



Verastem Reports Third Quarter 2015 Financial and Corporate Results

November 9, 2015

BOSTON--(BUSINESS WIRE)--Nov. 9, 2015-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today reported financial results for the third quarter ended September 30, 2015, and also provided an overview of certain corporate developments.

"Our mission is to improve survival and the quality of life for patients battling cancer through the combination of our cancer stem cell-targeting agents with current and emerging standard of care treatments," said Robert Forrester, President and Chief Executive Officer of Verastem. "We are supported by a strong balance sheet, three clinical compounds and a team of talented and dedicated professionals. We continue to believe in our pipeline candidates and are focused on executing their clinical development."

Mr. Forrester continued: "We are working with thought-leading researchers on innovative anti-cancer applications for our compounds. For example, researchers from the University of Edinburgh recently published a ground-breaking paper in "Cell" which highlights the potential of focal adhesion kinase (FAK) inhibition to enable the body's immune system to better fight cancer. While this is early stage data, it provides support for the thesis that FAK inhibitors may be useful in combination with immuno-oncology agents with the goal of yielding more durable responses for a greater number of cancer patients."

Recent Developments

Stopped Enrollment in the COMMAND Study Evaluating VS-6063 in Mesothelioma Due to Futility – In September 2015, Verastem announced its decision to stop enrollment in the Phase 2 registration-directed, double-blind, placebo-controlled study (COMMAND) of VS-6063 for patients with mesothelioma. The decision to stop enrollment followed a Data Safety Monitoring Board (DSMB) review of a pre-planned interim analysis. The results of the analysis demonstrated that VS-6063 had a generally well tolerated safety profile but that there was not a sufficient level of efficacy to warrant continuation of the study.

New Research Published in the Journal "Cell" Highlighting the Potential of FAK Inhibition to Enhance the Efficacy of Anti-Tumor Immunotherapy – In September 2015, Verastem announced that researchers from the University of Edinburgh published a study in the journal "Cell" which discusses results from preclinical research showing that focal adhesion kinase (FAK), a protein which is often overproduced in tumors, enables cancer cells to evade attack by the body's immune system. In this study, researchers discovered that FAK inhibition can favorably modulate the balance of immune cells in the tumor, inducing immune-mediated tumor elimination in preclinical models.

Presented Data on VS-6063, VS-4718 and VS-5584 at Key Oncology-Focused Medical Meetings – In November 2015, Verastem presented a total of six posters at the 2015 AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics and the Society for Immunotherapy of Cancer (SITC) 30th Anniversary Annual Meeting. The presented data described the results of preclinical research showing that FAK inhibitors may enhance the effects of anti-cancer immunotherapy for both typically responsive and non-responsive tumors.

During the third quarter, Verastem also gave several oral and poster presentations at the European Society for Medical Oncology (ESMO)/18th European Cancer Congress (ECC) and at the 16th World Conference on Lung Cancer (WCLC) describing clinical and preclinical data relating to its pipeline product candidates.

All of the posters and presentations can be found on the Presentations page of [Verastem's website](#).

Third Quarter 2015 Financial Results

As of September 30, 2015, Verastem had cash, cash equivalents and investments of \$120.5 million compared to \$92.7 million as of December 31, 2014. Verastem used \$11.6 million for operating activities in the third quarter ended September 30, 2015 (the "2015 Quarter").

Net loss for the 2015 Quarter was \$15.4 million, or \$0.42 per share, as compared to a net loss of \$13.3 million, or \$0.52 per share, for the same period in 2014 (the "2014 Quarter"). Net loss includes stock-based compensation expense of \$2.1 million and \$2.8 million for the 2015 Quarter and 2014 Quarter, respectively.

Research and development expense for the 2015 Quarter was \$11.3 million compared to \$9.0 million for the 2014 Quarter. The \$2.3 million increase from the 2014 Quarter to the 2015 Quarter was primarily related to an increase of \$2.3 million in contract research organization expense for outsourced biology, chemistry, development and clinical services, which includes our clinical trial costs, an increase in consulting fees of approximately \$299,000, an increase in personnel related costs of approximately \$115,000 due to increased headcount and salaries, and an increase of approximately \$102,000 in lab supplies due to increased research activity. These increases were partially offset by a decrease of approximately \$537,000 in stock-based compensation.

General and administrative expense. General and administrative expense for the 2015 Quarter was \$4.2 million compared to \$4.3 million for the 2014 Quarter. The decrease of approximately \$100,000 from the 2014 Quarter to the 2015 Quarter primarily resulted from a decrease in professional fees of approximately \$249,000, primarily related to lower IP and general legal costs, and a decrease in stock-based compensation expense of approximately \$245,000. These decreases were partially offset by increases in personnel related costs of approximately \$247,000, primarily due to an increase in headcount and salaries, and in consulting fees of approximately \$164,000.

The number of outstanding common shares as of September 30, 2015, was 36,934,804.

Financial Guidance

We expect our existing cash, cash equivalents and investments will enable us to fund our current operating plan and capital expenditure requirements at least through the first half of 2017.

About VS-6063

VS-6063 (defactinib) is an orally available compound designed to target cancer stem cells through the potent inhibition of focal adhesion kinase (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 is currently being studied in the "Window of Opportunity" study in patients with mesothelioma prior to surgery, a Phase 1/1b study in combination with paclitaxel in patients with ovarian cancer, a trial in patients with KRAS-mutated non-small cell lung cancer and a trial evaluating the combination of VS-6063 and VS-5584 in patients with relapsed mesothelioma.

About VS-4718

VS-4718 is an orally available compound designed to target cancer stem cells through the potent inhibition of focal adhesion kinase (FAK). VS-4718 is currently being studied in a Phase 1 dose escalation study in patients with advanced cancers.

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 as a single agent and a combination trial of VS-5584 and VS-6063 in patients with relapsed mesothelioma. VS-5584 has been granted orphan drug designation for use in mesothelioma in the U.S. and EU.

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.

Forward-looking statements:

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.

Verastem, Inc.

Unaudited Selected Consolidated Balance Sheet Information

(in thousands)

	September 30, 2015	December 31, 2014
Cash, cash equivalents and investments	\$ 120,467	\$ 92,675
Prepaid expenses and other current assets	714	2,641
Property and equipment, net	2,282	2,825
Other assets	203	508
Total assets	\$ 123,666	\$ 98,649
Accounts payable and accrued expenses	\$ 10,817	\$ 8,735
Other liabilities	606	1,148

Stockholders' equity	112,243	88,766
Total liabilities and stockholders' equity	\$ 123,666	\$ 98,649

Verastem, Inc.

Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended September 30,		Nine months ended September 30,	
	2015	2014	2015	2014
Operating expenses:				
Research and development	\$ 11,304	\$ 9,047	\$ 32,877	\$ 25,763
General and administrative	4,230	4,341	13,361	13,846
Total operating expenses	15,534	13,388	46,238	39,609
Loss from operations	(15,534)	(13,388)	(46,238)	(39,609)
Interest income	89	56	236	193
Net loss	\$ (15,445)	\$ (13,332)	\$ (46,002)	\$ (39,416)
Net loss per share—basic and diluted	\$ (0.42)	\$ (0.52)	\$ (1.29)	\$ (1.54)
Weighted-average number of common shares used in net loss per share—basic and diluted	36,898	25,811	35,594	25,654

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