



## Verastem Reports Year-End 2013 Financial Results

March 6, 2014

*--A year of clinical execution: Three small molecule compounds in clinical development including the registration-directed COMMAND study--*

*--Multiple clinical data readouts anticipated starting in first half 2014--*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 6, 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 year ended December 31, 2013, and also commented on corporate accomplishments and plans.

"In 2013, Verastem achieved critical milestones in our mission to bring new therapies targeting cancer stem cells to patients," said Robert Forrester, President and Chief Executive Officer of Verastem. "We are conducting clinical trials with three product candidates that target cancer stem cells in multiple indications and geographies. Looking forward to 2014, we have multiple clinical data readouts planned throughout the year."

Verastem reported yesterday the successful outcome of its Phase 1 study of lead Focal Adhesion Kinase (FAK) inhibitor, VS-6063, in Japanese subjects. The study results demonstrated that VS-6063 was well tolerated and side effects were consistent with previously reported results from the US Phase 1 trial. The trial is ongoing with three patients still on study. The positive results of this trial support the application to the Japanese PMDA for the potential initiation of additional COMMAND clinical study sites in Japan during the second half of 2014.

"The COMMAND study is accruing patients in many countries worldwide," said Dr. Joanna Horobin, Chief Medical Officer of Verastem. "While the exact timing of the interim analysis is dependent on enrollment and response to treatment with VS-6063, we currently anticipate this to be midyear 2015. Our goal is to pursue parallel development in the major markets, including the US, Europe and Japan."

In 2013, Verastem reported at the EORTC/NCI/AACR meeting the successful completion of a dose escalation study evaluating VS-6063 in combination with weekly paclitaxel in patients with ovarian cancer. The expansion phase of the study is now fully accrued and Verastem plans to report preliminary data on these patients in the second quarter of 2014.

Verastem anticipates several data readouts from clinical trials in 2014. In addition to the paclitaxel combination study, Verastem is expecting to complete an interim analysis of the ongoing VS-6063 study in Kras-mutated non-small cell lung cancer (NSCLC) in the second half of the year. Verastem also anticipates giving interim updates on the Phase 1 trials of VS-4718 and VS-5584 in the latter part of 2014.

"We ended 2013 on firm financial ground with a cash, cash equivalents and investments balance of approximately \$124 million which we anticipate will enable the continued execution of our clinical programs into the first half of 2016," said Christoph Westphal, M.D., Ph.D., Verastem Executive Chairman. "We believe that we have the opportunity to make a significant difference in many patients' lives through the preferential targeting of cancer stem cells in both solid tumors and hematological malignancies like B-ALL. It is our mission to deliver a greater clinical benefit for patients and create value for our shareholders by changing the way cancer is treated."

### 2013 and Recent Accomplishments

- **COMMAND (Control Of Mesothelioma with MAIntenance Defactinib)**
  - Initiation of a registration-directed, randomized, double blind, placebo controlled study of VS-6063 immediately following frontline therapy in patients with malignant pleural mesothelioma
  - Met with regulatory agencies in the United States and United Kingdom and interacted with the regulatory agencies in an additional 10 countries
  - Accruing patients at 28 clinical sites
  - Received orphan drug designation for VS-6063 in mesothelioma in both the U.S. and Europe
- **Progressed the FAK inhibition program (VS-6063 and VS-4718)**
  - Reported the successful completion of the dose-escalation portion of VS-6063 in combination with weekly paclitaxel in patients with ovarian cancer.
  - Completed accrual of the expansion phase of the study of VS-6063 in combination with weekly paclitaxel at the recommended Phase 2 dose
  - Successfully accrued a Phase 1 study of VS-6063 in Japanese patients and confirmed the recommended Phase 2 dose as 400mg BID, consistent with dosing in other countries
  - Initiated a Phase 2 study of VS-6063 in patients with Kras-mutated NSCLC with or without secondary alterations in either p16 or p53 genes
  - Initiated a Phase 1 dose escalation study of VS-4718 in patients with advanced solid tumors
  - Conducted research for the use of FAK inhibitors in Ikaros-loss of function B-cell Acute Lymphoblastic Leukemia
  - Eliminated significant potential milestone and royalty payments for VS-4718 by acquiring product rights from previous licensor
- **Progressed the dual PI3K/mTOR inhibition program (VS-5584)**
  - Initiated a Phase 1 dose escalation study of VS-5584 in patients with advanced solid tumors and lymphomas

- **Expanded the research collaboration with Eisai**
  - In response to encouraging results, the company extended the research collaboration with Eisai focused on generating novel inhibitors of Wnt/ $\beta$ -catenin signaling
- **Increased the understanding of cancer stem cell biology**
  - Presented research results at major scientific conferences including AACR, ASCO, EORTC, World Lung, and SABCS
  - Published data on NF2 and merlin biology in cancer stem cells found in mesothelioma in *Cancer Research*
- **Strengthened our development advisory team**
  - Named Jose Baselga, M.D., Ph.D., Physician in Chief at Memorial Sloan Kettering Cancer Center, as Senior Medical Advisor. Dr. Baselga is a world-renowned expert in breast cancer and PI3K-inhibitor translational research
- **Enhanced corporate stability**
  - Hired Jack Green as Chief Financial Officer. Mr. Green was the Senior Vice President and Chief Financial Officer of GTC Biotherapeutics (formerly Genzyme Transgenics Corporation), and is a Certified Public Accountant (CPA) with over 30 years of financial experience, including 20 within the biotechnology industry
  - Completed a secondary offering raising \$63.8 million in gross proceeds

## 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 during Q2 2014**
- **Update of projected timing of COMMAND interim analyses at R&D Day on July 10<sup>th</sup> at 12:30pm ET in New York, NY**
- **Assuming approval from regulatory authorities, initiate COMMAND study centers 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**

## Full Year 2013 Financial Results

As of December 31, 2013, Verastem had cash, cash equivalents and investments of \$123.7 million compared to \$91.5 million on December 31, 2012. The number of outstanding common shares as of February 28, 2014 was 25,822,939.

Net loss for the year ended December 31, 2013 ("2013 Period") was \$41.2 million, or \$1.82 per share applicable to common stockholders, as compared to \$32.0 million, or \$1.70 per share applicable to common stockholders, for the year ended December 31, 2012 ("2012 Period"). Net loss for 2013 includes non-cash stock-based compensation expense of \$10.4 million for the year ended December 31, 2013, as compared to \$7.4 million for the year ended December 31, 2012.

Research and development expense for the year ended December 31, 2013 was \$25.9 million compared to \$21.7 million for the year ended December 31, 2012. The \$4.2 million increase from the 2012 Period to the 2013 Period was primarily related to an increase of \$4.9 million in contract research organization expense for outsourced biology, chemistry and development services, which includes our clinical trial costs, a \$1.8 million increase in personnel costs primarily due to increased average headcount, an approximate \$619,000 increase in stock-based compensation expense and an increase of approximately \$368,000 in travel fees primarily due to increased travel associated with our clinical trials. These increases were partially offset by a decrease of \$3.5 million in license fee expense primarily due to the upfront payment in the 2012 Period related to our agreement with Pfizer, Inc.

General and administrative expense for the 2013 Period was \$15.5 million compared to \$10.5 million for the 2012 Period. The \$5.0 million increase from the 2012 Period to the 2013 Period primarily resulted from an increase of \$2.4 million in stock-based compensation expense, an increase in professional fees and other costs of \$1.5 million, an increase in consulting fees of approximately \$514,000, an approximate \$274,000 increase in corporate franchise taxes and an approximate \$339,000 increase in personnel costs primarily due to an increase in salaries and headcount.

## Financial Guidance

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

## Conference Call Information

The Verastem management team will host a conference call discussing the Company's financial results, recent developments and management's outlook for 2014 on Thursday, March 6, 2014, at 8:00 AM (ET). The call can be accessed by dialing 1-866-953-6857 five minutes prior to the start of the call and providing the passcode 51092744. A replay will be available approximately two hours after the completion of the call and can be accessed by dialing 1-888-286-8010 and providing the passcode 64460610. The replay will be available for two weeks from the date of the live call.

The live, listen-only webcast of the conference call can be accessed by visiting the investors section of the Company's website at [www.verastem.com](http://www.verastem.com). A replay of the webcast will be archived on the Company's website for 90 days following the call.

## 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 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](http://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 Europe 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 pathways. 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 is currently conducting a Phase 1 dose escalation trial of VS-5584 in patients with advanced solid tumors and lymphomas.

#### 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](http://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, 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, 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 the Japanese regulatory authorities will not approve the initiation of the COMMAND trial, 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.

#### (A development-stage company)

#### Unaudited Condensed Consolidated Balance Sheets

(in thousands)

	December 31,	
	2012	2013
Cash, cash equivalents and investments	\$ 91,520	\$ 123,656
Prepaid expenses and other current assets	506	643
Property and equipment, net	811	631
Other assets	86	331
<b>Total assets</b>	<b>\$ 92,923</b>	<b>\$ 125,261</b>
Accounts payable and accrued expenses	\$ 2,399	\$ 7,087
Other liabilities	58	728
Stockholders' equity	90,466	117,446
<b>Total liabilities and stockholders' equity</b>	<b>\$ 92,923</b>	<b>\$ 125,261</b>

#### Verastem, Inc.

#### (A development-stage company)

#### Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Year ended December 31,	
	2012	2013
Operating expenses:		
Research and development	\$ 21,712	\$ 25,930
General and administrative	10,518	15,472
Total operating expenses	32,230	41,402
Loss from operations	(32,230 )	(41,402 )
Interest income	246	200
Net loss	(\$31,984 )	(\$41,202 )
Accretion of preferred stock	(6 )	—
Net loss applicable to common stockholders	(\$31,990 )	(\$41,202 )
Net loss per share applicable to common stockholders—basic and diluted	(\$1.70 )	(\$1.82 )
Weighted-average number of common shares used in net loss per share applicable to common stockholders-basic and diluted	18,765	22,680

Source: Verastem, Inc.

**Verastem, Inc.**

Brian Sullivan, 617-252-9314

[bsullivan@verastem.com](mailto:bsullivan@verastem.com)