Verastem Provides Year-End 2014 Update and Outlook for 2015

January 8, 2015

-COMMAND Study Currently On Track for Completion of Enrollment by Year-End 2015-

-Signals of Clinical Activity in all Programs Targeting Cancer Stem Cells Have Been Observed-

-Management to Host Conference Call Today at 4:30 PM ET-

-(877) 341-5660 (U.S. and Canada) or (315) 625-3226 (international). Passcode: 60568979. To access the live slide presentation: http://bit.ly/1vYH8yN-

BOSTON--(BUSINESS WIRE)--Jan. 8, 2015-- Verastem, Inc., (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today provided an overview of the Company's developments in 2014 and outlined upcoming milestones for 2015.

"In 2014, we achieved critical milestones across all of our development programs targeting cancer stem cells," said Robert Forrester, President and Chief Executive Officer of Verastem. "We remain highly focused on the successful execution of COMMAND for the treatment of patients with mesothelioma, an orphan disease, with VS-6063. We currently have 55 sites open in 13 countries and have enrolled 180 patients. We expect to conduct the pre-specified interim analysis in the second quarter of 2015. We currently remain on track to complete enrollment by the end of 2015. We are seeing activity of VS-6063 across multiple tumor types, including mesothelioma, ovarian cancer, and also now in advanced non-small cell lung cancer. These results continue to increase our confidence in the potential of a successful outcome from COMMAND. Our goal is to achieve the first approval of VS-6063 in mesothelioma and then to broaden its use to many major cancers such as lung, ovarian and breast cancer."

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.

"The signals of clinical activity and long-term tolerability that we are seeing across the VS-6063 program are encouraging," said Dr. Joanna Horobin, Verastem Chief Medical Officer. "In addition, both VS-4718 and VS-5584 continue to progress well through Phase 1 clinical trials with favorable tolerability profiles and initial signs of clinical activity beginning to emerge."

2014 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
  - 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 to date. The study is currently accruing on track to complete enrollment by the end of 2015.
  - So far 41% of patients have merlin low tumors, which is consistent with the Company's assumptions
  - Interim analysis to define the primary patient population is expected in Q2 2015
- Encouraging data from our Phase 2 clinical trial evaluating single agent VS-6063 in patients with previously treated KRAS-mutated NSCLC
  - Study is enrolling well at 9 U.S. sites
  - Accrual has completed in 2 cohorts; expect to enroll and dose the remaining cohorts in H1 2015
  - 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
  - Long-term use is generally well tolerated
  - Disease control exceeding 6 months for some patients
  - Next steps to be determined once all cohorts are complete
  - 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

    - 5 patients continue on study with 3 patients continuing past 12 months to date. The longest patient on study, who has had a Complete Response, has been on VS-6063 for more than 18 months
    - 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 prior to surgery for malignant pleural mesothelioma
    - VS-6063 was 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: an additional 10-15 patients are anticipated at this schedule
    - 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 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
- Planned clinical trial to evaluate combination of VS-5584 and VS-6063 in relapsed mesothelioma
    - Expect to begin enrollment in Q1 2015
- Reported supportive preclinical data at AACR, EORTC and published supportive preclinical data in *Molecular Cancer Therapeutics*
- Japanese patent issued with claims covering the composition of matter and VS-5584’s ability to inhibit and regulate cellular metabolism, growth, and proliferation

Leadership team

- Industry veterans, Timothy J. Barberich, 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

- Initiate clinical trial evaluating combination of VS-5584 and VS-6063 in relapsed mesothelioma: Q1 2015
- Report preliminary Phase 1 results in patients with advanced solid tumors expected: H2 2015

Conference Call Information

The call can be accessed by dialing (877) 341-5660 (U.S. and Canada) or (315) 625-3226 (international), and entering passcode 60568979. To access the live slide presentation, please use either the following link: http://bit.ly/1vYH8yN or visit the investors section of the Verastem website at www.verastem.com.

The teleconference replay will be available for one week by dialing (855) 859-2056 (U.S. and Canada) or (404) 537-3406 (international). Replay Passcode 60568979 is required for playback. The webcast will also be recorded and available for replay on the company's website for 90 days.

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, 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 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 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, 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.

Source: Verastem, Inc.

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