Verastem Oncology Announces New Strategic Direction to Advance Its Clinical Development Programs

February 28, 2020

Transformative Initiative Expected to Accelerate Development of CH5126766 (VS-6766), a RAF/MEK Inhibitor, in Combination with Defactinib, a FAK Inhibitor, for the Treatment of KRAS Mutant Solid Tumors

Company Continues to Advance Development of Duvelisib for the Treatment of Relapsed/Refractory PTCL

Strengthened Balance Sheet Through Private Placement Resulting in Anticipated Gross Proceeds of $100 Million; Projected Cash Runway into 4Q 2021

New Strategic Direction Expected to Result in an Approximately 40% Reduction in Operating Expenses for 2020 Compared to 2019

Management to Host Conference Call Today at 8:00 AM ET

BOSTON--(BUSINESS WIRE)-- Verastem, Inc. (Nasdaq:VSTM) (also known as Verastem Oncology), a biopharmaceutical company committed to developing and commercializing new medicines for patients battling cancer, today announced a new strategic direction to accelerate the advancement of certain of its clinical development programs. The Company's primary focus will be on the development of CH5126766 (VS-6766), its RAF/MEK inhibitor, in combination with defactinib, its focal adhesion kinase (FAK) inhibitor, for the treatment of KRAS mutant solid tumors. Verastem Oncology will also continue to advance the development of duvelisib (COPIKTRA®) for the treatment of relapsed or refractory peripheral T-cell lymphoma (PTCL).

"With our newly expanded development pipeline and strengthened balance sheet, we believe this new strategic direction will be transformative for Verastem Oncology as we will have the opportunity to rapidly advance the development of the clinical programs that we believe will yield the greatest results for patients, physicians and shareholders," said Brian Stuglik, Chief Executive Officer of Verastem Oncology. "We are honored to have leading life science investors participate in our recently announced private placement. Verastem Oncology's mission is centered on improving the lives of cancer patients and we believe our work in collaboration with the scientific community has presented significant opportunity to make further meaningful strides in areas of critical need."

Accelerating Development of CH5126766 (VS-6766) and Defactinib Combination

In early 2020, Verastem Oncology licensed exclusive global development and commercialization rights to CH5126766 (VS-6766), a unique and promising inhibitor of the RAF/MEK signaling pathway. The combination of CH5126766 (VS-6766) and defactinib is currently being investigated in a Phase 1 clinical study and expansion cohorts in patients with KRAS mutant advanced solid tumors, including low grade serous ovarian cancer (LGSOC), non-small cell lung cancer (NSCLC) and colorectal cancer (CRC). Data from this Phase 1 study have been submitted for presentation at the upcoming American Association for Cancer Research (AACR) 2020 Annual Meeting. Verastem Oncology plans to initiate discussions with regulatory authorities during the first half of 2020, with the goal of commencing a registration-directed trial as soon as possible.

Advancing Duvelisib in Relapsed/Refractory PTCL

At the American Society of Hematology (ASH) 2019 Annual Meeting, Verastem Oncology presented positive data from the dose optimization portion of the Phase 2 PRIMO study evaluating duvelisib in patients with relapsed or refractory PTCL, an aggressive disease with a lack of effective therapeutic options. This initial phase of the trial demonstrated promising clinical activity including complete and durable responses, as assessed by independent central review, with a manageable safety profile. The expansion phase of this registration-directed study continues to accrue patients and Verastem Oncology expects to complete enrollment in 2020 and report top-line results from the expansion cohorts in early 2021. Verastem Oncology intends to build on the existing Fast Track and Orphan Drug Designations and submit a regulatory package to the U.S. Food and Drug Administration to expand the approved indications for COPIKTRA to include relapsed or refractory PTCL.

Focusing COPIKTRA Commercial Activities

Verastem Oncology generated preliminary unaudited COPIKTRA net product revenue of $3.6 million for the fourth quarter of 2019, compared to $4.0 million for the third quarter of 2019, and $12.3 million for the full year 2019. Net sales were impacted by timing of purchases and gross to net adjustments associated with Medicare Part D ("donut hole"). Demand units increased 20% from third quarter to fourth quarter 2019. Going forward, Verastem Oncology will be reducing the resources directed to the promotion and sale of COPIKTRA in its current indications, including reducing the size of its salesforce and non-core clinical research. Verastem Oncology plans to shift its COPIKTRA promotional resources toward large, community-based practices and academic institutions, which represent the majority of the appropriate third-line patients with chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) and follicular lymphoma (FL).

Financial Benefits of the Strategic Realignment and 2020 Financial Outlook

As a result of this strategic realignment, Verastem Oncology expects to reduce its operating expenses by approximately 40% for 2020 compared to 2019. Based on its current operating plans, Verastem Oncology expects its research and development and selling, general and administrative expenses for the full year 2020 to be in the range of $70 million to $85 million. As of December 31, 2019, Verastem Oncology had preliminary unaudited cash and short-term investments of $111.3 million. As announced today, Verastem Oncology anticipates completing a private placement of approximately 46.5 million shares of its common stock at an offering price of $2.15 per share on March 3, 2020, resulting in gross proceeds of approximately $100 million to Verastem Oncology before deducting expenses to the placement agents and other estimated offering expenses.

Verastem Oncology expects that its existing cash and cash equivalents, along with the revenue it expects to generate from COPIKTRA, will be sufficient to fund its planned operations into the fourth quarter of 2021.

Conference Call and Webcast Information

The Verastem Oncology management team will host a conference call and webcast today, Friday, February 28, 2020, at 8:00 AM ET. The call can be accessed by dialing (877) 341-5660 (U.S. and Canada) or (315) 625-3226 (international), five minutes prior to the start of the call and providing the passcode 4164157.

The live, listen-only webcast of the conference call can be accessed by visiting the investors section of the Company's website at www.verastem.com. A replay of the webcast will be archived on the Company's website for 90 days following the call.

About CH5126766

CH5126766 (VS-6766) (previously referred to as CKI27 and RO5126766) is a unique inhibitor of the RAF/MEK signaling pathway. In contrast to other MEK inhibitors in development, CH5126766 (VS-6766) blocks both MEK kinase activity and the ability of RAF to phosphorylate MEK. This unique mechanism allows CH5126766 (VS-6766) to block MEK signaling without the compensatory activation of MEK that appears to limit the efficacy of other inhibitors. The combination of CH5126766 (VS-6766) and the focal adhesion kinase (FAK) inhibitor defactinib is currently being investigated in a clinical study (Phase 1 followed by expansion cohorts) with the expansion cohorts now ongoing in patients with KRAS mutant advanced solid tumors, including low grade serous ovarian cancer (LGSOC), non-small cell lung cancer (NSCLC) and colorectal cancer (CRC). The ongoing clinical study of the CH5126766 (VS-6766)/defactinib combination is supported by single-agent Phase 2 studies which investigated defactinib in KRAS mutant NSCLC and CH5126766 (VS-6766) in KRAS mutant NSCLC and LGSOC.

About Defactinib

Defactinib is an oral small molecule inhibitor of FAK and PYK2 that is currently being evaluated as a potential combination therapy for various solid tumors. The Company has received Orphan Drug designation for defactinib in ovarian cancer and mesothelioma in the US, EU and Australia. Preclinical research by Verastem Oncology scientists and collaborators at world-renowned research institutions has described the effect of FAK inhibition to enhance immune response by decreasing immuno-suppressive cells, increasing cytotoxic T cells, and reducing stromal density, which allows tumor-killing immune cells to enter the tumor.[1,2] A Phase 1/2 clinical trial of defactinib in combination with CH5126766 (VS-6766) in patients with KRAS mutant advanced solid tumors, including low grade serous ovarian cancer (LGSOC), non-small cell lung cancer (NSCLC) and colorectal cancer (CRC) is underway.[3] The CH5126766 (VS-6766)/defactinib combination is supported by single-agent Phase 2 studies which investigated defactinib in KRAS mutant NSCLC[4] and CH5126766 (VS-6766) in KRAS mutant NSCLC and LGSOC.[5] Defactinib is also in clinical testing in combination with pembrolizumab for treatment of patients with pancreatic cancer, NSCLC and mesothelioma.[6]

About COPIKTRA® (duvelisib)

COPIKTRA is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, two enzymes known to help support the growth and survival of malignant B-cells. PI3K signaling may lead to the proliferation of malignant B-cells and is thought to play a role in the formation and maintenance of the supportive tumor microenvironment.[7,8,9]

COPIKTRA is indicated for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. COPIKTRA is also being developed by Verastem Oncology for the treatment of peripheral T-cell lymphoma (PTCL), for which it has received Fast Track status and Orphan Drug Designation, and is being investigated in combination with other agents through investigator-sponsored studies.[10] For more information on COPIKTRA, please visit www.COPIKTRA.com. Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

About Verastem Oncology

Verastem Oncology (Nasdaq: VSTM) is a commercial biopharmaceutical company committed to the development and commercialization of new medicines to improve the lives of patients diagnosed with cancer. Our pipeline is focused on novel small molecule drugs that inhibit critical signaling pathways in cancer that promote cancer cell survival and tumor growth, including phosphoinositide 3-kinase (PI3K), focal adhesion kinase (FAK) and RAF/MEK inhibition.

Our first FDA approved product is available for the treatment of patients with certain types of indolent non-Hodgkin’s lymphoma (iNHL).

For more information, please visit www.verastem.com.

Forward-Looking Statements Notice

This press release includes forward-looking statements about Verastem Oncology’s strategy, future plans and prospects, including statements related to the opportunity to rapidly advance the development of clinical programs through Verastem Oncology’s expanded development pipeline and strengthened balance sheet, the timing of top-line results for clinical trials, anticipated reductions in operating expenses from Verastem Oncology’s strategic realignment, the timing of commencing a registration-directed trial for CH5126766 (VS-6766) and financial guidance estimates. The words "anticipate," "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.

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 and uncertainties, among other things, regarding: the success in the development and potential commercialization of our product candidates, including defactinib in combination with CH5126766 (VS-6766); the occurrence of adverse safety events and/or unexpected concerns that may arise from additional data or analysis or result in unmanageable safety profiles as compared to their levels of efficacy; our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; the scope, timing, and outcome of any legal proceedings; decisions by regulatory authorities regarding labeling and other matters

that could affect the availability or commercial potential of our product candidates; whether preclinical testing of our product candidates and preliminary or interim data from clinical trials will be predictive of the results or success of ongoing or later clinical trials; that the timing, scope and rate of reimbursement for our product candidates is uncertain; that third-party payors (including government agencies) may not reimburse; that there may be competitive developments affecting our product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected; that our product candidates will experience manufacturing or supply interruptions or failures; that we will be unable to successfully initiate or complete the clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; that we or Chugai Pharmaceutical Co., Ltd. will fail to fully perform under the CH5126766 (VS-6766) license agreement; that we may not have sufficient cash to fund our contemplated operations; that we may be unable to make additional draws under our debt facility or obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; that we will be unable to execute on our partnering strategies for defactinib in combination with CH5126766 (VS-6766); that we will not pursue or submit regulatory filings for our product candidates, and that our product candidates will not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients.

Other risks and uncertainties include those identified under the heading “Risk Factors” in the Company’s Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2019, as filed with the Securities and Exchange Commission (SEC) on October 30, 2019, its Annual Report on Form 10-K for the year ended December 31, 2018 as filed with the SEC on March 12, 2019 and in any subsequent filings with the SEC. The forward-looking statements contained in this press release reflect Verastem Oncology’s views as of the date hereof, and the Company does not assume and specifically disclaims any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by law.

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