



Verastem Oncology Reports First Quarter 2021 Financial Results and Highlights Recent Company Progress

May 11, 2021 at 4:05 PM EDT

Updated Phase 1/2 Data in Recurrent Low-Grade Serous Ovarian Cancer (LGSOC) Continues to Demonstrate Activity, Durability and a Favorable Tolerability Profile for VS-6766 + Defactinib Combination

Two Registration-Directed Phase 2 Trials Underway in LGSOC and KRAS G12V Non-Small Cell Lung Cancer with Multiple Key Catalysts Expected in 1H 2022

BOSTON--(BUSINESS WIRE)--May 11, 2021-- Verastem, Inc. (Nasdaq: VSTM) (also known as Verastem Oncology), a biopharmaceutical company committed to advancing new medicines for patients battling cancer, today reported financial results for the three months ended March 31, 2021, highlighted recent progress and outlined key corporate objectives.

"During the first quarter of 2021, we expanded the selection portion of the Phase 2 RAMP 201 study to include all recurrent low-grade serous ovarian cancer types. This decision was based on the positive updated data from the LGSOC cohort of the Phase 1/2 FRAME which continues to show strong response rates across both KRAS mutant and wild-type recurrent LGSOC, along with robust durability and a favorable tolerability profile," said Brian Stuglik, Chief Executive Officer of Verastem Oncology. "We closed the first quarter with just over \$127 million in cash, cash equivalent and investments, leaving us well positioned to execute on our two ongoing Phase 2 studies evaluating VS-6766 and defactinib in LGSOC and KRAS G12V non-small cell lung cancer (NSCLC), as well as our other key corporate objectives."

Recent Corporate Highlights

LGSOC

- Reported updated data from the LGSOC cohort of the ongoing, investigator-sponsored Phase 1/2 FRAME study evaluating VS-6766 in combination with defactinib in patients with recurrent LGSOC. Combination continues to demonstrate activity, durability and a favorable tolerability profile, including in patients who have progressed following treatment with a MEK inhibitor.
 - Overall response rate (ORR) across all patients was 52% (11 of 21 patients).
 - ORR for patients with KRAS mutant LGSOC was 70% (7 of 10 patients).
 - ORR for patients with wild type LGSOC was 44% (4 of 9 patients).
 - The most common side effects were Grade 1/2 rash, creatine kinase elevation, nausea, hyperbilirubinemia and diarrhea, which were reversible.
- Company-sponsored, registration-directed Phase 2 study (RAMP 201) underway investigating VS-6766 alone and in combination with defactinib for the treatment of recurrent LGSOC. Study recently expanded to include both KRAS mutant and KRAS wild-type patients in the selection phase to determine the optimal go-forward regimen for both types of LGSOC.

KRAS G12V Mutant NSCLC

- Company-sponsored, registration-directed Phase 2 study (RAMP 202) underway investigating VS-6766 alone and in combination with defactinib for the treatment of patients with KRAS G12V mutant NSCLC.

Upcoming Milestones and Key Priorities for 2021-2022

LGSOC

- Updated data from Phase 1/2 FRAME study to be submitted for presentation at a major medical meeting during the second half of 2021.
- Complete selection portion of RAMP 201 during first half of 2022; commence expansion portion.

G12V NSCLC

- Complete selection portion of RAMP 202 during first half of 2022; commence expansion portion.

First Quarter 2021 Financial Results

Verastem Oncology ended the first quarter 2021 with cash, cash equivalents and investments of \$127.1 million.

Total revenue for the three months ended March 31, 2021 (2021 Quarter) was \$1.0 million, compared to \$5.1 million for the three months ended March 31, 2020 (2020 Quarter).

Total operating expenses for the 2021 Quarter were \$15.1 million, compared to \$31.4 million for the 2020 Quarter.

Selling, general and administrative expenses for the 2021 Quarter were \$6.2 million, compared to \$19.6 million for the 2020 Quarter. The decrease of \$13.4 million, or 68.4%, primarily resulted from the Company's shift in strategic direction and COPIKTRA sale to Secura Bio, Inc., which led to lower employee related expenses and consulting and professional fees.

Research and development expense for the 2021 Quarter was \$8.9 million, compared to \$10.9 million for the 2020 Quarter. The decrease of \$2.0 million, or 18.3%, was primarily related to the upfront non-refundable payment of \$3.0 million to Chugai Pharmaceutical Co., Ltd for the VS-6766 license in the 2020 Quarter and decreased contract research organization costs, partially offset by increased drug substance and drug product costs and increased investigator sponsored trial expenses.

Net loss for the 2021 Quarter was \$15.0 million, or \$0.09 per share (basic and diluted), compared to \$38.0 million, or \$0.35 per share (basic and diluted), for the 2020 Quarter.

For the 2021 Quarter, non-GAAP adjusted net loss was \$12.4 million, or \$0.07 per share (diluted), compared to non-GAAP adjusted net loss of \$21.3 million, or \$0.20 per share (diluted), for the 2020 Quarter. Please refer to the GAAP to Non-GAAP Reconciliation attached to this press release.

Financial Guidance and Outlook

With the proceeds from the sale of COPIKTRA, Verastem Oncology expects that it will have a cash runway until at least 2024 to deliver on the current programs for VS-6766 and defactinib, including clinical and regulatory milestones and development in LGSOC and KRAS mutant NSCLC. Verastem Oncology expects its 2021 annual operating expenses to be approximately \$50 million.

Use of Non-GAAP Financial Measures

To supplement Verastem Oncology's condensed consolidated financial statements, which are prepared and presented in accordance with generally accepted accounting principles in the United States (GAAP), the Company uses the following non-GAAP financial measures in this press release: non-GAAP adjusted net loss and non-GAAP net loss per share. These non-GAAP financial measures exclude certain amounts or expenses from the corresponding financial measures determined in accordance with GAAP. Management believes this non-GAAP information is useful for investors, taken in conjunction with the Company's GAAP financial statements, because it provides greater transparency and period-over-period comparability with respect to the Company's operating performance and can enhance investors' ability to identify operating trends in the Company's business. Management uses these measures, among other factors, to assess and analyze operational results and trends and to make financial and operational decisions. Non-GAAP information is not prepared under a comprehensive set of accounting rules and should only be used to supplement an understanding of the Company's operating results as reported under GAAP, not in isolation or as a substitute for, or superior to, financial information prepared and presented in accordance with GAAP. In addition, these non-GAAP financial measures are unlikely to be comparable with non-GAAP information provided by other companies. The determination of the amounts that are excluded from non-GAAP financial measures is a matter of management judgment and depends upon, among other factors, the nature of the underlying expense or income amounts. Reconciliations between these non-GAAP financial measures and the most comparable GAAP financial measures for the three months ended March 31, 2021 and 2020 are included in the tables accompanying this press release after the unaudited condensed consolidated financial statements.

About VS-6766

VS-6766 (formerly known as CH5126766 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, KRAS mt NSCLC and colorectal cancer (CRC). The FRAME study was expanded to include new cohorts in pancreatic cancer, KRASmt endometrioid cancer and KRAS-G12V NSCLC. Verastem Oncology is also supporting an investigator-initiated Phase 2 trial evaluating VS-6766 with defactinib in patients with metastatic uveal melanoma.

Verastem Oncology has initiated Phase 2 registration-directed trials of VS-6766 with defactinib in patients with recurrent LGSOC and in patients with recurrent KRAS-G12V NSCLC as part of its RAMP (Raf And Mek Program).

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 and future plans and prospects, including statements related to the expected use of the Company's cash and cash equivalents, the potential clinical value of the RAF/MEK/FAK combination and the timing of commencing and completing 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 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, 2020 as filed with the Securities and Exchange Commission (SEC) on March 18, 2021 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 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.

Verastem, Inc.

Condensed Consolidated Balance Sheets

(in thousands)

(unaudited)

March 31, December 31,

2021 2020

Cash, cash equivalents, & investments	\$ 127,095	\$ 147,221
Accounts receivable, net	1,125	239
Prepaid expenses and other current assets	5,456	3,473
Property and equipment, net	335	416
Right-of-use asset, net	2,628	2,726
Restricted cash and other assets	262	274
Total assets	\$ 136,901	\$ 154,349

Current Liabilities	\$ 11,844	\$ 17,093
Convertible senior notes	19,672	19,051
Lease Liability, long-term	2,776	2,931
Stockholders' equity	102,609	115,274
Total liabilities and stockholders' equity	\$ 136,901	\$ 154,349

Verastem, Inc.

Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

(unaudited)

Three months ended March 31,

	2021	2020
Revenue:		
Product revenue, net	\$ —	\$ 5,034
License and collaboration revenue	—	22
Sale of COPIKTRA license and related assets revenue	850	—
Transition services revenue	156	—
Total revenue	1,006	5,056
Operating expenses:		

Cost of sales - product	—	495
Cost of sales - intangible amortization	—	392
Research and development	8,896	10,924
Selling, general and administrative	6,218	19,604
Total operating expenses	15,114	31,415
Loss from operations	(14,108)	(26,359)
Other expense	—	(1,313)
Interest income	52	356
Interest expense	(975)	(10,674)
Net loss	(15,031)	(37,990)
Net loss per share—basic and diluted	\$ (0.09)	\$ (0.35)

Weighted average common shares outstanding used in computing:

Net loss per share – basic and diluted	171,586	108,153
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Verastem, Inc.

Reconciliation of GAAP to Non-GAAP Financial Information

(in thousands, except per share amounts)

(unaudited)

Three months ended March 31,

2021 2020

Net loss reconciliation

Net loss (GAAP basis)	\$ (15,031)	\$ (37,990)
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Adjust:

Amortization of acquired intangible asset	—	392
Stock-based compensation expense	1,980	1,370
Non-cash interest, net	636	8,779
Severance and Other	—	1,788
Change in fair value of derivative	—	1,313
Chugai license payment	—	3,000

Adjusted net loss (non-GAAP basis)	\$ (12,415)	\$ (21,348)
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Reconciliation of Net loss Per Share

Net loss per share – diluted (GAAP Basis)	\$ (0.09)	\$ (0.35)
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Adjust per diluted share

Amortization of acquired intangible asset	—	—
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Stock-based compensation expense	0.01	0.01
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Non-cash interest, net	0.01	0.08
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Severance and Other	—	0.02
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Change in fair value of derivative	—	0.01
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Chugai license payment	—	0.03
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Adjusted net loss per share – diluted (non-GAAP Basis)	\$ (0.07)	\$ (0.20)
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Weighted average common shares outstanding used in computing net loss per share—diluted	171,586	108,153
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Investors:

Ajay Munshi
Vice President, Corporate Development
+1 781-469-1579
amunshi@verastem.com

Sherri Spear
Argot Partners
+1 212 600 1902
sherri@argotpartners.com

Media:

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

Source: Verastem Oncology