oncology's 2016 Annual Meeting on Women's Cancer, the Keystone Symposium on Cancer Pathology, the Keystone Symposium on Stem Cells and Cancer, and Immunotherapy World 2016.
- ***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-4718 Continues in Solid Tumors*** – Dosing continues in a Phase 1 dose escalation trial evaluating single-agent VS-4718 and a Phase 1 clinical trial evaluating VS-4718 in combination with gemcitabine and Abraxane® is currently ongoing. Following results from the dose escalation trial, an expansion cohort of VS-4718 + Gemcitabine/Abraxane® vs Gemcitabine/Abraxane® alone in patients with pancreatic cancer is planned.

Dual PI3K/mTORC1/2 Inhibition Program

- ***Confirmatory Recommended Phase 2 Dose and Expansion Cohorts*** – 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 has been observed in some tumor

types.

Corporate

- ***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 highly accomplished physician and a well-regarded oncology veteran with more than 25 years of both industry and academic experience, will be responsible for leading the Company's global clinical development strategy and clinical operations.
- ***Announced Key Executive Management Appointments and Changes*** – In April 2016, the Company strengthened its management team through the appointment and promotion of several key individuals. Jonathan Pachter, PhD was promoted to Chief Scientific Officer, David Weaver, PhD was appointed Vice President, Translational Medicine, Joe Chiapponi, Vice President, Finance, was named Treasurer, Principal Accounting and Financial Officer and Oluwatoyin (Toyin) Shonukan, MD, has been appointed Vice President, Clinical Development. Dr. Shonukan most recently served as Senior Medical Director, Oncology Clinical Development at Vertex Pharmaceuticals and has held previous senior appointments at Millennium: The Takeda Oncology Company, Novartis Oncology and Eli Lilly.

First Quarter 2016 Financial Results

Net loss for the first quarter ended March 31, 2016 (2016 Quarter) was $8.3 million, or $0.22 per share, as compared to a net loss of $15.2 million, or $0.46 per share, for the first quarter ended March 31, 2015 (2015 Quarter). Net loss for the 2016 Quarter and 2015 Quarter, excluding non-cash stock-based compensation expense of $1.7 million and $2.9 million, was $6.6 million and $12.3 million, respectively.

Research and development expense for the 2016 Quarter was $4.2 million compared to $10.5 million for the 2015 Quarter. The $6.3 million decrease from the 2015 Quarter to the 2016 Quarter was primarily related to a decrease of $4.2 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 $1.4 million, a decrease of approximately $550,000 in stock-based compensation, and a decrease of approximately $441,000 in travel, facilities and other research and development costs. These decreases were partially offset by an increase of approximately $276,000 in consulting fees.

General and administrative expense for the 2016 Quarter was $4.3 million compared to $4.7 million for the 2015 Quarter. The decrease of approximately $400,000 from the 2015 Quarter to the 2016 Quarter primarily resulted from approximate decreases in stock-based compensation expense of $734,000 and $148,000 in personnel related costs. These decreases were offset by an increase of approximately $411,000 in consulting and professional fees.

As of March 31, 2016, Verastem had cash, cash equivalents and investments of $99.5 million compared to $110.3 million as of December 31, 2015. Verastem used $10.8 million for operating activities during the 2016 Quarter settling one-time compensation payments, severance payments and paying down accounts payable and accruals.

The number of outstanding common shares as of March 31, 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 their ability to improve patient survival.

About VS-5584

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 advanced solid tumors.

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 or pending clinical trials, additional planned studies, the expected timing for the reporting of data from ongoing trials 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 Sheet Information

(in thousands)

	March 31, 2016	**December 31, 2015**
Cash, cash equivalents and investments	$ 99,535	$ 110,258
Prepaid expenses and other current assets	1,002	585
Property and equipment, net	1,865	2,048
Other assets	162	203
Total assets	**$ 102,564**	**$ 113,094**
Accounts payable and accrued expenses	$ 6,124	$ 10,040
Other liabilities	472	585
Stockholders' equity	95,968	102,469
Total liabilities and stockholders' equity	**$ 102,564**	**$ 113,094**

Verastem, Inc.

Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended March 31,	
	2016	**2015**
Operating expenses:		
Research and development	$ 4,179	$ 10,528
General and administrative	4,255	4,714
Total operating expenses	8,434	15,242
Loss from operations	(8,434)	(15,242)
Interest income	140	62
Net loss	**$ (8,294)**	**$ (15,180)**
Net loss per share—basic and diluted	$ **(0.22)**	**$ (0.46)**
Weighted-average number of common shares used in net loss per share-basic and diluted	**36,975**	**33,323**

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