



Verastem Oncology Announces Closing of COPIKTRA® (duvelisib) Sale to Secura Bio

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Verastem Now Focused on Development of VS-6766 and Defactinib Combination in Low-Grade Serous Ovarian Cancer and KRAS Mutant Non-Small Cell Lung Cancer

Registration-Directed Clinical Trials in LGSOC and KRAS Mutant NSCLC Expected to Commence by Year End 2020

BOSTON--(BUSINESS WIRE)--Sep. 30, 2020-- Verastem, Inc. (Nasdaq:VSTM) (also known as Verastem Oncology), a biopharmaceutical company committed to advancing new medicines for patients battling cancer, today announced that it has completed the sale of Verastem's COPIKTRA (duvelisib), a marketed oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first FDA-approved dual inhibitor of PI3K-delta and PI3K-gamma, to Secura Bio, Inc.

In consideration for the COPIKTRA assets, Verastem received from Secura Bio \$70 million in cash. Additionally, Verastem is eligible to receive up to a total deal value of \$311 million if certain regulatory and sales-based milestones are successfully met by Secura Bio and COPIKTRA's other rest-of-world partners. Verastem will also receive low double-digit royalties on net sales over \$100 million in the U.S., Europe and the United Kingdom. Secura Bio has assumed all operational and financial responsibility for COPIKTRA program activities, including commercialization efforts in the United States and Europe, ongoing clinical trials, development and commercialization partnerships with Yakult, CSPC and Sanofi and existing royalty obligations.

Verastem's sale of COPIKTRA to Secura Bio follows Verastem's previously announced new strategic direction focused on development of its RAF/MEK inhibitor (VS-6766) and FAK inhibitor (defactinib) program in KRAS mutant (KRASmt) solid tumors. Verastem's first potential indications for the VS-6766 and defactinib combination will be in low-grade serous ovarian cancer (LGSOC) and KRASmt non-small cell lung cancer (NSCLC).

"This transaction delivers clear benefits for both Verastem Oncology and Secura Bio. At Verastem, we will now focus all of our efforts and resources on development of the VS-6766 and defactinib combination in LGSOC and KRASmt NSCLC," said Brian Stuglik, Chief Executive Officer of Verastem Oncology. "The ongoing Phase 1/2 FRAME study continues to provide encouraging data and we plan to commence registration-directed clinical trials in LGSOC and KRASmt NSCLC by the end of 2020."

MTS Health Partners, L.P and Ropes & Gray LLP acted as advisors to Verastem Oncology on this transaction.

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.

About Defactinib

Defactinib (VS-6063) 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}

About the VS-6766/Defactinib Combination

RAS mutant tumors are present in ~30% of all human cancers, have historically presented a difficult treatment challenge and are often associated with significantly worse prognosis. Challenges associated with identifying new treatment options for these types of cancers include resistance to single agents, identifying tolerable combination regimens with MEK inhibitors and new RAS inhibitors in development addressing only a minority of all RAS mutated cancers.

The combination of VS-6766 and defactinib has been found to be clinically active in patients with KRAS mt tumors. In an ongoing investigator-initiated Phase 1/2 FRAME study, the combination of VS-6766 and defactinib is being evaluated in patients with LGSOC, KRASmt NSCLC and colorectal cancer (CRC). Updated interim data from this study presented at the 2nd Annual RAS-Targeted Drug Development Summit in September 2020 demonstrated a 56% overall response rate and long duration of therapy among patients with KRAS-G12 mt LGSOC. Based on an observation of higher response rates seen in NSCLC patients with KRAS-G12V mutations in the study, Verastem will also be further exploring the role of VS-6766 and defactinib in KRAS-G12V NSCLC. The FRAME study was expanded in August 2020 to include new cohorts in pancreatic cancer, KRASmt endometrial cancer and KRAS-G12V NSCLC.

About Verastem Oncology

Verastem Oncology (Nasdaq: VSTM) is a development-stage 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 RAF/MEK inhibition and focal adhesion kinase (FAK) inhibition. 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 potential clinical value of the RAF/MEK/FAK combination and the timing of commencing registration-directed trials for the RAF/MEK/FAK combination. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," "can," "promising" 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 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 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 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 Annual Report on Form 10-K for the year ended December 31, 2019 as filed with the Securities and Exchange Commission (SEC) on March 11, 2020 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 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.

² <https://clinicaltrials.gov>, NCT03875820

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