Verastem Reports First Quarter 2015 Financial and Corporate Results

May 11, 2015

— COMMAND Interim Analysis Anticipated in Third Quarter 2015 —

BOSTON--(BUSINESS WIRE)--May 11, 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 first quarter ended March 31, 2015, and also provided an overview of certain corporate accomplishments and plans.

"The team continues to efficiently execute on each of our ongoing programs targeting cancer stem cells," said Robert Forrester, President and Chief Executive Officer of Verastem. "Accrual to the COMMAND study evaluating our cancer stem cell inhibitor, VS-6063, as a switch maintenance treatment immediately following frontline therapy in mesothelioma remains on track. As of May 11th, 255 patients have been randomized to the COMMAND study. The number of progression events required to trigger the pre-planned interim analysis has not yet been reached, so we now anticipate that the independent Data and Safety Monitoring Board will conduct the interim analysis in the third quarter of 2015. In its review, the DSMB will examine pre-specified efficacy and safety data sets to decide whether to continue the study as planned in all patients, enrich the study population based upon the biomarker merlin, or stop the study early for futility. This will be an important milestone for Verastem to allow the study to continue and define the primary patient population for this registration-directed study."

Mr. Forrester added: "On the financial front, we raised over $55 million in new equity capital in the first quarter, which significantly strengthened our balance sheet and enables the continuing execution of our current clinical programs. We ended the quarter with $136.1 million in cash, cash equivalents and investments."

Q1 2015 and Recent Highlights

VS-6063 (Focal Adhesion Kinase Inhibition)

- COMMAND (**C**ontrol **O**f **M**esothelioma with **MA**inte**N**ance **D**efactinib) Study
  - Registration-directed, randomized, double-blind, placebo-controlled study of VS-6063 as a switch maintenance treatment in patients with malignant pleural mesothelioma benefiting from frontline therapy with pemetrexed (Alimta) and platinum
  - Primary endpoints are Progression Free Survival (PFS) and Overall Survival (OS). The study is designed to provide 90% power to assess the superiority of PFS, a co-primary efficacy endpoint, with a 1 sided type I error rate of 0.025, assuming a hazard ratio of 0.67.
  - 255 patients enrolled at 60 centers in 13 countries to date
  - Interim analysis to allow the study to continue and define the primary patient population anticipated in Q3 2015
- Received orphan medicinal product designation from the European Commission and orphan drug designation from the FDA for use in ovarian cancer and orphan drug designation from Australia's Department of Health Therapeutic Goods Administration for use in mesothelioma.

VS-5584 (Oral Dual mTORC 1/2 and PI3K Inhibitor)

- Initiated Phase 1 clinical trial evaluating combination of VS-5584 and VS-6063 in relapsed mesothelioma
  - Open-label, dose escalation and schedule finding study designed to assess safety, pharmacokinetics, pharmacodynamics and initial observations of clinical activity
  - Study expected to enroll up to 56 patients at clinical sites in the UK and US
  - Supported by preclinical work demonstrating the synergistic activity of VS-6063 and VS-5584 in mesothelioma models *in vitro* and *in vivo*
- Received orphan medicinal product/drug designation from the FDA and European Commission for use in mesothelioma

Presentations and Publications

- Reported encouraging scientific data in support of Verastem's cancer stem cell inhibitors (VS-6063, VS-4718 and VS-5584) in multiple tumor types, including mesothelioma, small cell lung cancer, breast cancer and hematologic malignancies at the 2015 American Association of Cancer Research (AACR) Annual Meeting. Copies of the presentations can be accessed at: http://bit.ly/12otlcV
- Published preclinical data supporting VS-5584 in *Cancer Research,* a peer-reviewed journal of the AACR

Financial/Corporate

- Raised $55.4 million (net of expenses) in new equity capital through the sale of approximately 8.8 million shares of

common stock to the public

Upcoming Clinical Milestones

Verastem's planned upcoming milestones include:

VS-6063

- Report COMMAND interim analysis: Q3 2015
- Report Phase 2 results in KRAS-mutated NSCLC: H2 2015
- Report updated results from the VS-6063/paclitaxel combination in patients with ovarian cancer: H2 2015
- Report on the biomarker "Window of Opportunity" study with preliminary results from the extended treatment cohort: H1 2016

VS-4718

- Report preliminary Phase 1 results in patients with advanced solid tumors: H2 2015

VS-5584

- Report preliminary Phase 1 results in patients with advanced solid tumors: H2 2015

First Quarter 2015 Financial Results

As of March 31, 2015, Verastem had cash, cash equivalents and investments of $136.1 million compared to $92.7 million as of December 31, 2014. Verastem used $11.7 million for operating activities in the first quarter ended March 31, 2015 (the "2015 Quarter").

Net loss for the 2015 Quarter was $15.2 million, or $0.46 per share, as compared to net loss of $13.1 million, or $0.51 per share, for the same period in 2014 (the "2014 Quarter"). Net loss includes stock-based compensation expense of $2.9 million and $3.6 million for the 2015 Quarter and 2014 Quarter, respectively.

Research and development expense for the 2015 Quarter was $10.5 million compared to $8.4 million for the 2014 Quarter. The $2.1 million increase from the 2014 Quarter to the 2015 Quarter is primarily related to an increase of $2.9 million in contract research organization expense for outsourced biology, development and clinical services, which includes our clinical trial costs, and an approximate $770,000 increase in personnel costs primarily due to an increase in headcount. These increases were partially offset by a decrease in license fees of $1.2 million primarily due to fees related to the Encarta asset purchase in the 2014 Quarter and a decrease of stock-based compensation of approximately $379,000.

General and administrative expense for the 2015 Quarter was $4.7 million compared to $4.7 million for the 2014 Quarter. In the 2015 Quarter as compared with the 2014 Quarter, personnel costs increased by approximately $466,000, professional fees decreased by approximately $365,000, stock-based compensation decreased by approximately $259,000 and other G&A expense increased by approximately $158,000.

The number of outstanding common shares as of March 31, 2015, was 36,152,396.

Financial Guidance

Based on current operating plans, the Company expects to have sufficient cash, cash equivalents and investments to fund our research and development programs and operations into 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 registration-directed COMMAND trial in mesothelioma (www.COMMANDmeso.com), a "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. VS-6063 has been granted orphan drug designation for use in mesothelioma in the U.S. and EU.

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 the Company's strategy, future plans and prospects, including statements regarding the development and activity of the Company's product candidates, VS-6063, VS-4718 and VS-5584, and the Company's FAK, PI3K/mTOR and diagnostics programs generally, the timeline for clinical development and regulatory approval of our product candidates, the expected timing for the reporting of data from on-going trials and for the COMMAND interim analysis, the structure of our planned or pending clinical trials, additional planned studies, 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 the Company'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 the Company will be unable to successfully initiate or complete the clinical development of its product candidates, that the development of the Company's product candidates will take longer or cost more than planned, and that the Company'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 the Company's Annual Report on Form 10-K for the year ended December 31, 2014 and in any subsequent SEC filings. The forward-looking statements contained in this press release reflect the Company's current views with respect to future events, and the Company 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, 2015	December 31, 2014
Cash, cash equivalents and investments	$ 136,072	$ 92,675
Prepaid expenses and other current assets	2,801	2,641
Property and equipment, net	2,645	2,825
Other assets	485	508
Total assets	**$ 142,003**	**$ 98,649**
Accounts payable and accrued expenses	$ 9,205	$ 8,735
Other liabilities	893	1,148
Stockholders' equity	131,905	88,766
Total liabilities and stockholders' equity	**$ 142,003**	**$ 98,649**

Verastem, Inc.
Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended March 31, 2015	2014
Operating expenses:		
Research and development	$ 10,528	$ 8,411
General and administrative	4,714	4,723
Total operating expenses	15,242	13,134
Loss from operations	(15,242)	(13,134)
Interest income	62	72
Net loss	($15,180)	($13,062)
Net loss per share–basic and diluted	($0.46)	($0.51)
Weighted-average number of common shares used in net loss per share -basic and diluted	33,323	25,478

Source: Verastem, Inc.

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