



Verastem Oncology Reports Fourth Quarter and Full Year 2020 Financial Results, Clinical Updates and Guidance for Key Milestones in 2021 and 1H 2022

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Two Phase 2, Registration-Directed Trials Underway in High Unmet Need Indications with Multiple Key Catalysts Expected in 1H 2022

RAMP 201 Low-Grade Serous Ovarian Cancer Trial Expanded to Now Include Patients with KRAS Wild-Type Tumors in Selection Phase Based on New Clinical Data

Strong Balance Sheet with Cash, Cash Equivalents and Investments Totaling \$147.2 Million, Expected to Have Cash Runway Until at Least 2024

BOSTON--(BUSINESS WIRE)--Mar. 18, 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 and full year ending December 31, 2020, and highlighted its key corporate objectives for 2021 and early 2022.

"We are encouraged as we continue to gain greater understanding of the profile of our RAF/MEK inhibitor VS-6766 and its role in blocking multiple nodes in the RAS pathway without the resistance and tolerability issues that have historically been challenges. Based on its unique profile, we believe that VS-6766 could play a significant role in combination therapy across multiple tumor types and where patients need better options," said Brian Stuglik, Chief Executive Officer of Verastem Oncology. "We have a strong balance sheet to execute on our corporate objectives and report across multiple key catalysts. Our annual spend is projected to be approximately \$50 million for 2021, and we still anticipate being comfortably funded until at least 2024."

In Q4 2020, the Company advanced VS-6766 into two Phase 2 registration-directed clinical studies: RAMP 201 in patients with recurrent low-grade serous ovarian cancer (LGSOC) and RAMP 202 in patients with KRAS-G12V mutant non-small cell lung cancer (NSCLC). Both are evaluating VS-6766 with the Company's FAK inhibitor, defactinib. The combination of VS-6766 and defactinib had been found to be clinically active in patients with KRAS mutant tumors through an ongoing investigator-initiated Phase 1/2 FRAME study. The FRAME study is evaluating the combination in patients with recurrent LGSOC, KRAS mutant NSCLC, KRAS-G12V mutant NSCLC, pancreatic cancer and KRAS mutant endometrioid cancer.

"In the FRAME study, additional patients with recurrent LGSOC have now responded to treatment with clinically meaningful results for patients with KRAS wild-type tumors, which represents approximately 70% of all LGSOC patients. The results add to the robust and durable responses that have been demonstrated for patients with KRAS mutations," said Jonathan Pachter, Chief Scientific Officer of Verastem Oncology. "The majority of LGSOC cases are RAS pathway-driven, and these new data support the premise that VS-6766 could be a treatment for all LGSOC patients. Therefore, we are expanding the selection phase of our company-sponsored RAMP 201 study to now include both patient populations (KRAS mutant and KRAS wild-type) to determine the optimal go-forward regimen for both."

In an updated December 2020 read-out of the FRAME study LGSOC cohort (n=24), the overall response rate (ORR) is 52% (11 of 21 response evaluable patients), with KRAS mutant ORR at 70% (7 of 10 response evaluable patients), KRAS wild-type ORR at 44% (4 of 9 response evaluable patients) and KRAS status undetermined ORR at 0% (0 of 2 response evaluable patients). As reported previously, the most common side effects seen in the study were rash, creatine kinase elevation, nausea, hyperbilirubinemia and diarrhea, most being NCI CTC Grade 1/2 and all were reversible. The data from the LGSOC cohort are anticipated to be presented at a major medical meeting during the second half of 2021.

Recent Corporate Highlights

- Phase 2 registration-directed (RAMP 201) study underway investigating VS-6766 alone and in combination with defactinib for the treatment of patients with recurrent LGSOC.
 - Supportive updated data from LGSOC cohort of the investigator-sponsored Phase 1/2 FRAME study continues to show encouraging clinical activity, durability and a favorable safety profile in both the overall patient population and in the subgroup of patients with KRAS mutant LGSOC, including patients who had previously progressed following treatment with a MEK inhibitor.
 - Ongoing data from the FRAME study are demonstrating clinically meaningful results in patients with KRAS wild-type LGSOC in addition to those with KRAS mutation as more patients have had an opportunity to respond to treatment.
- Phase 2 registration-directed (RAMP 202) study underway investigating VS-6766 alone and in combination with defactinib for the treatment of patients with recurrent KRAS-G12V mutant NSCLC.
- Closed COPIKTRA sale to Secura Bio, Inc. (Secura) in a deal valued at up to \$311 million, plus royalties.

- Ended 2020 with \$147.2 million in cash, cash equivalents and investments.

Upcoming Milestones and Key Priorities for 2021-2022

LGSOC

- Amend RAMP 201 protocol to add enrollment of patients with KRAS wild-type LGSOC in selection phase.
- Report top-line results from the selection phase of RAMP 201 and commence expansion phase during the first half of 2022.
- Report updated data from Phase 1/2 FRAME study LGSOC cohort, including the KRAS wild-type population, during the second half of 2021.

NSCLC

- Report top-line results from the selection phase of RAMP 202 and commence expansion phase during the first half of 2022.
- Updated data from the Phase 1/2 FRAME study NSCLC cohort will be presented by the investigator at the 2021 American Association for Cancer Research (AACR) Annual Meeting. The presentation will include an update on all variants of mutant KRAS, including further updates on the KRAS G12V NSCLC patients who had been previously highlighted. As no new KRAS G12V patients were enrolled in the original FRAME study, a KRAS G12V-specific NSCLC cohort of FRAME has been opened in addition to the Company-sponsored RAMP 202 registration-directed trial.

Corporate and Financial

- Expect 2021 annual operating expenses of approximately \$50 million.

Fourth Quarter 2020 Financial Results

Total revenue for the three months ended December 31, 2020 (2020 Quarter) was \$0.5 million, compared to \$3.6 million for the three months ended December 31, 2019 (2019 Quarter).

Total research and development (R&D) and selling, general and administrative (SG&A) expenses for the 2020 Quarter were \$17.2 million, compared to \$36.2 million for the 2019 Quarter.

R&D expense for the 2020 Quarter was \$10.1 million, compared to \$12.5 million for the 2019 Quarter. The decrease of \$2.4 million, or 18.5%, was primarily related to a reduction in contract research organization costs partially offset by an increase in drug substance and drug product manufacturing costs.

SG&A expense for the 2020 Quarter was \$7.1 million, compared to \$23.7 million for the 2019 Quarter. The decrease of \$16.6 million, or 70.1%, primarily resulted from the Company's shift in strategic direction and COPIKTRA sale to Secura which led to lower employee related expenses and consulting and professional fees.

Net loss for the 2020 Quarter was \$(19.9) million, or \$(0.12) per share (basic and diluted), compared to \$(38.8) million, or \$(0.51) per share (basic and diluted), for the 2019 Quarter.

For the 2020 Quarter, non-GAAP adjusted net loss was \$(14.8) million, or \$(0.09) per share (diluted), compared to non-GAAP adjusted net loss of \$(30.3) million, or \$(0.40) per share (diluted), for the 2019 Quarter. Please refer to the GAAP to Non-GAAP Reconciliation attached to this press release.

Full-Year 2020 Financial Results

Verastem Oncology ended 2020 with cash, cash equivalents and investments of \$147.2 million.

During the year ended December 31, 2020 (2020 Period), Verastem Oncology repaid in full all principal, accrued and unpaid interest, fees, and expenses under the Amended Loan Agreement with Hercules in an aggregate amount of \$37.4 million and the Amended Loan Agreement was terminated along with Hercules' commitment to provide funding under any future term loans. All liens on substantially all of the Company's assets to secure the loans under the Amended Loan Agreement have been terminated and released.

Total revenue for the 2020 Period was \$88.5 million, compared to \$17.5 million for the year ended December 31, 2019 (2019 Period).

Sale of COPIKTRA license and related assets revenue for the 2020 Period was \$70.0 million, compared to \$0.0 million for the 2019 Period. The 2020 Period was comprised of a \$70.0 million upfront payment received as part of the COPIKTRA sale to Secura.

Net product revenue for the 2020 Period was \$15.2 million, compared to \$12.3 million for the 2019 Period. License and collaboration revenue for the 2020 Period was \$2.9 million, compared to \$5.1 million for the 2019 Period.

Total R&D and SG&A expenses for the 2020 Period were \$104.1 million, compared to \$147.0 million for the 2019 Period.

R&D expense for the 2020 Period was \$41.4 million, compared to \$45.8 million for the 2019 Period. The decrease of \$4.4 million, or 9.6%, was primarily related to a reduction in contract research organization costs and employee related expense, partially offset by the \$3.0 million up-front non-refundable payment made to Chugai Pharmaceuticals, Co. Ltd. for the VS-6766 license in the first quarter of 2020.

SG&A expense for the 2020 Period was \$62.7 million, compared to \$101.2 million for the 2019 Period. The decrease of \$38.5 million, or 38.0%, was

primarily due to the Company's shift in strategic direction and sale of COPIKTRA business which led to lower employee related expense and consulting and professional fees.

Net loss for the 2020 Period was \$(67.7) million, or \$(0.44) per share (basic and diluted), compared to \$(149.2) million, or \$(2.00) per share (basic and diluted), for the 2019 Period.

For the 2020 Period, non-GAAP adjusted net loss was \$(37.8) million, or \$(0.25) per share (diluted), compared to non-GAAP adjusted net loss of \$(126.0) million, or \$(1.69) per share (diluted), for the 2019 Period. 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 and year ended December 31, 2020 and 2019 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, 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, 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)

	December 31, December 31,	
	2020	2019
Cash, cash equivalents, & investments	\$ 147,221	\$ 75,506
Accounts receivable, net	239	2,524
Inventory	—	3,096
Restricted cash, prepaid expenses and other current assets	3,473	3,835
Property and equipment, net	416	947
Intangible assets, net	—	20,008
Right-of-use asset, net	2,726	3,077

Restricted cash and other assets	274	36,053
Total assets	\$ 154,349	\$ 145,046

Current Liabilities	\$ 17,093	\$ 29,890
Long-term debt	—	35,067
Convertible senior notes	19,051	68,556
Lease Liability, long-term	2,931	3,489
Other liabilities	—	870
Stockholders' equity	115,274	7,174
Total liabilities and stockholders' equity	\$ 154,349	\$ 145,046

Verastem, Inc.

Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

(unaudited)

Three months ended December 31, Year ended December 31,

	2020	2019	2020	2019
Revenue:				
Product revenue, net	\$ 134	\$ 3,617	\$ 15,232	\$ 12,339
License and collaboration revenue	—	—	2,912	5,117
Sale of Copiktra license and related assets revenue	—	—	70,000	—
Transition services revenue	372	—	372	—
Total revenue	506	3,617	88,516	17,456
Operating expenses:				
Cost of sales - product	12	332	1,765	1,238
Cost of sales - intangible amortization	—	393	793	1,569
Cost of sales – sale of Copiktra license and related assets	—	—	31,187	—
Research and development	10,153	12,455	41,376	45,778

Selling, general and administrative	7,095	23,728	62,755	101,212
Total operating expenses	17,260	36,908	137,876	149,797
Loss from operations	(16,754)	(33,291)	(49,360)	(132,341)
Other expense	—	(641)	(1,313)	(641)
Interest income	18	611	515	4,381
Interest expense	(1,354)	(5,453)	(15,794)	(20,608)
Loss on debt extinguishment	(1,580)	—	(1,580)	—
Net loss before income taxes	\$ (19,670)	\$ (38,774)	\$ (67,532)	\$ (149,209)
Income tax expense	194	—	194	—
Net loss	(19,864)	(38,774)	(67,726)	(149,209)
Net loss per share—basic and diluted	\$ (0.12)	\$ (0.51)	\$ (0.44)	\$ (2.00)

Weighted average common shares outstanding used in computing:

Net loss per share – basic and diluted	169,902	76,331	153,330	74,578
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Verastem, Inc.

Reconciliation of GAAP to Non-GAAP Financial Information

(in thousands, except per share amounts)

(unaudited)

	Three months ended December 31,		Year ended December 31,	
	2020	2019	2020	2019
Net loss reconciliation				
Net loss (GAAP basis)	\$ (19,864)	\$ (38,774)	\$ (67,726)	\$ (149,209)
Adjust:				
Amortization of acquired intangible asset	—	393	793	1,569
Stock-based compensation expense	2,933	1,311	8,118	8,539
Non-cash interest, net	554	2,705	10,319	7,131
Severance and Other	(160)	1,232	4,621	3,200
Change in fair value of derivative	—	641	1,313	641

Chugai license payment	—	—	3,000	—
Loss on debt extinguishment	1,580	—	1,580	
Notes third party exchange costs	171	2,168	171	2,168
Adjusted net loss (non-GAAP basis)	\$ (14,786)	\$ (30,324)	\$ (37,811)	\$ (125,961)

Reconciliation of Net loss Per Share

Net loss per share – diluted (GAAP Basis)	\$ (0.12)	\$ (0.51)	\$ (0.44)	\$ (2.00)
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Adjust per diluted share

Amortization of acquired intangible asset	—	—	—	0.02
Stock-based compensation expense	0.02	0.02	0.05	0.11
Non-cash interest, net	—	0.03	0.07	0.10
Severance and Other	—	0.02	0.03	0.04
Change in fair value of derivative	—	0.01	0.01	0.01
Chugai license payment	—	—	0.02	—
Loss on debt extinguishment	0.01	—	0.01	—
Notes third party exchange costs	—	0.03	—	0.03
Adjusted net loss per share – diluted (non-GAAP Basis)	\$ (0.09)	\$ (0.40)	\$ (0.25)	\$ (1.69)

Weighted average common shares outstanding used in computing net loss per share—diluted	169,902	76,331	153,330	74,578
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