Verastem Oncology Reports First Quarter 2020 Financial Results and Highlights Recent Company Progress

May 7, 2020

Company to Pursue Low-Grade Serous Ovarian Cancer Indication for VS-6766 with Defactinib; On Track to Initiate Discussions with FDA

Company Reports $5.0M in Net Product Revenue; With Newly Strengthened Balance Sheet, Company is Well Positioned to Execute on Key Corporate Objectives in 2020 and Beyond

BOSTON--(BUSINESS WIRE)--May 7, 2020-- Verastem, Inc. (Nasdaq:VSTM) (also known as Verastem Oncology), a biopharmaceutical company committed to developing and commercializing new medicines for patients battling cancer, today reported financial results for the three months ending March 31, 2020, and provided an overview of recent corporate highlights.

"The early part of 2020 was marked most importantly by the strategic in-licensing of the novel RAF/MEK inhibitor VS-6766 which is being investigated in combination with defactinib, our lead FAK inhibitor, in KRAS mutant tumors," commented Brian Stuglik, Chief Executive Officer of Verastem Oncology. "The encouraging preliminary Phase 1 results from the investigator-initiated FRAME study recently reported at AACR in KRAS mutant low-grade serous ovarian cancer (LGSOC) will form the basis of our upcoming discussions with the U.S. Food and Drug Administration (FDA) and other regulatory authorities. We look forward to identifying the path forward for this novel combination and to embarking on a registration-directed clinical trial in LGSOC as rapidly as possible."

RAF/MEK and FAK Inhibition in KRAS Mutant Solid Tumors

- ***Presented Preliminary Results from investigator-initiated Phase 1 FRAME Study Investigating the Combination of VS-6766 and Defactinib in KRAS Mutant Solid Tumors at AACR 2020 Virtual Meeting I.*** In a virtual poster presentation, Udai Banerji, MBBS, MD, DNB, PhD, FRCP, NIHR, Professor of Molecular Cancer Pharmacology at The Institute of Cancer Research and Honorary Consultant in Medical Oncology at The Royal Marsden NHS Foundation Trust, highlighted data from this ongoing, open-label, dose-escalation and expansion investigator-initiated study investigating the combination of VS-6766, Verastem Oncology's RAF/MEK inhibitor, with defactinib, the Company's FAK inhibitor, in patients with KRAS mutant advanced solid tumors, including LGSOC and non-small cell lung cancer (NSCLC).

  The VS-6766/defactinib combination was well tolerated by the patients in the trial. The recommended Phase 2 dose has now been established. In the patients with KRAS mutant LGSOC (n=6), 4 patients responded for an overall response rate (ORR) of 67%. The median time on treatment for these 4 patients was 20.5 months. In the patients with KRAS mutant NSCLC (n=10), 1 patient responded (partial response) for an ORR of 10% and a total of 8 patients achieved disease control for a disease control rate (DCR) of 80%. Additionally, in patients with KRAS mutant NSCLC, 8 remained on therapy for at least 12 weeks and 3 remained on therapy for at least 24 weeks. Expansion cohorts remain ongoing in LGSOC and NSCLC.

  Verastem Oncology plans to initiate discussions with the FDA during second quarter of 2020, with the goal of commencing a registration-directed clinical trial investigating the VS-6766/defactinib combination in patients with LGSOC by the end of 2020.

  Subsequent Analysis

  Based on an observation of higher response rates seen in patients with $KRAS^{G12V}$ mutations in the investigator-initiated Phase 1 combination study, Verastem Oncology conducted a post-hoc combined analysis with data from the combination study and the prior single-agent study that utilized a twice-weekly dosing schedule of VS-6766 to get a more complete picture of activity in $KRAS^{G12V}$ mutations. This analysis showed early signals of activity in patients with $KRAS^{G12V}$ mutated NSCLC. The Company plans to evaluate this finding further in a prospective NSCLC clinical trial.

- ***New Strategic Direction.*** Verastem Oncology recently licensed exclusive global development and commercialization rights to VS-6766 (CH5126766), a unique and promising inhibitor of the RAF/MEK signaling pathway. The Company then announced its plans to accelerate development of VS-6766 in combination with defactinib for the treatment of KRAS mutant solid tumors. The rationale for investigating the combinations of VS-6766 and defactinib is supported by single-agent Phase 1 and Phase 2 studies which investigated defactinib in KRAS mutant NSCLC[1] and VS-6766 in KRAS mutant NSCLC and LGSOC[2].

COPIKTRA® (Duvelisib)

- ***First Patient Dosed in Chinese Study Evaluating Duvelisib in Patients with Relapsed or Refractory Follicular Lymphoma (FL).*** Verastem Oncology's partner, CSPC Pharmaceutical Group Limited, has dosed the first patient in a single-arm, open-label, multi-center pivotal study designed to evaluate the antitumor activity and safety of duvelisib in patients with relapsed or refractory FL. This study is expected to serve as a bridging study based on the efficacy and safety observed in Verastem Oncology's Phase 2 DYNAMO study. The results of this study will form the basis of a regulatory submission for duvelisib for the treatment of relapsed or refractory FL in China.

- ***Ongoing U.S. Commercial Rollout of COPIKTRA***. COPIKTRA, the Company's marketed oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first FDA-approved dual inhibitor of PI3K-delta and PI3K-gamma generated $5.0 million in net product revenues during the first quarter of 2020, a 194% increase over the first quarter of 2019 and a 39% increase over the fourth quarter of 2019.

Corporate and Financial

- ***Strengthened the Balance Sheet Through a Private Placement with Premier Life Science Investors.*** On March 3, 2020, Verastem Oncology completed a private placement offering of approximately 46.5 million shares of its common stock to certain institutional investors, including RA Capital Management, Vivo Capital, Venrock Healthcare Capital Partners, Farallon Capital Management, Acuta Capital, EcoR1 Capital LLC, Avidity Partners and Logos Capital at a price of $2.15 per share, a 12.6% premium to the February 27, 2020 closing price. The gross proceeds to Verastem Oncology were $100 million. After deducting the underwriting discounts and commissions and other estimated offering expenses, net proceeds to the Company were approximately $93.8 million.

- ***Convertible Senior Second Lien Notes (2019 Notes) Fully Converted to Common Stock.*** During the first quarter of 2020, all of the remaining 2019 Notes were converted into shares of common stock. As of March 31, 2020, the Company had approximately $63.3 million in outstanding debt.

- ***Appointed John H. Johnson to the Board of Directors.*** In April, Verastem Oncology announced the appointment of John H. Johnson to its Board of Directors. Mr. Johnson's career spans multiple executive management roles at leading global corporations where he was responsible for overseeing oncology and immunology drug development initiatives and commercialization. Mr. Johnson will serve on the Compensation and Nominating and Governance Committees.

First Quarter 2020 Financial Results

Net product revenue for the three months ending March 31, 2020 (2020 Quarter) was $5.0 million, compared to $1.7 million for the three months ending March 31, 2019 (2019 Quarter). COPIKTRA demand units for the 2020 Quarter increased 178% compared to the 2019 Quarter. License and collaboration revenue for the 2020 Quarter was less than $0.1 million. There was no license and collaboration revenue for the 2019 Quarter. 2020 Quarter license and collaboration revenue was comprised of duvelisib shipments to our partner, CSPC Pharmaceutical Group Limited.

Total operating expenses for the 2020 Quarter were $31.4 million, compared to $36.3 million for the 2019 Quarter. Included within operating expenses for the 2020 Quarter is a non-recurring charge of $3.0 million related to an up-front non-refundable payment to Chugai Pharmaceutical Co. Ltd. (Chugai) for the VS-6766 license, $1.8 million of severance expense and $1.4 million of non-cash stock-based compensation expense.

Research and development (R&D) expense for the 2020 Quarter was $10.9 million, compared to $9.8 million for the 2019 Quarter. The increase of $1.1 million, or 11%, was primarily related to the up-front non-refundable payment of $3.0 million to Chugai for the VS-6766 license. This was partially offset by a decrease of $1.3 million in contract research organization costs and a decrease of $0.6 million in costs for clinical supply, drug substance and drug product.

Selling, general and administrative (SG&A) expense for the 2020 Quarter was $19.6 million, compared to $26.0 million for the 2019 Quarter. The decrease of $6.4 million, or 25%, primarily resulted from a decrease of $2.8 million in consulting and professional fees, principally related to the support of commercial launch activities in the 2019 Quarter, a decrease of $2.3 million in personnel related costs, including non-cash stock-based compensation as a result of reduced headcount, and a decrease of $1.3 million in reduced travel and other costs.

Net loss for the 2020 Quarter was $38.0 million, or $0.35 per share (basic and diluted), compared to $38.1 million, or $0.52 per share (basic and diluted), for the 2019 Quarter. The 2020 Quarter includes $8.1 million of non-cash interest expense related to conversions of Convertible Senior Notes into shares of common stock.

For the 2020 Quarter, non-GAAP adjusted net loss was $21.3 million, or $0.20 per share (diluted), compared to non-GAAP adjusted net loss of $33.8 million, or $0.46 per share (diluted), for the 2019 Quarter. Please refer to the GAAP to Non-GAAP Reconciliation attached to this press release.

Verastem Oncology ended the first quarter of 2020 with cash, cash equivalents and short-term investments of $170.7 million.

Financial Guidance for Fiscal 2020

As a result of its new strategic direction, Verastem Oncology expects to reduce its operating expenses by approximately 40% for 2020 compared to 2019. Based on its current operating plans, the Company expects its R&D and SG&A expenses for the full year 2020 to be in the range of $70 million to $85 million. The company is guiding that 2020 COPIKTRA revenues may be approximately $16 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, 2020 and 2019 are included in the tables accompanying this press release after the unaudited condensed consolidated financial statements.

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 opportunity to rapidly advance the development of clinical programs through Verastem Oncology's expanded development pipeline and strengthened balance sheet, the timing of top-line results for clinical trials, anticipated reductions in operating expenses from Verastem Oncology's strategic realignment, the timing of commencing a registration-directed trial for VS-6766 and financial guidance estimates. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," 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.

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 CH5126766 (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 CH5126766 (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 CH5126766 (VS-6766); that we will not pursue or submit regulatory filings for our product candidates, that our product candidates will not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients, and that the duration and impact of COVID-19 may affect, precipitate or exacerbate one or more of the foregoing risks and uncertainties.

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] 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 2020	December 31, 2019
Cash, cash equivalents, & investments	$ 135,061	$ 75,506
Accounts receivable, net	3,326	2,524
Inventory	4,372	3,096
Prepaid expenses and other current assets	5,887	3,835
Property and equipment, net	866	947
Intangible assets, net	19,616	20,008
Right-of-use asset, net	2,995	3,077
Restricted cash and other assets	36,031	36,053
Total assets	**$ 208,154**	**$ 145,046**
Current Liabilities	$ 25,728	$ 29,890
Long-term debt	35,276	35,067
Convertible senior notes	19,938	68,556
Lease Liability, long-term	3,359	3,489
Other liabilities	870	870
Stockholders' equity	122,983	7,174
Total liabilities and stockholders' equity	**$ 208,154**	**$ 145,046**

Verastem, Inc.

Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

(unaudited)

	Three months ended March 31,	
	2020	**2019**
Revenue:		
Product revenue, net	$ 5,034	$ 1,671
License and collaboration revenue	22	—
Total revenue	5,056	1,671
Operating expenses:		
Cost of sales - product	495	158
Cost of sales - intangible amortization	392	392
Research and development	10,924	9,758
Selling, general and administrative	19,604	26,033
Total operating expenses	31,415	36,341
Loss from operations	(26,359)	(34,670)
Other expense	(1,313)	—
Interest income	356	1,497
Interest expense	(10,674)	(4,929)
Net Loss	$ (37,990)	$ (38,102)
Net loss per share—basic and diluted	$ (0.35)	$ (0.52)
Weighted average common shares outstanding used in computing net loss per share—basic and diluted	108,153	73,854

Verastem, Inc.

Reconciliation of GAAP to Non-GAAP Financial Information

(in thousands, except per share amounts)

(unaudited)

	Three months ended March 31,	
	2020	**2019**
Net Loss Reconciliation		
Net Loss (GAAP basis)	$ (37,990)	$ (38,102)

Adjust:		
Amortization of acquired intangible asset	392	392
Stock-based compensation expense	1,370	2,248
Non-cash interest, net	8,779	1,608
Severance and Other	1,788	37
Change in fair value of derivative	1,313	—
Chugai License Payment	3,000	—
Adjusted Net Loss (non-GAAP basis)	$ (21,348)	$ (33,817)

Reconciliation of Net Loss Per Share		
Net Loss per share – diluted (GAAP Basis)	(0.35)	(0.52)
Adjust per diluted share		
Amortization of acquired intangible asset	0.00	0.01
Stock-based compensation expense	0.01	0.03
Non-cash interest, net	0.08	0.02
Severance and Other	0.02	0.00
Change in fair value of derivative	0.01	—
Chugai License Payment	0.03	—
Adjusted Net Loss per share – diluted (non-GAAP Basis)	$ (0.20)	$ (0.46)
Weighted average common shares outstanding used in computing net loss per share—diluted	108,153	73,854

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