



Verastem Files for Orphan Disease Status for VS-6063 in Mesothelioma

February 14, 2013

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 14, 2013-- Verastem, Inc., (NASDAQ: VSTM), a clinical-stage biopharmaceutical company focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, announced the filing of orphan drug designation for VS-6063.

Verastem is seeking orphan drug designation in the US and the EU for the use of VS-6063 in mesothelioma. VS-6063 is anticipated to enter a potentially pivotal study in mesothelioma midyear 2013.

Orphan designation grants US market exclusivity to a drug for the treatment of a specified condition for a period of seven years following FDA marketing approval and 10 years of market exclusivity in member states of the EU if received. Additional potential benefits of orphan designation include development grants, tax credits related to clinical trial expenses, protocol development assistance and exemption or reduction from certain regulatory fees.

Application for orphan drug designation is available for treatment of medical conditions affecting fewer than 200,000 people in the US, or no more than 5 in 10,000 people in the EU, from the respective regulatory agencies in each region.

About VS-6063

VS-6063 is an oral small molecule inhibitor of focal adhesion kinase (FAK), which is a critical pathway for cancer stem cells. VS-6063 was well-tolerated and demonstrated signs of clinical activity in a Phase 1 study in advanced solid tumors. VS-6063 is currently in a Phase 1/2 study in patients with advanced ovarian cancer, where VS-6063 is being tested in combination with paclitaxel. VS-6063 is anticipated to enter a potentially pivotal study in mesothelioma midyear 2013.

About Verastem, Inc.

Verastem, Inc. (NASDAQ: VSTM) is a clinical-stage biopharmaceutical company focused on 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 of the Company's compounds, including VS-6063, and the Company's FAK and diagnostic programs generally, the timeline for clinical development and regulatory approval of the Company's compounds and the structure of the Company's planned clinical trials. 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 may not be predictive of the success of later clinical trials, 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, 2011 and in any subsequent SEC filings. The forward-looking statements contained in this presentation 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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