Verastem Reports First Quarter 2013 Financial and Corporate Results

May 9, 2013

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May. 9, 2013-- 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 quarter ended March 31, 2013, and also commented on certain corporate accomplishments and plans.

“During the first quarter we made key strides in advancing our development programs targeting cancer stem cells,” said Christoph Westphal, M.D., Ph.D., Chairman and Chief Executive Officer of Verastem.

“The combination trial of VS-6063 plus paclitaxel for ovarian cancer is open and enrolling patients at all sites,” said Robert Forrester, President and Chief Operating Officer of Verastem. “In addition, we met with the regulatory agencies in the US and UK and are on track to initiate midyear the potentially pivotal trial of VS-6063 in mesothelioma.”

Q1 2013 and Recent Accomplishments

Our significant accomplishments include the following:

- **Advanced the FAK inhibition program and defined a potential registration pathway**
  - Met with the regulatory agencies in the US and UK and, based on these discussions, we believe that positive results from our anticipated trial of VS-6063 in mesothelioma will enable us to seek regulatory approval
  - Advanced our diagnostic strategy through an agreement with LabCorp to develop a companion diagnostic for VS-6063 to stratify patients in the mesothelioma trial
  - Filed for orphan drug designation for VS-6063 in mesothelioma within the European Union and United States
  - Initiated a Phase 1/1b study of VS-6063 in combination with paclitaxel for patients with ovarian cancer, which is open and enrolling at all sites
  - Presented data at the 2013 AACR Annual Meeting showing that treatment with FAK inhibitor VS-4718 results in an approximate 200-fold reduction in the tumor-initiating capability of cells extracted from tumors as compared to paclitaxel in a xenograft model of triple negative breast cancer
  - Received IND allowance from the FDA for the VS-4718 Phase 1 trial to proceed in advanced solid tumors
- **Progressed the dual PI3K/mTOR inhibition program**
  - Conducted IND-enabling studies of VS-5584 with a goal of initiating Phase 1 clinical development in H2 2013
  - Presented data at the 2013 AACR Annual Meeting demonstrating the ability of VS-5584 treatment to induce tumor regression in taxane-resistant patient-derived xenograft models
  - Published data on PI3K/mTOR inhibitor VS-5584 in *Molecular Cancer Therapeutics*
- **Increased the understanding of cancer stem cell biology**
  - Presented research results widely at scientific conferences including AACR, Keystone PI3 Kinase Symposium and the Molecular Medicine Tri-Conference Symposium on Targeting Cancer Stem Cells

2013 Milestones

Our planned upcoming clinical milestones include the following:

- **Initiate the potentially pivotal trial in mesothelioma for VS-6063 midyear 2013**
- **Complete the dose finding portion of the Phase 1/1b trial of VS-6063 plus paclitaxel in ovarian cancer**
- **Begin enrollment of the expanded cohort of the Phase 1/1b trial of VS-6063 plus paclitaxel in ovarian cancer**
- **Initiate Phase 1 clinical development of VS-4718 H1 2013**
- **Initiate Phase 1 clinical development of VS-5584 H2 2013**

Upcoming Events

- **UBS Global Healthcare Conference**
  - Wednesday, May 22, 2013, at 8:30am ET at the Sheraton New York Hotel, New York, NY
- **ASCO Breakfast**
  - Saturday, June 1, 2013, at 6:45am CT at the Hyatt Regency McCormick Place, Chicago, IL. Special guest Dr. Dean Fennel, Chair of Thoracic Medical Oncology, University of Leicester, will be presenting together with Chief Medical Officer, Dr. Joanna Horobin, and Head of Research, Jonathan Pachter, Ph.D. Topics will include mesothelioma etiology, the role of cancer stem cells in disease progression, current clinical treatments and the design of

Verastem's potentially pivotal trial of lead FAK inhibitor VS-6063. RSVP to bsullivan@verastem.com

- **Research and Development Day**
  - Thursday, July 11, 2013, at the Harvard Club in New York, NY. Special guests include: Robert Weinberg, Ph.D., Founding Member, Whitehead Institute; José Baselga, M.D., Ph.D., Physician in Chief, Memorial Sloan-Kettering Cancer Center; and Lee Krug, M.D., Thoracic Oncologist and Director of the Mesothelioma Program at Memorial Sloan-Kettering Cancer Center. Topics will include recent updates on the biology of cancer stem cells, clinical needs in the treatment of mesothelioma and strategies for the therapeutic targeting of PI3K/mTOR in cancer. Verastem will provide updates on the status of research and development and upcoming plans. RSVP to bsullivan@verastem.com

First Quarter 2013 Financial Results

As of March 31, 2013, Verastem had cash, cash equivalents and investments of $84.4 million compared to $91.5 million on December 31, 2012, a difference of $7.1 million. The number of outstanding common shares as of April 30, 2013, was 21,289,319.

Net loss for the three months ended March 31, 2013 (the "2013 Quarter"), was $9.0 million, or $0.44 per share applicable to common shareholders, as compared to net loss of $6.9 million, or $0.47 per share, for the three months ending March 31, 2012 (the "2012 Quarter"). Net loss for the 2013 Quarter includes non-cash stock-based compensation expense of $2.5 million as compared to $1.5 million for the 2012 Quarter.

Research and development expense for the 2013 Quarter was $5.3 million compared to $4.8 million for the 2012 Quarter. The $494,000 increase from the 2012 Quarter to the 2013 Quarter is primarily related to an increase of $650,000 in contract research organization expense for outsourced biology, chemistry and development services and an increase of $309,000 for personnel costs primarily due to increased headcount. These increases are partially offset by a decrease of $367,000 in license fee expense primarily related to the revaluation of the obligation to issue the warrant to Poniard Pharmaceuticals in the 2012 Quarter.

General and administrative expense for the 2013 Quarter was $3.8 million compared to $2.1 million for the 2012 Quarter. The $1.7 million increase from the 2012 Quarter to the 2013 Quarter primarily resulted from an increase of $1.0 million for personnel costs primarily due to stock-based compensation expense associated with restricted stock units and restricted stock units with performance-based vesting provisions, an increase of $437,000 in professional fees and an increase of $111,000 in consulting fees.

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, PI3K/mTOR and Wnt. 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 of the Company's compounds, including VS-6063, VS-4718 and VS-5584, and the Company's FAK, PI3K/mTOR and diagnostic programs generally, the timeline for clinical development and regulatory approval of the Company's compounds, the structure of the Company's planned clinical trials and estimates of the Company's ability to fund operations. 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 compounds may not be predictive of the success of later clinical trials, that the Company will be unable to successfully complete the clinical development of its compounds, including VS-6063, VS-4718 and VS-5584, that the development of the Company's compounds will take longer or cost more than planned, and that the Company's compounds 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, 2012 and in any subsequent SEC filings. The forward-looking statements contained in this presentation 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.

(A development-stage company)

Unaudited Selected Consolidated Balance Sheet Information

(in thousands)

	March 31, 2013	December 31, 2012
Cash, cash equivalents and investments	$84,414	$91,520
Prepaid expenses and other current assets	986	506
Property and equipment, net	755	811
Other assets	86	86
Total assets	**$86,241**	**$92,923**

Accounts payable and accrued expenses	$3,040	$2,399
Other liabilities	370	58
Stockholders' equity	82,831	90,466
Total liabilities and stockholders' equity	**$86,241**	**$92,923**

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Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three Months Ended March 31,	
	2013	2012
Operating expenses:		
Research and development	$5,296	$4,803
General and administrative	3,785	2,125
Total operating expenses	9,081	6,928
Loss from operations	(9,081)	(6,928)
Interest income	44	57
Net loss	(9,037)	(6,871)
Accretion of preferred stock	-	(6)
Net loss applicable to common stockholders	($9,037)	($6,877)
Net loss per share applicable to common stockholders–basic and diluted	($0.44)	($0.47)
Weighted-average number of common shares used in net loss per share applicable to common stockholders-basic and diluted	20,483	14,693

Source: Verastem, Inc.

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