



## Verastem Supports Worldwide Rare Disease Day on February 28, 2014

February 28, 2014

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 28, 2014-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today announced its support of the rare disease awareness events being held globally. Verastem's lead compound targeting cancer stem cells, VS-6063, is currently in the registration-directed COMMAND study and has been granted orphan drug designation in the U.S. and E.U. for use in mesothelioma.

Researchers, advocates, patients and companies will come together with a reception and presentation to be held at the Queen Elizabeth Hospital in Birmingham, England. Boston-based pharmaceutical companies will unite with a coalition of local Massachusetts-based patient advocacy groups and state legislators at the Massachusetts State House in recognition of Rare Disease Day. This event is being coordinated by the National Organization of Rare Disorders (NORD), which includes patients and patient groups, biopharmaceutical companies, government agencies, caregivers, and medical researchers. There are many events occurring globally to work towards a united front in addressing the significant need in rare diseases.

Verastem is committed to working with patient advocacy groups that support rare cancers worldwide. On March 5, Professor Dean Fennell, Chair of Thoracic Oncology, University of Leicester, incoming President of the International Mesothelioma Interest Group and Principal Investigator of the COMMAND trial, will deliver the keynote presentation at the International Symposium on Malignant Mesothelioma, hosted by the Meso Foundation, in Washington, D.C.

The COMMAND (Control Of Mesothelioma with MAInenance Defactinib) study is a registration-directed, multinational, placebo-controlled trial of Verastem's lead cancer stem cell inhibitor, VS-6063, in patients with malignant pleural mesothelioma. Mesothelioma is a highly aggressive cancer with limited treatment options. Verastem has been granted orphan drug status for VS-6063 for the treatment of mesothelioma.

In addition to Professor Fennell's address, Verastem representatives will present at the conference in support of the company's mission to foster research, patient advocacy and legislative initiatives for improved treatment in mesothelioma. The Meso Foundation is organizing a legislative walk-up session to the houses of Congress for March 5<sup>th</sup>, 2014 in Washington, D.C.

"The heightened awareness presented by these rare disease events reminds us that, although these illnesses affect smaller patient populations, effective treatments remain an acute global health need," said Robert Forrester, Verastem President and Chief Executive Officer. "We are developing drugs to treat diseases where there is a significant unmet medical need and bringing forth new treatment options for patients. We are committed to fostering the development of potentially life-changing medicines in the pursuit of a more optimistic and positive outcome for patients."

### 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 E.U. 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, 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, the Company's FAK inhibition program and potential indications for clinical development. 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 Company will be unable to successfully complete the clinical development of its compounds, including VS-6063, 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, 2012 and in any subsequent SEC filings. The forward-looking statements contained in this 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.**

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