Verastem Reports First Quarter 2014 Financial and Corporate Results

May 8, 2014

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 8, 2014-- 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, 2014, and also provided an update of certain corporate accomplishments and plans.

“We continue to advance our development programs targeting cancer stem cells,” said Robert Forrester, President and Chief Executive Officer of Verastem. “We have the financial stability, product candidates and experienced team to execute on our clinical and corporate goals. We are committed to our mission of bringing new treatment options to patients with cancer.”

Verastem has multiple trials ongoing targeting cancer stem cells including the COMMAND study for patients with malignant mesothelioma. Mesothelioma is an aggressive form of lung cancer believed to be driven by cancer stem cells. Cancer stem cells are an underlying cause of cancer progression and recurrence. The incidence of mesothelioma is rapidly growing worldwide and the survival rate for these patients is very poor.

“In March, we reported on a Phase 1 trial in Japanese patients where VS-6063 was generally well tolerated and the safety and pharmacokinetic profile was comparable to that seen in the US Phase 1 trial,” said Dr. Joanna Horobin, Chief Medical Officer of Verastem. “We have presented these data to the Japanese regulatory authorities and plan to open COMMAND clinical sites in Japan this year. In 2014, we also expect to finalize the results of the Japanese Phase 1 trial and to present interim data on the combination study of VS-6063 with weekly paclitaxel in patients with ovarian cancer, the Phase 2 study in patients with non-small cell lung cancer, and the Phase 1 studies of VS-4718 and VS-5584.”

Q1 2014 and Recent Accomplishments

- **COMMAND (Control Of Mesothelioma with MAinteNance Defactinib)**
  - -- Registration-directed, randomized, double blind, placebo controlled study of VS-6063 immediately following frontline therapy in patients with malignant pleural mesothelioma
  - -- Open in 11 countries worldwide
- **Progressed the FAK inhibition program (VS-6063 and VS-4718)**
  - -- Reported preliminary data from the Phase 1 study of VS-6063 in Japanese patients
    - -- VS-6063 was well tolerated at all dose levels; no SAEs or evidence of dose-limiting toxicity
    - -- Confirmed the recommended Phase 2 dose as 400mg BID, consistent with dosing in other countries
    - -- Safety and pharmacokinetic results support the advancement of the VS-6063 development program in Japanese patients
  - -- Acquired additional license rights to VS-4718 from Encarta, reducing future milestones and royalties associated with ongoing development
- **Strengthened the dual PI3K/mTOR inhibition program (VS-5584)**
  - -- Granted new Japanese patent titled “Pyrimidine Substituted Purine Compounds As Kinase(s) Inhibitors” with claims covering the composition of matter for VS-5584 and VS-5584’s ability to inhibit and regulate cellular metabolism, growth, and proliferation
- **Increased the understanding of cancer stem cell biology**
  - -- Presented research results at the 2014 American Academy of Cancer Research (AACR) Annual Meeting. The data presented at AACR expanded understanding of the mechanisms of VS-6063, VS-4718 and VS-5584 and their ability to target cancer stem cells. Of significant interest is that VS-6063 inhibits the focal adhesion kinase family members FAK and PYK2 which leads to the preferential targeting of cancer stem cells both directly and through inhibition of tumor-associated macrophages in the tumor microenvironment. Published evidence in both

mesothelioma and breast cancer has 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: http://bit.ly/R3M6wc

-- Published data in Cancer Research on NF2 and merlin biology in cancer stem cells found in mesothelioma. The paper is titled “Tumor Suppressor Alterations Cooperate to Drive Aggressive Mesotheliomas with Enriched Cancer Stem Cells via a p53–miR-34a–c-Met Axis.” The study results demonstrated that inactivation of specific tumor suppressors cooperate to drive the development of highly aggressive mesothelioma characterized by enhanced disease progression and an increase in cancer stem cells. The paper can be accessed here: http://bit.ly/1figRWX

-- Research was published in Nature Immunology highlighting the potential role of FAK inhibition in hematological malignancies. The paper can be accessed here: http://bit.ly/1bKYJ6M

- **Strengthened leadership team**

-- Appointed Timothy J. Barberich to the Board of Directors. Mr. Barberich founded Sepracor in 1984 and served as CEO and Chairman for more than 20 years. Under his leadership at Sepracor, revenues grew to more than a billion dollars as the company partnered and commercialized a number of successful products, including Allegra®, Clarinex®, Lunesta® and Xopenex®.

2014 Clinical Milestones

Verastem’s planned upcoming clinical milestones include the following:

- Report interim data from the Phase 1/1b trial of VS-6063 in combination with weekly paclitaxel in patients with ovarian cancer at the 50th Annual American Society of Clinical Oncologists (ASCO) meeting
- Open COMMAND study sites in Japan in H2 2014
- Report the interim analysis for the Phase 2 study of VS-6063 in patients with Kras-mutated NSCLC in H2 2014
- Report interim data on the Phase 1 trial of VS-4718 in H2 2014
- Report interim data on the Phase 1 trial of VS-5584 in H2 2014

Upcoming Events

ASCO Breakfast

Sunday, June 1, 2014, at 6:30am CT at the Hyatt Regency McCormick Place, Chicago, IL. Special guest Professor Dean Fennell, Ph.D., FCRP, Chair of Thoracic Oncology, University of Leicester and Incoming President of the International Mesothelioma Interest Group, will be presenting together with Chief Medical Officer, Joanna Horobin, MB, ChB, and Head of Research, Jonathan Pachter, Ph.D. Topics will include a scientific update on the COMMAND study and the rationale for targeting cancer stem cells in mesothelioma. RSVP to verastem@argotpartners.com

Research and Development Day

Thursday, July 10, 2014, at 12:30pm ET at the NASDAQ Marketsite in New York, NY. Verastem will provide updates on the status of research and development, anticipated clinical milestones and upcoming plans. Special guest speakers to include Jose Baselga, M.D., Ph.D., Physician in Chief, Memorial Sloan Kettering Cancer Center, Professor Dean Fennell, Ph.D., FCRP, Chair of Thoracic Oncology, University of Leicester, Mary Hesdorffer, N.P., Executive Director, Mesothelioma Applied Research Foundation. RSVP to verastem@argotpartners.com

First Quarter 2014 Financial Results

As of March 31, 2014, Verastem had cash, cash equivalents and investments of $113.9 million compared to $123.7 million on December 31, 2013. Verastem used $9.3 million for operating activities in the first quarter ended March 31, 2014 (the “2014 Quarter”).

Net loss for the 2014 Quarter was $13.1 million, or $0.51 per share, as compared to net loss of $9.0 million, or $0.44 per share, for the same period in 2013 (the “2013 Quarter”). Net loss includes stock-based compensation expense of $3.6 million and $2.5 million for the 2014 Quarter and 2013 Quarter, respectively.

Research and development expense for the 2014 Quarter was $8.4 million compared to $5.3 million for the 2013 Quarter. The $3.1 million increase from the 2013 Quarter to the 2014 Quarter is primarily related to an increase of $1.3 million in contract research organization expense for outsourced biology, development and clinical services, which includes our clinical trial costs, a $1.2 million dollar increase in license fees related to the Encarta asset purchase, an approximate $419,000 increase in stock-based compensation expense and an approximate $200,000 increase in personnel costs primarily due to increased average headcount.

General and administrative expense for the 2014 Quarter was $4.7 million compared to $3.8 million for the 2013 Quarter. The approximately $900,000 increase from the 2013 Quarter to the 2014 Quarter primarily resulted from an approximate increase of $647,000 in stock-based compensation expense associated with restricted stock units, an approximate $215,000 increase in personnel costs primarily due to increase in salaries and headcount and an increase in consulting fees of approximately $151,000. These increases were partially offset by a decrease in professional fees of approximately $191,000.

The number of outstanding common shares as of March 31, 2014, was 25,834,945.

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 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 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 (www.COMMANDmeso.com), a Phase 1/1b study in combination with paclitaxel for patients with ovarian cancer, a Phase 1 study in Japan in patients with advanced solid tumors and a Phase 2 trial in patients with 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). 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 with Progression Free Survival (PFS) and Overall Survival (OS) as the primary endpoints. 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, 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, VS-4718 and VS-5584 and the Company's FAK and mTOR/PI3K inhibition and diagnostic programs generally, the timeline for clinical development and regulatory approval of the Company's compounds, including the potential for opening COMMAND trial sites in Japan, the expected timing for the reporting of data from ongoing trials, and the structure of the Company's planned or pending clinical trials including estimates for enrollment, and potential indications for clinical development 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 ongoing or later clinical trials, that data may not be available when we expect it to be, that enrollment of clinical trials will 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 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)

	March 31,	December 31,

	2014	2013
Cash, cash equivalents and investments	$ 113,876	$ 123,656
Prepaid expenses and other current assets	943	643
Property and equipment, net	577	631
Other assets	330	331
Total assets	**$ 115,726**	**$ 125,261**
Accounts payable and accrued expenses	$ 6,100	$ 7,087
Other liabilities	301	728
Stockholders' equity	109,325	117,446
Total liabilities and stockholders' equity	**$ 115,726**	**$ 125,261**

Verastem, Inc.

(A development-stage company)

Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended, March 31,	
	2014	2013
Operating expenses:		
Research and development	$ 8,411	$ 5,296
General and administrative	4,723	3,785
Total operating expenses	13,134	9,081
Loss from operations	(13,134)	(9,081)
Interest income	72	44
Net loss	($13,062)	($9,037)
Net loss per share	($0.51)	($0.44)
Weighted-average number of common shares used in net loss per share	25,478	20,483

Source: Verastem, Inc.

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