Verastem Reports Second Quarter 2014 Financial and Corporate Results

August 7, 2014

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 7, 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 second quarter ended June 30, 2014, and also provided an overview of certain corporate accomplishments and plans.

“We ended the second quarter with a cash, cash equivalents and investments balance of $104.4 million which we expect will fund our clinical programs into the first half of 2016,” said Robert Forrester, President and Chief Executive Officer of Verastem. “By targeting cancer stem cells, we want to change the way that cancer is treated. Through diligent execution by our research and development team, we continue to advance our portfolio of cancer stem cell targeting agents through clinical development.”

“We continue to see encouraging clinical signals from the VS-6063 program, including reductions in markers of cancer stem cells, prolonged disease stabilization and objective responses in the ongoing Phase 1/1b combination study of VS-6063 and paclitaxel in patients with ovarian cancer,” said Dr. Joanna Horobin, Chief Medical Officer of Verastem. “Our registration-directed COMMAND study for patients with mesothelioma is progressing well and is now up and running in 12 countries. The planned interim analysis of COMMAND remains on track for midyear 2015.”

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. Mesothelioma is an aggressive form of lung cancer believed to be driven by cancer stem cells, which are an underlying cause of resistance to anticancer therapies, disease recurrence and metastasis. The incidence of mesothelioma is growing worldwide and the survival rate for these patients is very poor.

Q2 2014 AND RECENT HIGHLIGHTS:

Mesothelioma

- COMMAND (Control Of Mesothelioma with MAinteNance Defactinib) Study
  - Registration-directed, randomized, double-blind, placebo-controlled study of VS-6063 immediately following frontline therapy in patients with malignant pleural mesothelioma
  - With the addition of Japanese sites, we are pursuing simultaneous development in the US, EU, Japan, Australia and other regions of mesothelioma incidence
  - 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

Ovarian Cancer

- Phase 1/1b study of VS-6063 in combination with weekly paclitaxel
  - Presented interim data at the American Society of Clinical Oncology (ASCO) 2014 and a clinical update at the Company’s Research and Development Day

    - Interesting signs of early clinical activity; 64% best response of stable disease or better including two complete responses and three partial responses in the ongoing study

    - Combination therapy was generally well-tolerated with no dose limiting toxicities

    - Treatment with VS-6063 as a single agent for 10 days decreased both FAK activity and cancer stem cells in patient biopsies

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 lead candidate, VS-6063
- A replay of the webcast can be accessed here or by visiting the Verastem website

Analyst Event at ASCO 2014

- Provided a scientific update on the COMMAND study and discussed the rationale for targeting cancer stem cells in mesothelioma
- A replay of the event webcast can be accessed here or by visiting the Verastem website

Increased the Understanding of Cancer Stem Cell Biology

- Presented posters at the ASCO Annual Meeting. In addition to the interim data that were presented for the ongoing trial of VS-6063 in combination with paclitaxel in patients with ovarian cancer, Verastem presented posters describing the trial designs for two of the Company's other ongoing clinical trials: one for the registration-directed COMMAND study of VS-6063 for patients with malignant pleural mesothelioma, and the other for the study of VS-6063 for patients with non-small cell lung cancer. The posters presented at ASCO can be accessed here.
- Presented research results at the 2014 American Academy of Cancer Research (AACR) Annual Meeting. The presented data expanded understanding of the mechanisms of VS-6063, VS-4718 and VS-5584 and their ability to target cancer stem cells. The data also highlighted VS-6063's inhibitory effect on focal adhesion kinase family members FAK and PYK2 leading to the preferential targeting of cancer stem cells both directly and through inhibition of tumor-associated macrophages in the tumor microenvironment. Published research studies in both mesothelioma and breast cancer have demonstrated a correlation between an increase in tumor-associated macrophages and poor prognosis in these patients. The posters presented at AACR can be accessed here.

Strengthened Leadership Team and Intellectual Property Portfolio

- Appointed industry veteran Paul A. Friedman, M.D. to the Verastem Board of Directors. Dr. Friedman previously served as Chief Executive Officer of Incyte Corporation, where he oversaw the development and commercialization of Jakafi®. During his career, he also served in leadership positions at DuPont Pharmaceuticals and Merck Research Laboratories and was involved in the discovery and/or development of a number of successful pharmaceutical products, including Aggrastat®, Trusopt®, Crixivan®, Sustiva®, Pedvax®, Pneumovax®, Vaqta®, Varivax® Cozaar®/Hyzaar® and Fosamax®. Dr. Friedman earned his M.D. from Harvard Medical School where he then became an Associate Professor of Medicine and Pharmacology and was a practicing physician at New York-Presbyterian Hospital, College of Physicians and Surgeons.
- Granted US Patent No. 8,754,080 and European Patent No. 2,414,362 titled "Pyrimidine Substituted Purine Compounds As Kinase(s) Inhibitors" that cover the composition of matter for VS-5584 and its ability to inhibit and regulate cellular metabolism, growth, and proliferation.

SECOND QUARTER 2014 FINANCIAL RESULTS

As of June 30, 2014, Verastem had cash, cash equivalents and investments of $104.4 million compared to $123.7 million on December 31, 2013. Verastem used $8.7 million for operating activities in the second quarter ended June 30, 2014 (the "2014 Quarter").

Net loss for the 2014 Quarter was $13.0 million, or $0.51 per share, as compared to net loss of $10.3 million, or $0.49 per share, for the same period in 2013 (the "2013 Quarter"). Net loss includes stock-based compensation expense of $3.2 million and $2.7 million for the 2014 Quarter and 2013 Quarter, respectively.

Research and development expense for the three months ended June 30, 2014 (2014 Quarter) was $8.3 million compared to $6.0 million for the three months ended June 30, 2013 (2013 Quarter). The $2.3 million increase from the 2013 Quarter to the 2014 Quarter was primarily related to an increase of $1.9 million in contract research organization expense for outsourced biology, chemistry, development and clinical services, which includes our clinical trial costs, an approximate $302,000 increase in personnel costs primarily due to increased headcount and approximately $126,000 increase in consulting fees.

General and administrative expense for the 2014 Quarter was $4.8 million compared to $4.2 million for the 2013 Quarter. The approximately $600,000 increase from the 2013 Quarter to the 2014 Quarter primarily resulted from an increase of approximately $529,000 in stock-based compensation expense, an increase in consulting fees of approximately $438,000, an increase of approximately $318,000 in personnel costs and an increase in travel and other costs of approximately $169,000. These increases were partially offset by a decrease in professional fees and other costs of approximately $701,000 and an approximate $210,000 decrease in corporate franchise taxes.

The number of outstanding common shares as of June 30, 2014, was 25,842,328.

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 and lymphomas.

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, and the structure of the Company's planned or pending clinical trials. 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 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)

	June 30, 2014	December 31, 2013
Cash, cash equivalents and investments	$104,352	$123,656
Prepaid expenses and other current assets	963	643
Property and equipment, net	1,489	631
Other assets	538	331
Total assets	**$107,342**	**$125,261**
Accounts payable and accrued expenses	$7,324	$7,087
Other liabilities	839	728
Stockholders' equity	99,179	117,446
Total liabilities and stockholders' equity	**$107,342**	**$125,261**

Verastem, Inc.

Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended June 30,		Six months ended June 30,	
	2014	2013	2014	2013
Operating expenses:				
Research and development	$8,305	$6,045	$16,716	$11,341
General and administrative	4,782	4,239	9,505	8,024
Total operating expenses	13,087	10,284	26,221	19,365
Loss from operations	(13,087)	(10,284)	(26,221)	(19,365)
Interest income	65	34	137	78
Net loss	($13,022)	($10,250)	($26,084)	($19,287)
Net loss per share – basic and diluted	($0.51)	($0.49)	($1.02)	($0.94)
Weighted-average number of common shares used in net loss per share – basic and diluted	25,669	20,729	25,574	20,607

Source: Verastem, Inc.

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