Verastem Receives Orphan Drug Designation from FDA for VS-5584 in Mesothelioma

February 12, 2015

BOSTON--(BUSINESS WIRE)--Feb. 12, 2015-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells, today announced that VS-5584 has received orphan drug designation from the U.S. Food and Drug Administration for use in the treatment of mesothelioma. The designation was created to encourage the development of drugs that may provide significant benefit to patients suffering from rare diseases.

“This is an important regulatory milestone for Verastem and, together with our European orphan medicinal product designation, will facilitate our global development of VS-5584 to help improve the available treatment options for patients suffering from this highly aggressive cancer,” said Robert Forrester, Verastem President and Chief Executive Officer. "We look forward to taking full advantage of the opportunities that orphan designation allows in order to bring this potential new treatment option to patients as rapidly as possible."

Verastem recently initiated a Phase 1 clinical study evaluating the combination of VS-5584 and VS-6063 in patients with relapsed or progressive malignant pleural mesothelioma. The combination clinical trial is supported by preclinical work demonstrating the synergistic activity of VS-6063 and VS-5584 in mesothelioma models in vitro and in vivo. VS-5584 is also being evaluated in a Phase 1 study in advanced solid tumors where the compound has been generally well tolerated and preliminary activity has been observed, including in mesothelioma. Some patients have been on study for over 6 months and the maximum tolerated dose of VS-5584 has not been reached.

In the U.S., under the Orphan Drug Act, the FDA's Office of Orphan Products Development (OOPD) grants orphan drug status to a drug intended to treat a rare disease or condition, which is generally a disease that affects fewer than 200,000 individuals in the country. Upon approval, if received, the designation provides VS-5584 with certain benefits, including seven years of U.S. market exclusivity in the specified indications if the sponsor complies with certain FDA requirements. Additional incentives for the sponsor include tax credits related to qualified clinical trial expenses and a possible exemption from FDA application fees.

About Mesothelioma

Mesothelioma is an aggressive form of cancer that occurs in the mesothelium, the thin layer of tissue that covers the lungs and other organs. 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. Current treatment in the front line setting consists of 4-6 cycles of Alimta (pemetrexed) in combination with platinum-based therapy. Alimta is the only approved treatment for mesothelioma and there are no approved therapies for relapsed mesothelioma. Relapsed mesothelioma is highly aggressive with a median time to disease progression of only 6 weeks.

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 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 EU for use in 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 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 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 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 including 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 of the Company’s product candidates, including VS-5584 and VS-6063, the timeline for clinical development and regulatory approval of the Company’s product candidates, including the impact of and any potential benefits from FDA’s orphan drug designation, and the structure of the Company’s pending 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 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 of clinical trials may take longer than expected, that the Company will be unable to successfully complete the clinical development of its product candidates, including VS-5584 and VS-6063, 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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