# Verastem Reports Year-End 2014 Financial Results

March 10, 2015

*— COMMAND Interim Analysis Expected Second Quarter 2015 —*

BOSTON--(BUSINESS WIRE)--Mar. 10, 2015-- 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, 2014, and also commented on corporate accomplishments and plans.

"COMMAND enrollment remains on track and we continue to anticipate conducting the pre-specified interim analysis in the second quarter of 2015," said Robert Forrester, President and Chief Executive Officer of Verastem. "Proceeds from the recently completed equity financing, which raised approximately $51 million in net proceeds, strengthened our balance sheet, expanded our shareholder base and provide us with the capital to reach the primary endpoint of the COMMAND study and fund our current development programs and operations into the first half of 2017. It is an exciting time at Verastem and we are pleased to see that these investors share our confidence in, and enthusiasm for, our development programs targeting cancer stem cells – an approach that has the potential to change the way cancer is treated."

At the interim analysis of COMMAND, pre-specified efficacy analyses will be conducted and all safety data will be examined by the independent Data Safety Monitoring Board. A decision will be made whether to stop the study early for futility, continue the study as planned in all patients, or enrich the study population based upon the biomarker merlin. This important milestone will define the primary patient population for the registration-directed study.

Verastem held a detailed 2014 Year-end Review and 2015 Update on January 8, 2015. A webcast of the presentation can be accessed through the following link: http://bit.ly/1vYH8yN

**2014 and Recent Accomplishments**

**VS-6063 (Oral Focal Adhesion Kinase Inhibitor)**

- COMMAND (**C**ontrol **O**f **M**esothelioma with **MA**inte**N**ance **D**efactinib) Study currently enrolling in 13 countries

-- Registration-directed, randomized, double-blind, placebo-controlled study of VS-6063 as a switch maintenance treatment in patients with malignant pleural mesothelioma benefiting from frontline therapy with pemetrexed (Alimta) and platinum

-- Primary endpoints are Progression Free Survival (PFS) and Overall Survival (OS). A sample size of 372 subjects will provide 90% power to assess the superiority of PFS, a co-primary efficacy endpoint, with a 1 sided type I error rate of 0.025, assuming a hazard ratio of 0.67.

-- 180 patients enrolled at 55 centers in 13 countries as of January 8, 2015. The study is currently accruing on track to complete enrollment by the end of 2015.

-- As of January 8, 2015, 41% of the 180 patients enrolled have merlin low tumors, which is consistent with the Company's assumptions

-- Interim analysis to define the primary patient population expected in Q2 2015

- Encouraging data from our Phase 2 clinical trial evaluating single agent VS-6063 in patients with previously treated KRAS-mutated non-small cell lung cancer

-- Study is being conducted at 9 U.S. sites

-- The two cohorts that have been fully enrolled have also crossed the interim analysis threshold of greater than or equal to 4 patients, out of 11, with greater than or equal to 12 week PFS

-- All cohorts have patients still on study

-- Disease control exceeding 6 months for some patients

-- Long-term use is generally well tolerated

-- We have stopped accrual to the study and are evaluating designs of potential follow-up trials

-- Expect to submit study results for presentation at a scientific meeting in H2 2015

- Reported updated Phase 1/1b data for VS-6063 in combination with paclitaxel in ovarian cancer

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

-- Early signs of clinical activity; 64% best response of stable disease at 8 weeks or better including two complete responses and three partial responses

-- Treatment with VS-6063 resulted in decreased pFAK activity and a reduction in markers of cancer stem cells in 4 of 5 patient biopsies following 10 days of VS-6063 treatment

-- Expect to report an update on the study in H2 2015

- Reported preliminary data from the biomarker “Window of Opportunity” study in mesothelioma

-- Single agent VS-6063 was given for 12 days, with pre- and post-treatment biopsies, to patients with malignant pleural mesothelioma prior to surgery

-- VS-6063 was generally well tolerated and reduced FAK activity (pFAK-Y397) by an average of 70% in patients evaluated to date and reduced markers of cancer stem cells 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

-- Tumor shrinkage consistent with a partial response (-30% and -49%) was seen in 2 patients

-- The study has been amended to increase the VS-6063 treatment period from 12 to 35 days: currently enrolling an additional 10-15 patients

-- Expect to report preliminary data on the extended treatment cohort in H1 2016

- Completed Phase 1 in Japanese patients; reported preliminary data

-- VS-6063 was generally well tolerated at all dose levels; no serious adverse events or dose-limiting toxicity

-- Confirmed the recommended Phase 2 dose as 400mg BID, consistent with dosing in other patient populations

-- Opened Japanese sites for the COMMAND study which facilitates a parallel regulatory pathway with other countries in development worldwide

-- The Japanese subjects included 1 patient with relapsed mesothelioma who had a symptom improvement and PFS of 5.6 months

**VS-4718 (Oral Focal Adhesion Kinase Inhibitor)**

- Phase 1 clinical trial ongoing 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

-- Maximum tolerated dose has not yet been reached

-- Generally well tolerated with patients on treatment for over 6 months

-- Two patients with mesothelioma have had disease control for greater than 5 months

-- Expect to report preliminary data in H2 2015

- Reported supportive preclinical data at AACR, EORTC, and ASH
- Published supportive preclinical data in the journals Science Translational Medicine and Blood
- Acquired additional license rights to VS-4718, reducing future milestones and royalties associated with ongoing development

**VS-5584 (Oral Dual mTORC 1/2 and PI3K Inhibitor)**

- Phase 1 dose escalation clinical trial ongoing in patients with advanced solid tumors

-- Open-label, dose and schedule finding study; designed to assess the safety, pharmacokinetics, pharmacodynamics, maximum tolerated dose and initial clinical activity of single agent VS-5584

-- Generally well tolerated and the expected on-target toxicities are clinically manageable

-- Maximum tolerated dose has not yet been reached

-- Clinical activity observed in multiple tumor types, including mesothelioma; observed disease control of over 6 months

-- Expect to report preliminary data in H2 2015

- Presented supportive preclinical data at iMig demonstrating the synergistic activities of VS-5584 and VS-6063 in cellular and animal models of mesothelioma
- Initiated Phase 1 clinical trial evaluating combination of VS-5584 and VS-6063 in relapsed mesothelioma in Q1 2015
- Received orphan medicinal product/drug designation from the FDA and European Commission for use in mesothelioma
- Reported supportive preclinical data at AACR and EORTC and published supportive preclinical data in Molecular Cancer Therapeutics and Cancer Research
- Japanese patent issued with claims covering the composition of matter and VS-5584’s ability to inhibit and regulate cellular metabolism, growth, and proliferation

**Financial/Corporate**

- Successfully completed public offering of 8,337,500 shares of common stock with net proceeds totaling $50.9 million
- Industry veterans, Timothy J. Barberich, former CEO and founder of Sepracor, and Paul A. Friedman, M.D., former CEO of Incyte Corporation (NASDAQ: INCY), appointed to Verastem Board of Directors
- Daniel Paterson, former Verastem Chief Business Officer, promoted to Chief Operating Officer

**Summary of Upcoming Milestones**

Verastem's planned upcoming milestones include:

**VS-6063**

- Report COMMAND interim analysis: Q2 2015
- Report Phase 2 results in KRAS-mutated NSCLC: H2 2015
- Report updated results from the VS-6063/paclitaxel combination in patients with ovarian cancer: H2 2015
- Report on the biomarker "Window of Opportunity" study with preliminary results from the extended treatment cohort: H1 2016

**VS-4718**

- Report preliminary Phase 1 results in patients with advanced solid tumors: H2 2015

**VS-5584**

- Report preliminary Phase 1 results in patients with advanced solid tumors: H2 2015

**Full Year 2014 Financial Results**

As of December 31, 2014, Verastem had cash, cash equivalents and investments of $92.7 million compared to $123.7 million on December 31, 2013. The number of outstanding common shares as of February 27, 2015 was 35,955,110. In January, Verastem completed a public offering of 8,337,500 shares of common stock, raising total gross proceeds of $54.2 million, before deducting the underwriting discounts and commissions. The net proceeds to Verastem from this offering were $50.9 million.

Net loss for the year ended December 31, 2014 ("2014 Period") was $53.4 million, or $2.07 per share, as compared to $41.2 million, or $1.82 per share, for the year ended December 31, 2013 ("2013 Period"). Net loss for 2014 includes non-cash stock-based compensation expense of $12.1 million for the year ended December 31, 2014, as compared to $10.4 million for the year ended December 31, 2013.

Research and development expense for the 2014 Period was $35.4 million compared to $25.9 million for the 2013 Period. The $9.5 million increase from the 2013 Period to the 2014 Period was primarily related to an increase of $7.6 million in contract research organization expense for outsourced biology, chemistry, development and clinical services, which includes our clinical trial costs, a $1.4 million increase in personnel costs and a net increase of approximately $500,000 in other research and development expense.

General and administrative expense for the 2014 Period was $18.2 million compared to $15.5 million for the 2013 Period. The $2.7 million increase from the 2013 Period to the 2014 Period primarily resulted from an increase of $1.6 million in stock-based compensation expense, an increase in consulting fees of $1.0 million and an increase of approximately $912,000 in personnel costs. These increases were partially offset by a net decrease in professional fees and other costs of approximately $800,000.

**Financial Guidance**

Based on current operating plans, we expect to have sufficient cash, cash equivalents and short-term investments to fund our research and development programs and operations into H1 2017.

**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 13 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 VS-6063. For more information visit www.COMMANDmeso.com.

**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, a trial in patients with Kras-mutated non-small cell lung cancer and a trial evaluating the combination of VS-6063 and VS-5584 in patients with relapsed mesothelioma. 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 dose escalation trial of VS-5584 in patients with advanced solid tumors as a single agent and a combination trial of VS-5584 and VS-6063 in patients with relapsed mesothelioma. VS-5584 has been granted orphan drug designation in the U.S. and EU for use in mesothelioma.

**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 and PI3K/mTOR. 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 and activity of the Company’s product candidates, VS-6063, VS-4718 and VS-5584, and the Company’s FAK, PI3K/mTOR and diagnostics programs generally, the timeline for clinical development and regulatory approval of the Company’s product candidates, the expected timing for the reporting of data from ongoing trials and for the COMMAND interim analysis, the expected timing of completion of COMMAND enrollment, the structure of the Company’s planned or pending clinical trials and the ability of the Company to finance contemplated development activities and to fund operations for a specified period. 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 product candidates 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 will take longer than expected, that our product candidates will cause unexpected safety events, that the Company will be unable to successfully initiate or complete the clinical development of its product candidates, that the development of the Company’s product candidates will take longer or cost more than planned, and that the Company’s product candidates 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, 2014 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)**

| | December 31, 2014 | December 31, 2013 |
|---|---|---|
| Cash, cash equivalents and investments | $92,675 | $123,656 |
| Prepaid expenses and other current assets | 2,641 | 643 |
| Property and equipment, net | 2,825 | 631 |
| Other assets | 508 | 331 |
| **Total assets** | **$98,649** | **$125,261** |
| | | |
| Accounts payable and accrued expenses | $8,735 | $7,087 |
| Other liabilities | 1,148 | 728 |
| Stockholders’ equity | 88,766 | 117,446 |
| **Total liabilities and stockholders’ equity** | **$98,649** | **$125,261** |

**Verastem, Inc.**
**Unaudited Condensed Consolidated Statements of Operations**
**(in thousands, except per share amounts)**

| | Twelve months ended December 31, | |
|---|---|---|
| | 2014 | 2013 |
| Operating expenses: | | |

| | | | | |
|---|---|---|---|---|
| Research and development | $35,448 | | $25,930 | |
| General and administrative | 18,159 | | 15,472 | |
| Total operating expenses | 53,607 | | 41,402 | |
| | | | | |
| Loss from operations | (53,607 | ) | (41,402 | ) |
| Interest income | 242 | | 200 | |
| Net loss | ($53,365 | ) | ($41,202 | ) |
| | | | | |
| Net loss per share–basic and diluted | ($2.07 | ) | ($1.82 | ) |
| | | | | |
| Weighted-average number of common shares used in net loss per share -basic and diluted | 25,804 | | 22,680 | |

Source: Verastem, Inc.

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