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

August 10, 2020

Announced Path Forward for VS-6766 and Defactinib Combination Following Meeting with FDA

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

Company Monetizes COPIKTRA® (duvelisib) Providing Cash Runway Until at Least 2024

BOSTON--(BUSINESS WIRE)--Aug. 10, 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 June 30, 2020, and provided an overview of recent corporate highlights.

"The first half of 2020 has been a time of transformational change at Verastem Oncology. We recently announced our newest strategic transaction, the sale of COPIKTRA to Secura Bio, which allows us to monetize this asset while focusing our resources and efforts on advancing the VS-6766 and defactinib combination program in KRAS mutant solid tumors," commented Brian Stuglik, Chief Executive Officer of Verastem Oncology. "We are now looking forward to a catalyst-driven second half of 2020, including reporting updated data from the LGSOC arm of the investigator-initiated Phase 1/2 FRAME study in September and commencing registration-directed clinical trials in low-grade serous ovarian cancer (LGSOC) and KRAS mutant non-small cell lung cancer (NSCLC) by the end of this year."

Second Quarter 2020 and Recent Highlights

- ***Announced Path Forward for VS-6766/Defactinib Combination in LGSOC Following Meeting with U.S. FDA.*** 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 KRAS mutant NSCLC by the end of 2020.

- ***Selling COPIKTRA Franchise to Secura Bio in a Deal Totaling $311 Million, Plus Royalties.*** Verastem recently announced its entry into a definitive agreement to sell its global commercial and development rights to COPIKTRA in all oncology indications to Secura Bio, Inc. The transaction, which carries a total deal value of up to $311 million, plus royalties, will provide Verastem's current programs with a cash runway until at least 2024 and will allow the Company to focus its resources and efforts on the clinical development of VS-6766, its RAF/MEK inhibitor, and defactinib, its FAK inhibitor, in KRAS mutant solid tumors. Verastem is pursuing development of this combination in LGSOC and KRAS mutant NSCLC.

- ***Presented Preliminary Results from Investigator-initiated Phase 1 FRAME Study Evaluating 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 study in patients with KRAS mutant advanced solid tumors, including LGSOC and NSCLC. Preliminary data demonstrated a 67% overall response rate and long duration of therapy among patients with LGSOC. Based on higher response rates seen in NSCLC patients with KRAS-G12V mutations, Verastem will also be further exploring the role of the VS-6766/defactinib combination in KRAS-G12V NSCLC. Expansion cohorts remain ongoing in LGSOC and NSCLC and the study will be expanding to include new cohorts in pancreatic, KRAS mutant endometrial and KRAS-G12V NSCLC.

- ***Presented New Preclinical VS-6766/Defactinib Combination Data in Uveal Melanoma at AACR 2020 Virtual Meeting II.*** In this study, researchers identified and reinforced that FAK inhibition is a viable pathway to inhibit downstream from the GNAQ pathway, which is constitutively active in uveal melanoma. It was observed that co-targeting FAK and RAF/MEK signaling led to tumor collapse in uveal melanoma xenograft and liver metastasis models *in vivo*. Based on these encouraging results, Verastem plans to support an investigator-sponsored, Phase 2 clinical testing of the VS-6766/defactinib combination in uveal melanoma, which is expected to commence by the end of 2020.

- ***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.

Upcoming Milestones

- Close transaction with Secura Bio during the third quarter of 2020.
- Present updated data from the LGSOC cohort of the investigator-initiated Phase 1/2 FRAME study evaluating VS-6766 and defactinib in KRAS mutant solid tumors in September, including at the 2nd Annual RAS-Targeted Drug Development Conference on September 16, 2020.
- Present new preclinical data from studies investigating VS-6766 and defactinib in combination with KRAS-G12C inhibitors in September, including at the 2nd Annual RAS-Targeted Drug Development Conference on September 16, 2020.
- Commence registration-directed clinical trials in LGSOC and KRAS mutant NSCLC by the end of 2020.
- Submit updated data from the NSCLC cohort of the investigator-initiated Phase 1/2 FRAME study to the International Association for the Study of Lung Cancer (IASLC) World Lung Cancer Conference, taking place in January 2021.

Second Quarter 2020 Financial Results

Net product revenue for the three months ending June 30, 2020 (2020 Quarter) was $4.2 million, compared to $3.0 million for the three months ending June 30, 2019 (2019 Quarter). License and collaboration revenue for both the 2020 Quarter and 2019 Quarter was $0.1 million.

Total operating expenses for the 2020 Quarter were $25.6 million, compared to $41.4 million for the 2019 Quarter.

Research and development (R&D) expense for the 2020 Quarter was $9.3 million, compared to $11.3 million for the 2019 Quarter. The decrease of $2.0 million, or 18%, was primarily related to a decrease in contract research organization (CRO) costs and lower employee related expense.

Selling, general and administrative (SG&A) expense for the 2020 Quarter was $15.4 million, compared to $29.3 million for the 2019 Quarter. The decrease of $13.9 million, or 47%, primarily resulted from the company's shift in strategic direction which led to lower commercial program and employee related expense.

Net loss for the 2020 Quarter was $23.0 million, or $0.14 per share (basic and diluted), compared to $42.2 million, or $0.57 per share (basic and diluted), for the 2019 Quarter.

For the 2020 Quarter, non-GAAP adjusted net loss was $20.5 million, or $0.12 per share (diluted), compared to non-GAAP adjusted net loss of $35.6 million, or $0.48 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 second quarter of 2020 with cash, cash equivalents and short-term investments of $160.8 million.

Financial Guidance and Outlook

With the proceeds from the sale of COPIKTRA, Verastem has 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 KRASmt NSCLC. Verastem expects its 2020 operating expenses to be approximately 40% lower than its 2019 operating expenses. As a result of its new strategic direction and operating plans, along with the expected sale of the COPIKTRA franchise during the third quarter and associated transition activities, the Company expects total operating expenses for the full year 2020 to be in the range of $80 million to $90 million. Beginning in 2021 Verastem expects its 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, 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, 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.[i,ii]

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 KRAS mutant tumors. In an ongoing investigator-initiated Phase I/2 FRAME study, the combination of VS-6766 and defactinib is being evaluated in patients with LGSOC, KRAS mutant 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, KRAS mutant 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.[iii,iv,v] 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.[vi] For more information on COPIKTRA, please visit www.COPIKTRA.com. Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

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.

Verastem, Inc.

Condensed Consolidated Balance Sheets

(in thousands)

(unaudited)

	June 30, 2020	**December 31, 2019**
Cash, cash equivalents, & investments	$ 125,328	$ 75,506
Accounts receivable, net	1,500	2,524
Inventory	6,316	3,096
Restricted cash, Prepaid expenses and other current assets	11,448	3,835
Property and equipment, net	791	947
Intangible assets, net	19,223	20,008
Right-of-use asset, net	2,909	3,077
Restricted cash and other assets	31,017	36,053
Total assets	**$ 198,532**	**$ 145,046**
Current Liabilities	$ 28,784	$ 29,890
Long-term debt	30,899	35,067
Convertible senior notes	20,381	68,556
Lease Liability, long-term	3,225	3,489
Other liabilities	870	870

Stockholders' equity	114,373	7,174
Total liabilities and stockholders' equity	**$ 198,532**	**$ 145,046**

Verastem, Inc.

Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

(unaudited)

	Three months ended June 30,		Six months ended June 30,	
	2020	**2019**	**2020**	**2019**
Revenue:				
Product revenue, net	$ 4,235	$ 3,019	$ 9,269	$ 4,690
License and collaboration revenue	72	117	94	117
Total revenue	4,307	3,136	9,363	4,807
Operating expenses:				
Cost of sales - product	392	377	887	534
Cost of sales - intangible amortization	393	392	785	785
Research and development	9,344	11,346	20,268	21,103
Selling, general and administrative	15,442	29,298	35,046	55,331
Total operating expenses	25,571	41,413	56,986	77,753
Loss from operations	(21,264)	(38,277)	(47,623)	(72,946)
Other expense	—	—	(1,313)	—
Interest income	122	1,268	478	2,765
Interest expense	(1,868)	(5,185)	(12,542)	(10,115)
Net Loss	$ (23,010)	$ (42,194)	$ (61,000)	$ (80,296)
Net loss per share—basic and diluted	$ (0.14)	$ (0.57)	$ (0.45)	$ (1.09)

Weighted average common shares outstanding used in computing net loss per share—basic and diluted	165,395	73,877	136,775	73,865

Verastem, Inc.

Reconciliation of GAAP to Non-GAAP Financial Information

(in thousands, except per share amounts)

(unaudited)

	Three months ended June 30,		**Six months ended June 30,**	
	2020	**2019**	**2020**	**2019**
Net Loss Reconciliation				
Net Loss (GAAP basis)	$ (23,010)	$ (42,194)	$ (61,000)	$ (80,296)
Adjust:				
Amortization of acquired intangible asset	393	392	785	785
Stock-based compensation expense	1,659	3,065	3,029	5,313
Non-cash interest, net	480	1,207	9,259	2,815
Severance and Other	11	1,957	1,798	1,994
Change in fair value of derivative	—	—	1,313	—
Chugai license payment	—	—	3,000	—
Adjusted Net Loss (non-GAAP basis)	$ (20,467)	$ (35,573)	$ (41,816)	$ (69,389)
Reconciliation of Net Loss Per Share				
Net Loss per share – diluted (GAAP Basis)	(0.14)	(0.57)	(0.45)	(1.09)
Adjust per diluted share				
Amortization of acquired intangible asset	—	—	0.01	0.01
Stock-based compensation expense	0.01	0.04	0.02	0.07
Non-cash interest, net	0.01	0.02	0.07	0.04

Severance and Other	—	0.03	0.01	0.03
Change in fair value of derivative	—	—	0.01	—
Chugai license payment	—	—	0.02	—
Adjusted Net Loss per share – diluted (non-GAAP Basis)	$ (0.12)	$ (0.48)	$ (0.31)	$ (0.94)
Weighted average common shares outstanding used in computing net loss per share—diluted	165,395	73,877	136,775	73,865

[i] 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.

[ii] 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.

[iii] 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.

[iv] 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.

[v] 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.

[vi] www.clinicaltrials.gov, NCT03372057.

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