Verastem Reports Third Quarter 2014 Financial and Corporate Results

October 30, 2014

BOSTON, Mass.--(BUSINESS WIRE)--Oct. 30, 2014-- Verastem, Inc. (NASDAQ: VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, reported financial results for the third quarter ended September 30, 2014, and also provided an overview of certain corporate accomplishments and plans.

"We ended the third quarter with $93.4 million in cash, cash equivalents and investments, which we anticipate will fund our clinical programs into the first half of 2016," said Robert Forrester, President and Chief Executive Officer of Verastem. "I am encouraged by the progress we are making with our clinical programs."

"We continue to see promising clinical signals from the VS-6063 program, as evidenced by the recently announced data from the Window of Opportunity study in surgically-eligible patients with mesothelioma, in which we saw a reduction in a marker of cancer stem cells and encouraging signs of clinical activity," said Dr. Joanna Horobin, Chief Medical Officer of Verastem. "In addition, accrual to our registration-directed COMMAND study for patients with mesothelioma is progressing well."

Verastem has multiple ongoing trials targeting cancer stem cells including the COMMAND study which is evaluating VS-6063, the Company's lead focal adhesion kinase (FAK) inhibitor, in patients with malignant pleural mesothelioma. Malignant pleural mesothelioma is an aggressive form of cancer that occurs in the mesothelium, the thin layer of tissue that covers the lungs. The incidence of mesothelioma is growing worldwide and the survival rate for these patients is very poor.

Q3 2014 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 immediately following frontline therapy in patients with malignant pleural mesothelioma
  - Pursuing simultaneous development in the US, EU, Japan, Canada, Australia and South Africa
  - COMMAND is now open in 12 countries worldwide
  - An interim analysis is expected midyear 2015
- "Window of Opportunity" study
  - Single agent treatment with VS-6063 for 12 days in patients with malignant pleural mesothelioma prior to surgery
  - Presented preliminary data at the 12th International Mesothelioma Interest Group (iMig) Conference in October 2014
    - VS-6063 treatment reduced FAK activity (pFAK-Y397) by an average of 70% in patients evaluated to date
    - VS-6063 reduced a cancer stem cell marker in post-treatment biopsies in 5 out of 7 patients with evaluable paired biopsies
    - Measurement of tumor size using RECIST modified for mesothelioma by CT/PET confirmed that there was no progression of disease while on the 12 day treatment with VS-6063 in any of the 10 patients. Moreover, 2 patients achieved tumor shrinkage consistent with a partial response (-30% and -49%)
    - VS-6063 was well tolerated
    - A protocol amendment has been submitted to expand the study to include an additional 10-15 patients and increase the treatment period from 12 to 35 days
    - The presentation can be viewed here: http://bit.ly/ZSf3zC

VS-4718 (Focal Adhesion Kinase Inhibition)

- VS-4718 is currently being evaluated in a Phase 1 clinical trial in patients with advanced solid tumors
  - Open-label, dose escalation study; designed to assess the safety, pharmacokinetics, pharmacodynamics, maximum tolerated dose and initial clinical activity of single agent VS-4718
- VS-4718 is anticipated to start a Phase 1 dose escalation study in hematological malignancies in Q1 2015
  - Open-label, dose escalation study; designed to assess the safety, pharmacokinetics, pharmacodynamics, maximum tolerated dose and initial clinical activity of single agent VS-4718 in patients with either relapsed or refractory Acute Myeloid or B-Cell Acute Lymphoblastic Leukemia
- Recently published article covering Verastem FAK inhibitors:
  - "FAK in cancer: mechanistic findings and clinical applications." Nat Rev Cancer. 2014 14: 598-610. http://1.usa.gov/1txeMxS. Comprehensive review of the important roles of FAK in cancer. FAK is critical for the survival and

function of bulk tumor cells, cancer stem cells and tumor-associated stromal cells (endothelial cells, tumor-associated macrophages, cancer-associated fibroblasts). This publication reviewed the status of FAK inhibitors in clinical development, including VS-4718 and VS-6063.

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

- VS-5584, a dual mTORC1/2 and PI3K inhibitor, is currently being evaluated in a Phase 1 clinical trial in patients with advanced solid tumors
  - Open-label, dose escalation, schedule finding study; designed to assess the safety, pharmacokinetics, pharmacodynamics, maximum tolerated dose and initial clinical activity of single agent VS-5584
  - Presented data at iMig demonstrating the synergistic activities of VS-5584 and VS-6063 in cellular and animal models of mesothelioma.
  - Expect to initiate a study evaluating the combination of VS-5584 and VS-6063 in patients with relapsed or progressive malignant pleural mesothelioma in Q1 2015

Corporate Events

- Third Annual Research and Development Day
  - Members of the Verastem leadership team, along with a panel of experts, provided in-depth reviews of the Company's development programs targeting cancer stem cells with a focus on VS-6063. The webcast of the presentation can be viewed here: http://bit.ly/1t5F34Z

THIRD QUARTER 2014 FINANCIAL RESULTS

As of September 30, 2014, Verastem had cash, cash equivalents and investments of $93.4 million compared to $123.7 million on December 31, 2013. Verastem used $9.3 million for operating activities in the third quarter ended September 30, 2014 (the "2014 Quarter").

Net loss for the 2014 Quarter was $13.3 million, or $0.52 per share, as compared to net loss of $10.6 million, or $0.44 per share, for the same period in 2013 (the "2013 Quarter"). Net loss includes stock-based compensation expense of $2.8 million for the 2014 Quarter and 2013 Quarter.

Research and development expense for the 2014 Quarter was $9.0 million compared to $6.8 million for the 2013 Quarter. The $2.2 million increase from the 2013 Quarter to the 2014 Quarter was primarily related to an increase of $2.4 million in contract research organization expense for outsourced biology, chemistry, development and clinical services, which includes Verastem's clinical trial costs, an approximate $517,000 increase in personnel costs primarily due to increased headcount, an approximate increase of $237,000 in occupancy expense partially due to the relocation to its new facility and an approximate $181,000 increase in consulting fees. These increases were partially offset by an approximate $765,000 decrease in license fees related primarily to the Poniard and S*Bio milestones incurred in the 2013 Quarter and an approximate decrease of $280,000 in lab supplies partially due to lower activity as Verastem transitioned to the new facility.

General and administrative expense for the 2014 Quarter was $4.3 million compared to $3.9 million for the 2013 Quarter. The approximately $400,000 increase from the 2013 Quarter to the 2014 Quarter primarily resulted from an increase of approximately $241,000 in personnel costs primarily related to increases in salaries and headcount, an increase in consulting fees of approximately $90,000 primarily related to preparation for commercialization and an increase in stock-based compensation of approximately $71,000 due to an increase in stock option grants.

The number of outstanding common shares as of September 30, 2014, was 25,878,418.

Financial Guidance

Based on current operating plans, we expect to have sufficient cash, cash equivalents and investments to fund our research and development programs and operations into the first half of 2016.

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 by Robert Weinberg, Ph.D., scientific cofounder and chair of Verastem's Scientific Advisory Board, and Verastem 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, and a trial in patients with Kras-mutated non-small cell lung cancer. VS-6063 has been granted orphan drug designation in the U.S. and EU for use in 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 Phase 1 dose escalation trial of VS-5584 in patients with advanced solid tumors.

About COMMAND

COMMAND is a registration-directed, double-blind, placebo-controlled trial of VS-6063 in patients with malignant pleural mesothelioma. The primary endpoints of COMMAND are progression free survival (PFS) and overall survival (OS). VS-6063 targets cancer stem cells which are an underlying cause of tumor progression and recurrence. The design of COMMAND allows the opportunity to enrich for patients with tumors low in the biomarker, merlin. Preclinical and early clinical research has demonstrated that low merlin levels may be predictive of increased effectiveness of FAK inhibitors such as VS-6063. The COMMAND study stratifies patients to evaluate the effect of VS-6063 in both the overall patient population and the subgroup of patients whose tumors are low in merlin.

COMMAND is expected to enroll approximately 350-400 patients at clinical sites in 12 countries, including the US, UK, Japan, Australia, Canada, South Africa, New Zealand and countries in mainland Europe. Eligible patients who had a partial response or stable disease following standard first-line therapy with platinum/pemetrexed will be stratified to merlin low or high and then randomized to receive either placebo or 400 mg of defactinib. For more information visit www.COMMANDmeso.com.

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 inhibition program, PI3K/mTOR and diagnostics programs generally, the timeline for clinical development and 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 estimates of how long its existing cash, cash equivalents and investments will 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 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 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, 2013 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)

	September 30, 2014	December 31, 2013
Cash, cash equivalents and investments	$ 93,366	$ 123,656
Prepaid expenses and other current assets	1,110	643
Property and equipment, net	2,885	631
Other assets	522	331
Total assets	**$ 97,883**	**$ 125,261**
Accounts payable and accrued expenses	$ 8,160	$ 7,087
Other liabilities	912	728
Stockholders' equity	88,811	117,446
Total liabilities and stockholders' equity	**$ 97,883**	**$ 125,261**

Verastem, Inc.

Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended September 30,		Nine months ended September 30,	
	2014	2013	2014	2013
Operating expenses:				
Research and development	$ 9,047	$ 6,789	$ 25,763	$ 18,130
General and administrative	4,341	3,855	13,846	11,879
Total operating expenses	13,388	10,644	39,609	30,009
Loss from operations	(13,388)	(10,644)	(39,609)	(30,009)
Interest income	56	53	193	131
Net loss	($13,332)	($10,591)	($39,416)	($29,878)
Net loss per share applicable to common stockholders–basic and diluted	($0.52)	($0.44)	($1.54)	($1.37)
Weighted-average number of common shares used in net loss per share applicable to common stockholders-basic and diluted	25,811	24,127	25,654	21,797

Source: Verastem, Inc.

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