Verastem Reports Year-End 2015 Financial Results

March 3, 2016

BOSTON--(BUSINESS WIRE)--Mar. 3, 2016-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer, today reported financial results for the year ended December 31, 2015, and also provided an overview of certain corporate developments.

"We are developing treatments that 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," said Robert Forrester, President and Chief Executive Officer of Verastem. "Our recently announced collaborations with Pfizer and Merck KGaA, and with Merck & Co. and Washington University in St. Louis, to evaluate the combination of our FAK inhibitors with immune-oncology agents speak to the understanding among the clinical community that innovative combination therapies have the potential to complement and enhance existing therapies. We begin 2016 with a strong balance sheet and expect significant progress from our ongoing programs targeting high unmet need cancers including non-small cell lung, ovarian, lymphoma, mesothelioma and pancreatic cancer. We are also planning for several trial initiations this year and we look forward to keeping you updated on our progress."

Recent Highlights:

Focal Adhesion Kinase Inhibition Program

There is a growing body of preclinical research suggesting that focal adhesion kinase (FAK) inhibition, when combined with PD-1 inhibitors, may increase the anti-tumor activity of immunotherapeutic agents such as programmed death receptor 1 (PD-1) and its corresponding ligand (PD-L1). Research reports on this were published in the September 24, 2015 edition of *Cell* and presented at the 2015 AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics, the 2015 Society for Immunotherapy of Cancer Conference, and the 2016 Immunotherapy World Conference.

The data presented provide an overview of preclinical research to date demonstrating how FAK inhibition increases the influx of cytotoxic T cells into tumors while reducing immuno-suppressive and stromal density barriers to antitumor immune attack. This research suggests that FAK inhibition creates a more favorable tumor microenvironment for the antitumor effects of immune checkpoint inhibitors and potentially other immunotherapies. Verastem expects multiple combination clinical trials, building on previously reported signals of clinical activity in addition to preclinical rationale, to be conducted this year in ovarian cancer, pancreatic cancer, mesothelioma and non-small cell lung cancer. To date, the following clinical studies have been announced:

- ***Clinical Collaboration with Pfizer and Merck KGaA to Evaluate Combination of VS-6063 and Avelumab in Ovarian Cancer –*** In March 2016, the companies announced their entry into a clinical trial collaboration agreement to evaluate the investigational combination of Verastem's focal adhesion kinase (FAK) inhibitor VS-6063 and Pfizer/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 Verastem's FAK inhibitor VS-6063 in combination with Merck & Co.'s anti-PD-1 immunotherapy pembrolizumab and gemcitabine in patients with pancreatic cancer.

In addition, Verastem's second FAK inhibitor, VS-4718, has demonstrated a generally well-tolerated safety profile in a single-agent ascending dose study and is suitable for progression into further clinical studies. Confirmatory cohorts to determine the recommended Phase 2 dose as well as expansion cohorts in biopsiable disease are planned for 2016. Additional studies for VS-4718 in 2016 include:

- ***Combination Trial of VS-4718 and Gemcitabine/Abraxane® –*** A clinical trial of VS-4718 in combination with gemcitabine and Abraxane® was recently initiated. Initial results of the dose escalation study are expected by year end 2016. Following results from the dose escalation, 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

The maximum tolerated dose of VS-5584 has been reached in the Phase 1, single-agent study of VS-5584, and the recommended Phase 2 dose is being confirmed. Reductions in pharmacodynamic markers of PI3K and mTOR activity and clinical activity has been observed in some tumor types. Additional studies for VS-5584 in 2016 include:

- ***Confirmatory Recommended Phase 2 Dose and Expansion Cohorts –*** A cohort of the single-agent VS-5584 trial is enrolling additional patients with solid tumors or lymphomas to confirm the recommended dose for Phase 2 trials. Expansion cohorts in ovarian and endometrial cancer, non-hodgkins lymphoma, and chronic lymphocytic leukemia are planned for 2016.

Full Year 2015 Financial Results

As of December 31, 2015, Verastem had cash, cash equivalents and investments of $110.3 million compared to $92.7 million as of December 31, 2014. Verastem used $45.6 million for operating activities during the year ended December 31, 2015 ("2015 Period").

Net loss for the 2015 Period was $57.9 million, or $1.61 per share, as compared to a net loss of $53.4 million, or $2.07 per share, for the year ended December 31, 2014 ("2014 Period"). Net loss for the 2015 Period and 2014 Period includes non-cash stock-based compensation expense of $9.7 million and $12.1 million, respectively.

Research and development expense for the 2015 Period was $40.6 million compared to $35.4 million for the 2014 Period. The $5.2 million increase from the 2014 Period to the 2015 Period was primarily related to an increase of $5.8 million in contract research organization expense for outsourced biology, chemistry, development and clinical services, which includes our clinical trial costs, an increase in personnel related costs of $1.4 million, and an increase of approximately $558,000 in consulting expense. These increases were partially offset by decreases of $1.3 million in stock-based compensation expense and $1.2 million in license fees.

General and administrative expense for the 2015 Period was $17.6 million compared to $18.2 million for the 2014 Period. The decrease of approximately $525,000 from the 2014 Period to the 2015 Period primarily resulted from decreases in stock-based compensation expense of $1.1 million and approximately $446,000 in professional fees. These decreases were offset by an increase of approximately $856,000 in personnel costs.

The number of outstanding common shares as of December 31, 2015, was 36,941,261.

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 utility of FAK inhibitors for the treatment of cancer including in combination with other cancer treatments, 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.

Verastem, Inc.

Unaudited Selected Consolidated Balance Sheet Information

(in thousands)

	December 31, 2015	December 31, 2014
Cash, cash equivalents and investments	$ 110,258	$ 92,675
Prepaid expenses and other current assets	585	2,641
Property and equipment, net	2,048	2,825
Other assets	203	508
Total assets	**$ 113,094**	**$ 98,649**
Accounts payable and accrued expenses	$ 10,040	$ 8,735
Other liabilities	585	1,148
Stockholders' equity	102,469	88,766
Total liabilities and stockholders' equity	**$ 113,094**	**$ 98,649**

Verastem, Inc.

Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Year ended December 31, 2015	2014
Operating expenses:		
Research and development	$ 40,565	$ 35,448
General and administrative	17,634	18,159
Total operating expenses	58,199	53,607
Loss from operations	(58,199)	(53,607)
Interest income	334	242
Net loss	$ (57,865)	$ (53,365)
Net loss per share–basic and diluted	$ (1.61)	$ (2.07)
Weighted-average number of common shares used in net loss per share-basic and diluted	35,932	25,804

View source version on businesswire.com: http://www.businesswire.com/news/home/20160303005326/en/

Source: Verastem, Inc.

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