Verastem Reports Third Quarter 2013 Financial and Corporate Results

November 12, 2013

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 12, 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 third quarter ended September 30, 2013, and also provided an overview of certain corporate accomplishments and plans.

“We continue to execute on our development plans and have made significant and rapid progress in the third quarter,” said Robert Forrester, President and Chief Executive Officer of Verastem. “We have initiated five of our six planned clinical trials in 2013. We recently received IND allowance for the dual mTORC1/2 and PI3K inhibitor, VS-5584, and are on track to start the Phase 1 trial by year end.”

“We recently initiated three additional clinical trials of lead candidate VS-6063,” said Dr. Joanna Horobin, Verastem Chief Medical Officer. “The multi-national COMMAND study in mesothelioma is now open and accruing patients. We are actively enrolling patients to the Phase 2 trial of VS-6063 in patients with KRas-mutated non-small cell lung cancer and the Phase 1 dose escalation trial of VS-6063 in Japan. In addition, we continue to enroll the expansion phase of the combination trial of VS-6063 and weekly paclitaxel in patients with ovarian cancer. These efforts are part of the ongoing expansion for VS-6063’s development program by both geography and therapeutic indication.”

“It is our mission to develop novel therapies targeting cancer stem cells to provide more durable responses for patients battling many types of cancer,” said Christoph Westphal, M.D., Ph.D., Verastem Executive Chairman.

Q3 2013 and Recent Accomplishments

Our significant accomplishments include the following:

Advanced the FAK inhibition program

- Initiated COMMAND: A randomized, placebo controlled study of VS-6063 as maintenance following frontline therapy in patients with mesothelioma being conducted at approximately 35 sites in 11 countries
- Reported on the dose escalation portion of the Phase 1/1b study of VS-6063 in combination with weekly paclitaxel in patients with ovarian cancer
    - The combination was well tolerated at both dose levels with no worsening of the well-known side effects of paclitaxel
    - Initial activity observed including a Complete Response in one of the three patients in the first cohort
- Opened the expansion portion of the Phase 1/1b study of VS-6063 and weekly paclitaxel in ovarian cancer
- Initiated a Phase 2 study of VS-6063 in KRas-mutated non-small cell lung cancer
- Initiated a Phase 1 study of VS-6063 in Japan to evaluate the safety profile and pharmacokinetics of VS-6063 in Japanese patients
- Opened all sites for the Phase 1 dose escalation trial of VS-4718 in patients with advanced cancers
- Presented 11 posters on the clinical and preclinical development of VS-6063 and the role of FAK in cancer stem cell-driven disease progression at the annual AACR-EORTC-NCI Molecular Targets, AACR Ovarian Cancer, Cancer Advance and World Lung conferences

Progressed the dual PI3K/mTOR inhibition program

- Received IND allowance to initiate a Phase 1 study of VS-5584. The study is anticipated to start by year end 2013 in patients with advanced solid tumors and lymphomas
- Presented data at the 2013 AACR-EORTC-NCI Molecular Targets and the American Chemical Society meetings demonstrating the ability of VS-5584 to reduce cancer stem cells across multiple preclinical tumor models

Strengthened balance sheet and clinical advisory team

- Closed on a $63.8 million public offering with net proceeds to Verastem totaling approximately $59.8 million in July 2013
- Named Jose Baselga, M.D., Ph.D., Physician in Chief at Memorial Sloan-Kettering Cancer Center, as Senior Medical Advisor

2013/14 Milestones

Our planned upcoming clinical milestones include the following:

- Initiate Phase 1 dose escalation study of VS-5584 in patients with advanced solid tumors and lymphomas by year end 2013

- Complete enrollment in the Phase 1b portion of the VS-6063 trial in combination with weekly paclitaxel in patients with ovarian cancer in H1 2014
- Complete the Phase 1 dose escalation study of VS-6063 in patients with advanced solid tumors in Japan in H1 2014, with a goal of facilitating inclusion of Japanese sites into COMMAND in H2 2014
- Complete enrollment in the stage 1 portion of the VS-6063 Phase 2 trial in patients with non-small cell lung cancer midyear 2014
- Complete enrollment for the Phase 1 trial of VS-4718 in H2 2014

Upcoming Events

- NY CEO Conference on November 12th and 13th at Apella in New York City, NY
- Target TME Conference on November 18th-20th at the Royal Sonesta Hotel in Boston, MA
- Jefferies 2013 Global Healthcare Conference on November 20th and 21st at The Waldorf Hilton in London, UK

Third Quarter 2013 Financial Results

As of September 30, 2013, Verastem had cash, cash equivalents and investments of $130.3 million compared to $78.0 million on June 30, 2013 and $91.5 million on December 31, 2012. Verastem used $7.1 million for operating activities in the third quarter ended September 30, 2013.

Net loss for the third quarter ended September 30, 2013 (the "2013 Quarter") was $10.6 million, or $0.47 per share applicable to common stockholders, as compared to net loss of $10.4 million, or $0.51 per share applicable to common stockholders, for the same period in 2012 (the "2012 Quarter"). Net loss includes stock-based compensation expense of $2.8 million and $1.6 million for the 2013 Quarter and 2012 Quarter, respectively.

Research and development expense for the 2013 Quarter was $6.8 million compared to $8.1 million for the 2012 Quarter. The $1.3 million decrease from the 2012 Quarter to the 2013 Quarter was primarily related to a decrease of $2.7 million in license fee expense related to our agreement with Pfizer, Inc., including the issuance of 192,013 shares of common stock in the 2012 Quarter. This was partially offset by an increase of approximately $534,000 in contract research organization expense for outsourced biology, chemistry and development services, an approximately $426,000 increase in personnel costs primarily due to increased headcount and an approximately $158,000 increase in stock based compensation expense.

General and administrative expense for the 2013 Quarter was $3.9 million compared to $2.3 million for the 2012 Quarter. The $1.6 million increase from the 2012 Quarter to the 2013 Quarter primarily resulted from an increase of $1.0 million in stock based compensation expense associated with restricted stock units and restricted stock units with performance based vesting provisions, an increase in consulting costs of approximately $183,000 and an approximately $137,000 increase in personnel costs primarily due to increase in salaries and headcount.

The number of outstanding common shares as of November 7, 2013 was 25,647,732.

About VS-6063

VS-6063 (defactinib) is an oral 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 by Robert Weinberg, Ph.D., scientific cofounder and chair of Verastem's Scientific Advisory Board, and Verastem have demonstrated that the FAK pathway 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, a Phase 1b study in ovarian cancer, a Phase 1 study in Japan and a Phase 2 trial in KRas-mutated non-small cell lung cancer. VS-6063 has been granted orphan drug designation in the U.S. and E.U. for use in mesothelioma.

About VS-4718

VS-4718 is an oral compound designed to target cancer stem cells through the potent inhibition of focal adhesion kinase (FAK). Research by Robert Weinberg, Ph.D., scientific cofounder and chair of Verastem's Scientific Advisory Board, and Verastem have demonstrated that the FAK pathway is critical for the growth and survival of cancer stem cells. 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 oral compound that potently and selectively inhibits the class 1 PI3K isoforms, mTORC1 and mTORC2. In preclinical studies, VS-5584 has been shown to reduce the percentage of cancer stem cells and induce tumor regression in taxane-resistant models. Verastem expects to initiate a Phase 1 dose escalation trial in patients with advanced solid tumors and lymphomas around year end 2013.

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, or defactinib, VS-4718 and VS-5584 and the Company's FAK, mTOR/PI3K and diagnostic programs generally, the timeline for clinical development including projected enrollment of trials, regulatory approval of the Company's compounds, the expected timing for the reporting of data from ongoing trials, the structure of the Company's planned or pending clinical trials and 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 and preliminary data from clinical trials may not be predictive of the results or success of pending or later clinical trials, that data may not be available when we expect it to be, that the Company will not be able to enroll a sufficient number of patients in the expected timeframe, 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.

(A development-stage company)

Unaudited Selected Consolidated Balance Sheet Information

(in thousands)

	September 30, 2013	December 31, 2012
Cash, cash equivalents and investments	$130,270	$91,520
Prepaid expenses and other current assets	1,077	506
Property and equipment, net	674	811
Other assets	408	86
Total assets	**$132,429**	**$92,923**
Accounts payable and accrued expenses	5,293	2,399
Other liabilities	431	58
Stockholders' equity	126,705	90,466
Total liabilities and stockholders' equity	**$132,429**	**$92,923**

Verastem, Inc.

(A development-stage company)

Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended, September 30,		Nine months ended, September 30,	
	2013	2012	2013	2012
Operating expenses:				
Research and development	$6,789	$8,132	$18,130	$17,618
General and administrative	3,855	2,298	11,879	6,636
Total operating expenses	10,644	10,430	30,009	24,254
Loss from operations	(10,644)	(10,430)	(30,009)	(24,254)
Interest income	53	63	131	191
Net loss	($10,591)	($10,367)	($29,878)	($24,063)
Accretion of preferred stock	-	-	-	(6)
Net loss applicable to common stockholders	($10,591)	($10,367)	($29,878)	($24,069)

Net loss per share applicable to common stockholders–basic and diluted	($0.47)	($0.51)	($1.37)	($1.32)
Weighted-average number of common shares used in net loss per share applicable to common stockholders-basic and diluted	22,437	20,160	21,797	18,246

Source: Verastem, Inc.

Verastem, Inc.
Brian Sullivan, 617-252-9314
bsullivan@verastem.com