



Verastem Oncology Provides Update on RAMP 201 Study Evaluating VS-6766 ± Defactinib in Low-Grade Serous Ovarian Cancer

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Interim Analysis Findings Support Continued Evaluation of Both Monotherapy and Combination Therapy

Encouraging Efficacy Results Include Independently Confirmed Responses in Both KRAS Mutant and KRAS Wild-Type Tumors with No New Safety Signals Observed

Substantial Majority (~80%) of Patients Remain on Therapy; Timing of Go Forward Treatment Regimen Selection Driven by Data Maturity

BOSTON--(BUSINESS WIRE)--Jun. 6, 2022-- Verastem Oncology (Nasdaq: VSTM), a biopharmaceutical company committed to advancing new medicines for people living with cancer, today announced an update from an interim analysis of its international Phase 2 RAMP 201 trial evaluating VS-6766 ± defactinib in recurrent low-grade serous ovarian cancer (LGSOC), regardless of KRAS status.

Verastem recently completed a planned interim analysis of its RAMP 201 trial with the goal of selecting a go forward treatment regimen of either VS-6766 monotherapy or VS-6766 in combination with defactinib. The analysis indicated encouraging efficacy results with confirmed responses by independent review in patients treated with VS-6766 monotherapy and patients treated with VS-6766 in combination with defactinib. The findings also include confirmed responses by independent review in both KRAS mutant and KRAS wild-type LGSOC. To date, there have been no additional safety signals with a continued favorable safety profile in both the monotherapy and combination treatment arms with approximately 6% of patients discontinuing due to adverse events.

With a substantial majority (approximately 80%) of patients remaining on study treatment with a median duration of follow-up of four months, the Company has concluded that the data from the interim analysis are not mature enough to make a final decision on the go forward treatment regimen at this time and the trial will continue with all four cohorts (VS-6766 ± defactinib in KRAS mutant and KRAS wild type patient populations).

"We are encouraged by the positive anti-tumor activity that we have seen to date in the RAMP 201 trial in patients with both KRAS mutant and KRAS wild-type tumors. We look forward to evaluating a more mature data set and expect to provide an update on progress once the go forward treatment regimen has been determined," said Brian Stuglik, Chief Executive Officer, Verastem Oncology. "This interim analysis adds to our optimism about the potential for VS-6766 with or without defactinib and our commitment to advancing the first new treatment specifically developed and approved for women with low-grade serous ovarian cancer where a high medical need remains."

The Company plans to complete enrollment of all four cohorts of the trial in the second half of this year. Each cohort is expected to have approximately 36 patients for a total of 144 patients.

Both VS-6766 and defactinib are in late-stage development and the combination has received Breakthrough Therapy Designation by the U.S. Food and Drug Administration for the treatment of all patients with recurrent low-grade serous ovarian cancer regardless of KRAS status after one or more prior lines of therapy, including platinum-based chemotherapy.

About the VS-6766/Defactinib Combination

VS-6766 is a RAF/MEK clamp that induces inactive complexes of MEK with ARAF, BRAF and CRAF potentially creating a more complete and durable anti-tumor response through maximal RAS pathway inhibition. In contrast to currently available MEK inhibitors, 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 FAK inhibitor, defactinib provides RAF/MEK vertical blockade and FAK parallel inhibition to overcome key resistance mechanisms. Both VS-6766 and defactinib are in late-stage development.

Verastem Oncology is conducting Phase 2 registration-directed trials of VS-6766 alone and with defactinib in patients with recurrent LGSOC and in patients with recurrent KRAS G12V-mutant NSCLC as part of its RAMP (**Raf And Mek Program**) clinical trials, RAMP 201 and RAMP 202, respectively (www.ramp201study.com and www.ramp202study.com). Verastem Oncology has also established clinical collaborations with Amgen, Inc. and Mirati Therapeutics, Inc. to evaluate LUMAKRAS™ (sotorasib) and adagrasib in combination with VS-6766 in KRAS G12C-mutant NSCLC as part of the RAMP 203 and RAMP 204 trials, respectively.

About Low-Grade Serous Ovarian Cancer

Low-grade serous ovarian cancer is a highly recurrent, chemotherapy-resistant cancer, associated with slow tumor growth and high mortality rate.¹ Approximately 6,000 women in the U.S. and 80,000 worldwide are living with this disease. Mutations in the KRAS gene are present in 35-57% cases of LGSOC.² LGSOC is most often diagnosed in women between the ages of 45-55 years and has a median survival of approximately ten years.² The majority of patients experience severe pain and complications as the disease progresses. Chemotherapy is the standard of care for this disease, with limited treatment options currently available.²

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 various of its clinical trials, the timing of commencing and completing trials, including topline data reports, and potential for additional development programs involving Verastem Oncology's lead compounds VS-6766 and defactinib. 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 VS-6766 in combination with other compounds, including defactinib, LUMAKRAS™ and others; 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 or our other collaboration partners may fail to perform under our collaboration agreements; that we may not have sufficient cash to fund our contemplated operations; that we may be unable to obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; that Secura Bio, Inc. will achieve the milestones that result in payments to us under our asset purchase agreement with Secura Bio, Inc.; that we will be unable to execute on our partnering strategies for VS-6766 in combination with other compounds; 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, 2021 as filed with the Securities and Exchange Commission (SEC) on March 28, 2022 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 Grisham R. Low grade serous carcinoma of the ovary. *Oncology*. 2016; 30(7):650-652. Available at: <https://www.cancernetwork.com/view/low-grade-serous-carcinoma-ovary>. Accessed February 8, 2022

² Slomovitz B, Gourley C, Carey S. M, Malpica A, Shih I, Huntsman D, et al. Low-Grade serous ovarian cancer: State of the Science. *Gynecol Oncol*. 2020;156(3):715-725. <https://doi.org/10.1016/j.ygyno.2019.12.033>.

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