Verastem Oncology Announces Global Licensing Agreement with Chugai Pharmaceutical Co., Ltd. to Develop and Commercialize RAF/MEK Inhibitor CH5126766

January 8, 2020

Combination of Defactinib and CH5126766 Shows Promise in Treating KRAS Mutant Solid Tumors in Clinical Trial

Clinical Data Presentation and Regulatory Discussions Planned for 1H 2020

BOSTON--(BUSINESS WIRE)--Jan. 8, 2020--

Verastem, Inc. (Nasdaq:VSTM) (Verastem Oncology or the Company), a biopharmaceutical company focused on developing and commercializing medicines seeking to improve the survival and quality of life of cancer patients, today announced a global licensing agreement with Chugai Pharmaceutical Co., Ltd., (Chugai) whereby Verastem Oncology is obtaining worldwide development and commercialization rights to the RAF/MEK inhibitor CH5126766 (CKI27) from Chugai currently under development for the treatment of KRAS mutant solid tumors. The Company will host an investor call to discuss the opportunity and a development update today (details below).

CH5126766 in combination with Verastem Oncology's focal adhesion kinase (FAK) inhibitor, defactinib, is currently the subject of a clinical study (Phase I 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).[1] This clinical study of the defactinib/CH5126766 combination is supported by the single-agent Phase 2 studies of defactinib in KRAS mutant NSCLC[2] and CH5126766 in KRAS mutant NSCLC and LGSOC.[3]

"Based on the single-agent defactinib results in KRAS mutant NSCLC, we conducted an internal pre-clinical effort to identify drug classes that were synergistic with defactinib and saw the highest level of synergy in combination with MEK inhibitors and, specifically, with CH5126766," said Dan Paterson, President and Chief Operating Officer of Verastem Oncology. "The exciting early clinical results led to our decision to enter into a partnership with Chugai for CH5126766 and accelerate the combination development program for patients with KRAS mutant cancers, which are highly aggressive and recurrent. We plan to initiate discussions with regulatory authorities about our development plans and to define the registration path early this year."

"We found that MEK blockade activates FAK signaling as a potential escape mechanism," stated Professor Udai Banerji, Professor of Molecular Cancer Pharmacology at The Institute of Cancer Research and Honorary Consultant in Medical Oncology, MBBS, MD, DNB, PhD, FRCP at The Royal Marsden NHS Foundation Trust, London, England, and lead investigator of the clinical study. "Based on the synergy between FAK and MEK inhibitors observed in preclinical KRAS mutant models, we have been assessing the combination of defactinib and CH5126766 for treatment of patients with KRAS mutant cancers. The results to date have been encouraging and we look forward to sharing our clinical findings, including the response rate in an upcoming scientific presentation."

"CH5126766 is a unique and particularly promising inhibitor of the RAS/RAF/MEK signaling pathway," noted Neal Rosen, MD, PhD, Memorial Sloan Kettering Cancer Center, NY, NY. "In contrast to other MEK inhibitors in development, CH5126766 blocks both MEK kinase activity and the ability of RAF to phosphorylate MEK. This unique mechanism allows CH5126766 to block MEK signaling without the compensatory activation of MEK that appears to limit the efficacy of other inhibitors. The clinical data with the combination of defactinib and CH5126766 are striking and suggest promise for patients with KRAS mutant solid tumors."

Under the terms of the agreement, Verastem Oncology is responsible for the development and worldwide commercialization of CH5126766. The Company will make an upfront payment of $3M and pay royalties to Chugai. Given the potential of the opportunity, the Company will be evaluating various partnering strategies.

Conference Call and Webcast Information

The Verastem Oncology management team will host a conference call and webcast on Wednesday, January 8, 2020, at 4:00 PM (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 3756707 and web PIN 1655.

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

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.[4,5] A Phase 1/2 clinical trial of defactinib in combination with CH5126766 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.[1] The defactinib/CH5126766 combination is supported by the single-agent Phase 2 studies of defactinib in KRAS mutant NSCLC[2] and CH5126766 in KRAS mutant NSCLC and LGSOC.[3] Defactinib is also in clinical testing in combination with pembrolizumab for treatment of patients

with pancreatic cancer, NSCLC and mesothelioma.[6]

About Verastem Oncology

Verastem Oncology (Nasdaq: VSTM) is a commercial biopharmaceutical company committed to the development and commercialization of medicines to improve the lives of patients diagnosed with cancer. We are driven by the strength, tenacity and courage of those battling cancer – single-minded in our resolve to deliver new therapies that not only keep cancer at bay, but improve the lives of patients diagnosed with cancer. Because for us, it's personal.

Our first FDA approved product is now available for the treatment of patients with certain types of indolent non-Hodgkin's lymphoma (iNHL). Our pipeline comprises product candidates that seek to treat cancer by modulating the local tumor microenvironment. 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 regarding the development and activity of Verastem Oncology's FAK inhibitor defactinib in combination with CH5126766 , and the potential commercial success of the combination therapy in patients with KRAS mutant cancers. 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. 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; 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 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; 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

[1] https://clinicaltrials.gov/, NCT03875820

[2] Gerber D. et al. Phase 2 study of the focal adhesion kinase inhibitor defactinib (VS-6063) in previously treated advanced KRAS mutant non-small cell lung cancer. Lung Cancer 2020: 139:60-67.

[3] Chénard-Poirier, M. et al. Results from the biomarker-driven basket trial of RO5126766 (CH5127566), a potent RAF/MEK inhibitor, in RAS- or RAF-mutated malignancies including multiple myeloma. Journal of Clinical Oncology 2017: 35. 10.1200/JCO.2017.35.15_suppl.2506.

[4] Jiang H et al. Targeting focal adhesion kinase renders pancreatic cancers responsive to checkpoint immunotherapy. Nat Med 2016: Aug 22(8) 851-60.

[5] Sulzmaier F.J. et al. FAK in cancer: mechanistic findings and clinical applications. Nature Rev Cancer. 2014 14: 598-610.

[6] www.clinicaltrials.gov, NCT02758587

View source version on businesswire.com: https://www.businesswire.com/news/home/20200108005086/en/

Source: Verastem, Inc.

Investors:
John Doyle
Vice President, Investor Relations & Finance
+1 781-469-1546
jdoyle@verastem.com

Media:
Lisa Buffington
Corporate Communications
+1 781-292-4205
lbuffington@verastem.com