Verastem Oncology Announces Presentation of Clinical Data from Investigator-initiated RAF/MEK and FAK Combination Study in KRAS Mutant Solid Tumors at the American Association for Cancer Research 2020 Virtual Annual Meeting I

April 13, 2020

Preliminary Results from Dose Escalation Portion and Expansion Cohorts Investigating VS-6766 in Combination with Defactinib in Patients with KRAS Mutant Advanced Solid Tumors to be Highlighted in a Virtual Poster Presentation

Management to Host Investor Conference Call Following the Virtual Poster Presentation to Discuss the Data

BOSTON--(BUSINESS WIRE)--Apr. 13, 2020-- 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 that an abstract highlighting preliminary results from the ongoing investigator-initiated clinical study investigating VS-6766, its RAF/MEK inhibitor, in combination with defactinib, its FAK inhibitor, in patients with KRAS mutant advanced solid tumors has been selected for a virtual poster presentation at the upcoming American Association for Cancer Research (AACR) 2020 Virtual Annual Meeting I, taking place April 27-28, 2020.

This ongoing study is an open label, dose escalation and expansion study. The expansion cohorts are currently 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). Following the virtual data presentation, Verastem Oncology will host an investor conference call to discuss the presented data. The exact date and time of the investor conference call will be announced soon.

“These early clinical results led to our decision to in-license VS-6766 earlier this year and accelerate development of this exciting combination program for patients with KRAS mutant cancers,” said Brian Stuglik, Chief Executive Officer of Verastem Oncology. “The synergy between VS-6766 and defactinib has been encouraging and we look forward to sharing the data with the medical and scientific communities later this month.”

Verastem Oncology plans to initiate discussions with regulatory authorities during the first half of 2020, with the goal of commencing a registration-directed trial investigating the VS-6766/defactinib combination as soon as possible.

Other Abstracts Selected for Presentation at AACR 2020 Virtual Meeting I

Two additional abstracts also selected for presentation at the April virtual meeting include: updated data from an ongoing Investigator-initiated Phase 1 study investigating defactinib in combination with pembrolizumab and gemcitabine in patients with advanced pancreatic ductal adenocarcinoma (PDAC) (Wang-Gilliam, *et al*); and preclinical research describing the synergistic antitumor efficacy of duvelisib, the Company’s oral inhibitor of phosphoinositide 3-kinase (PI3K)-delta and PI3K-gamma, in combination with PD-1 blockade in solid tumor and lymphoma models.

Details for the AACR 2020 Virtual Meeting I presentations are as follows:

Title: Phase 1 study of the combination of a RAF-MEK inhibitor CH5126766 and FAK inhibitor defactinib in an intermittent dosing schedule with expansions in KRAS mutant cancers
Lead author: Udai Banerji, Institute of Cancer Research and The Royal Marsden
Poster #: CT143
Session: VPO.CT01 - Phase I Clinical Trials
Date and Time: Monday, April 27, 2020; 9:00 a.m. to 6:00 p.m. ET
URL: https://www.abstractsonline.com/pp8/#!/9045/presentation/10642

Title: Phase 1 study of defactinib combined with pembrolizumab and gemcitabine in patients with advanced cancer: Experiences of pancreatic ductal adenocarcinoma (PDAC) patients
Lead author: Andrea Wang-Gilliam, Washington University in St. Louis
Poster #: CT118
Session: VPO.CT01 - Phase I Clinical Trials
Date and Time: Monday, April 27, 2020; 9:00 a.m. to 6:00 p.m. ET
URL: https://www.abstractsonline.com/pp8/#!/9045/presentation/10617

Title: Synergistic antitumor efficacy of the dual PI3K-δ/PI3K-γ inhibitor duvelisib with PD-1 blockade in solid tumor and lymphoma models
Lead author: Jonathan Pachter, Verastem Oncology
Abstract #: CT045
Session: VCTPL04 - Immunotherapy Clinical Trials 2
Date and Time: Tuesday, April 28, 2020; 3:15 – 3:25 p.m. ET
URL: https://www.abstractsonline.com/pp8/#!/9045/presentation/10754

About VS-6766

VS-6766 (formerly known as CH5126766, CKI27 and RO5126766) is a unique inhibitor of the RAF/MEK signaling pathway. In contrast to other MEK inhibitors in development, VS-6766 blocks both MEK kinase activity and the ability of RAF to phosphorylate MEK. This unique mechanism allows VS-6766 to block MEK signaling without the compensatory activation of MEK that appears to limit the efficacy of other inhibitors. The combination of VS-6766 and the focal adhesion kinase (FAK) inhibitor defactinib is currently being investigated in a Phase 1 dose escalation and expansion study.

The expansion cohorts are currently 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).[3] The ongoing clinical study of the VS-6766/defactinib combination is supported by single-agent Phase 2 studies which investigated defactinib in KRAS mutant NSCLC[4] and VS-6766 in KRAS mutant NSCLC and LGSOC.[5]

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] Additionally, in both preclinical and clinical studies, FAK activation has been shown to occur as a potential resistance mechanism in response to MEK inhibitor treatment, and synergy of a FAK inhibitor with a RAF/MEK inhibitor has been shown in several preclinical models. The combination of defactinib and VS-6766 is currently being investigated in a Phase 1 dose escalation and expansion study. The expansion cohorts are currently 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).[3] The ongoing clinical study of the VS-6766/defactinib combination is supported by single-agent Phase 2 studies which investigated defactinib in KRAS mutant NSCLC[4] and 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.

References

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