Verastem Oncology Signs Definitive Agreement to Sell COPIKTRA® (duvelisib) Rights to Secura Bio to Focus on Development of VS-6766 and Defactinib in KRAS Mutant Solid Tumors

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Verastem Will Receive $70 Million Up-Front with Total Deal Value Up to $311 Million, Plus Double-Digit Sales Royalties

Upon Closing, Verastem's Current Programs Will Be Funded Until At Least 2024 to Develop VS-6766 and Defactinib in Low-Grade Serous Ovarian Cancer and KRAS Mutant Non-Small Cell Lung Cancer

Phase 2 Registration-Directed Trials Expected to Commence by Year End 2020 in Both Low-Grade Serous Ovarian Cancer and KRAS Mutant Non-Small Cell Lung Cancer

Enrollment in Ongoing Investigator-Initiated Phase 1/2 FRAME Study of VS-6766 and Defactinib Now Expanding to Include Pancreatic, KRAS Mutant Endometrial and KRAS-G12V Non-Small Cell Lung Cancer Cohorts

BOSTON--(BUSINESS WIRE)--Aug. 10, 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 entered into a definitive agreement to sell its global commercial and development rights to COPIKTRA (duvelisib), its 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., an integrated biopharmaceutical company dedicated to the worldwide commercialization of significant oncology therapies.

Verastem's sale of COPIKTRA follows the Company's previously announced strategic direction to focus on maximizing the broad potential of its RAF/MEK inhibitor (VS-6766) and FAK inhibitor (defactinib) program in KRAS mutant (KRASmt) solid tumors. Upon closing of the transaction with Secura Bio, Verastem will be dedicated to the development of this program and to deliver on clinical and regulatory milestones for the first potential indications in low-grade serous ovarian cancer (LGSOC) and KRASmt non-small cell lung cancer (NSCLC). Both LGSOC and KRASmt NSCLC are areas of high unmet patient need as there are no approved treatments and existing therapies have low response rates.

"By focusing our expertise and efforts on rapidly advancing the RAF/MEK/FAK development program, we believe we will be providing the best path forward for patients, customers, our shareholders and our company. These strategic decisions will enable us to best deliver on our mission to advance new medicines on behalf of cancer patients," said Brian Stuglik, Chief Executive Officer of Verastem Oncology. "The agreement with Secura Bio will ensure COPIKTRA continues to help more patients, leveraging the established commercial structure, support of ongoing clinical study and potential expansion into new indications."

Terms of the Definitive Sale Agreement

Verastem will receive an up-front payment of $70 million upon the closing of the transaction and 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, including:

- A total of $45 million from two separate milestone payments for U.S. Food and Drug Administration (FDA) and European Medicines Agency approvals of COPIKTRA with label indicated for peripheral T-cell lymphoma
- A total of $50 million for cumulative worldwide net sales of COPIKTRA beginning at $100 million of cumulative net sales
- Verastem will receive low double-digit royalties on net sales over $100 million in U.S., Europe and the United Kingdom
- Verastem will also receive 50% of licensing milestones (up to $146 million) and royalties outside of U.S., Europe and the United Kingdom

In exchange, Secura Bio will receive an exclusive worldwide license for the research, development, commercialization and manufacture of COPIKTRA in all oncology indications. Secura Bio will assume all operational and financial responsibility for activities that were previously part of Verastem's duvelisib program, including commercialization efforts in the United States and Europe, ongoing clinical trials, Verastem's partnerships with Yakult, CSPC and Sanofi and existing royalty obligations. Secura Bio and Verastem are also in discussions related to the transfer of Verastem's field sales and medical professionals.

The transaction with Secura Bio is subject to customary closing conditions and is expected to close in the third quarter of 2020.*

VS-6766 and Defactinib Program Progress and Registration-Directed Trials

Verastem announced today that the company met with the FDA in July 2020 to discuss the registration-directed study design for the VS-6766/defactinib combination in patients with LGSOC. The FDA was supportive of the Company's development strategy and adaptive design for LGSOC.

Verastem's NSCLC study will also be an adaptive design with a focus on patients with KRAS-G12V mutant tumors. Verastem intends to seek input from the FDA after completing the initial cohort of the lung cancer study. Verastem expects to commence registration-directed clinical trials for potential accelerated approval in LGSOC and KRASmt NSCLC by the end of 2020.

Verastem is continuing its clinical collaboration with the Drug Development Unit at ICR/Royal Marsden Hospital. The ongoing investigator-initiated Phase 1/2 FRAME study evaluating the combination of VS-6766 with defactinib in LGSOC, KRASmt NSCLC and colorectal cancer (CRC) has resumed normal accrual and reporting rates following the global lockdown resulting from the COVID-19 pandemic. The FRAME study is now expanding to include new cohorts in pancreatic cancer, KRASmt endometrial cancer and KRAS-G12V NSCLC. Verastem expects that additional data from the LGSOC cohort of the FRAME study will be made available in September, including presentation at the 2nd Annual RAS-Targeted Drug Development Conference. The Company also expects that additional data from the NSCLC cohort of the FRAME study will be submitted to the International Association for the Study of Lung Cancer (IASLC) World Lung Cancer Conference, taking place in January 2021.

The Company has also begun preclinical combination studies investigating VS-6766 and defactinib in combination with KRAS-G12C inhibitors and initial data will be presented at the 2nd Annual RAS-Targeted Drug Development Conference. Based on the positive preclinical data presented at the AACR 2020 Virtual Annual Meeting II, Verastem plans to support a Phase 2 investigator-initiated study evaluating the combination of VS-6766 and defactinib in uveal melanoma, which is expected to begin in late 2020.

Corporate and Financial Overview

With the sale of COPIKTRA, Verastem will become a focused development company with reduced annual expenses of approximately $50 million. The company is in a position of financial strength with a cash runway expected to fund the clinical and regulatory milestones and development of VS-6766 and defactinib in LGSOC and KRASmt NSCLC until at least 2024.

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 and 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 KRASmt. In an ongoing investigator-initiated Phase I/2 FRAME study, the combination of VS-6766 and defactinib is being evaluated in patients with LGSOC, KRASmt NSCLC and colorectal cancer (CRC). Preliminary data from this study presented at the American Association for Cancer Research (AACR) 2020 Virtual Annual Meeting I demonstrated a 67% overall response rate and long duration of therapy among patients with KRASmt LGSOC. Based on an observation of higher response rates seen in 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 is expanding in August 2020 to include new cohorts in pancreatic, KRASmt endometrial and KRAS-G12V NSCLC.

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.[3,4,5] 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.[6] For more information on COPIKTRA, please visit www.COPIKTRA.com. Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

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

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 expected sale of COPIKTRA, the Company's future funding requirements and the potential clinical value of 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 satisfaction of closing conditions with respect to the sale of the COPIKTRA assets to Secura Bio; the ability of Secura Bio to achieve the clinical and sales milestones necessary to result in additional consideration payable to Verastem; the inherent uncertainty in forecasting expected funding needs of the Company in advancing its product candidates; 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 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.

[1] 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.

[2] 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.

[3] Winkler D.G., Faia K.L., DiNitto J.P. et al. PI3K-delta and PI3K-gamma inhibition by IPI-145 abrogates immune responses and suppresses activity in autoimmune and inflammatory disease models. Chem Biol 2013; 20:1-11.

[4] Reif K et al. Cutting Edge: Differential Roles for Phosphoinositide 3 kinases, p110-gamma and p110-delta, in lymphocyte chemotaxis and homing. J Immunol 2004:173:2236-2240.

[5] Schmid M et al. Receptor Tyrosine Kinases and TLR/IL1Rs Unexpectedly activate myeloid cell PI3K, a single convergent point promoting tumor inflammation and progression. Cancer Cell 2011;19:715-727.

[6] www.clinicaltrials.gov, NCT03372057.

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