



## Verastem's Published Scientific Data on Targeting Mesothelioma Cancer Stem Cells Receives Coverage in AACR's Cancer Discovery

June 24, 2014

- The Registration-Directed COMMAND Study of VS-6063 for Patients with Malignant Pleural Mesothelioma to be Highlighted at the Company's Upcoming R&D Day on July 10 -

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 24, 2014-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today announced publication of an article titled "FAK Inhibitor Kills Mesothelioma Cells" in *Cancer Discovery*, a medical journal published by the American Association for Cancer Research (AACR). The commentary references preclinical scientific data on targeting mesothelioma cancer stem cells through inhibition of focal adhesion kinase (FAK) that was recently published in *Science Translational Medicine* (Vol. 6, Issue 237, p.237ra68).

Two of Verastem's lead drug candidates, VS-6063 and VS-4718, target cancer stem cells through potent inhibition of FAK. Cancer stem cells are an underlying cause of tumor resistance to chemotherapy, recurrence and ultimate disease progression. VS-6063 is currently being studied in the registration-directed COMMAND trial in mesothelioma ([www.COMMANDmeso.com](http://www.COMMANDmeso.com)) and VS-4718 is currently in a Phase 1 clinical trial in patients with advanced solid tumors.

The Cancer Discovery commentary can be accessed at <http://bit.ly/1qsDCMo>

The Science Translational Medicine paper on VS-4718 that is the subject of the commentary can be accessed at <http://bit.ly/1imOZp8>

The Company will provide an overview of these programs at its upcoming Research and Development Day being held on Thursday, July 10, 2014 at 12:30pm ET at The Hudson Theatre at the Millennium Broadway Hotel in New York City. The Company will provide updates regarding its research and development programs including presentations by five key opinion leaders. The Company will also discuss recent clinical data, anticipated milestones and upcoming plans for its programs targeting cancer stem cells through inhibition of Focal Adhesion Kinase (FAK) and PI3K/mTOR signaling pathways. Lunch will be served. Please RSVP to [verastem@argotpartners.com](mailto:verastem@argotpartners.com).

Special guest speakers to include:

- **José Baselga, M.D., Ph.D.**, *Physician in Chief, Memorial Sloan Kettering Cancer Center*
- **Raphael Bueno, M.D.**, *Associate Chief, Division of Thoracic Surgery, Brigham & Women's Hospital*
- **Professor Dean Fennell, Ph.D., FCRP**, *Chair of Thoracic Oncology, University of Leicester, Incoming President of the International Mesothelioma Interest Group*
- **Mary Hesdorffer, N.P.**, *Executive Director, Mesothelioma Applied Research Foundation*
- **Manish R. Patel, M.D.**, *Associate Director of Research, Florida Cancer Specialists & Research Institute*

### Annual Research and Development Day Details and Webcast Info

**Title:** Verastem Research and Development Day 2014

**Date and Time:** Thursday, July 10, 2014 at 12:30pm ET

**Location:** The Hudson Theatre at the Millennium Broadway Hotel, 145 West 44th Street, New York, NY

**Webcast:** <http://bit.ly/1vvh3sF>

**Conference Call Dial-in (U.S.):** 866-906-7447

**Conference Call Dial-in (International):** 617-939-0999

**Conference Call Passcode:** 5946305

**RSVP:** [Verastem@argotpartners.com](mailto:Verastem@argotpartners.com)

A replay of the webcast will be archived for 90 days following the presentation date.

**Replay webcast:** <http://bit.ly/1vvh3sF>

**Replay Dial-in (U.S.):** 888-799-6166

**Replay Dial-in (International):** 857-288-2550

**Replay Passcode:** 5946305

### About Malignant Pleural Mesothelioma

Malignant pleural mesothelioma is an aggressive form of cancer that occurs in the mesothelium, the thin layer of tissue that covers the lungs. Mesothelioma is associated with exposure to asbestos in most cases. According to the World Health Organization, there are a total of 59,000 cases of mesothelioma worldwide each year. Most mesotheliomas begin as one or more nodules that progressively grow to form a solid coating of tumor surrounding the lung leading to eventual suffocation and death. A high percentage of mesotheliomas contain cancer stem cells which are generally resistant to the currently available treatment options for mesothelioma.

### 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 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 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 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. VS-6063 targets cancer stem cells. Cancer stem cells 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 12 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 defactinib. For more information visit [www.COMMANDmeso.com](http://www.COMMANDmeso.com)

#### **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 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:**

Any statements in this press release about future expectations, plans and prospects for the Company constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so.

Source: Verastem, Inc.

#### **Verastem, Inc.**

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