



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

November 9, 2020

Announced Positive Updated Data from Ongoing Investigator-Initiated Phase 1/2 FRAME Study Evaluating VS-6766 and Defactinib Combination in Patients with Low-Grade Serous Ovarian Cancer

On Track to Commence Company-Sponsored Phase 2 Registration-Directed Trials by Year-End 2020 in Both Low-Grade Serous Ovarian Cancer and KRAS Mutant Non-Small Cell Lung Cancer

Strong Balance Sheet with Cash, Cash Equivalents and Investments Totaling \$205.7 Million; Strategic Sale of COPIKTRA® (duvelisib) Provides Cash Runway Until at Least 2024

BOSTON--(BUSINESS WIRE)--Nov. 9, 2020-- 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 ending September 30, 2020, and provided an overview of recent corporate highlights.

"The third quarter of 2020 was marked most notably by the sale of the COPIKTRA (duvelisib) franchise to Secura Bio in a deal valued at up to \$311 million, plus royalties. This strategic transaction allows us to focus our resources and efforts on advancing the VS-6766 and defactinib combination program in KRAS mutant solid tumors and provides us with a cash runway until at least 2024," said Brian Stuglik, Chief Executive Officer of Verastem Oncology. "Looking ahead to the remainder of the year, we remain on track to commence two new company-sponsored, registration-directed Phase 2 clinical trials by year end, one in low-grade serous ovarian cancer (LGSOC) and one in KRAS mutant non-small cell lung cancer (NSCLC)."

Third Quarter 2020 and Recent Highlights

- **Presented Updated Data from the Phase 1/2 FRAME Study in Patients with LGSOC.** In mid-September, Verastem reported positive updated results from the ongoing investigator-initiated Phase 1/2 FRAME study coinciding with a virtual oral presentation by Dr. Udai Banerji, Institute of Cancer Research and The Royal Marsden, at the 2nd Annual RAS-Targeted Drug Development (RTDD) Summit. The FRAME study is evaluating VS-6766, Verastem's RAF/MEK inhibitor, in combination with defactinib, its FAK inhibitor, in patients with LGSOC. The results demonstrated that the novel, intermittent, combination dosing schedule used in the FRAME study continues to show encouraging clinical activity, durability and a favorable safety profile in patients with KRAS mutant LGSOC, including patients who had previously progressed following treatment with a MEK inhibitor.
- **New Data Published in The Lancet Oncology Supports Potential of VS-6766.** An investigator-initiated Phase 1 study evaluating the intermittent dosing schedule of VS-6766 was published in the November issue of *The Lancet Oncology*. Tolerability and antitumor activity were observed across various cancers with RAS/RAF/MEK pathway mutations. The dose escalation study was the first to evaluate a dual RAF/MEK inhibitor using innovative intermittent dosing schedules in patients harboring RAS/RAF pathway mutations.
- **On Track to Commence Phase 2 Registration-Directed Trials in Lead Indications This Year.** Following a meeting with the U.S. Food and Drug Administration (FDA), Verastem reported that the FDA is supportive of its adaptive study design for the planned Phase 2 registration-directed trial evaluating VS-6766 and defactinib in patients with recurrent LGSOC. Verastem expects to commence registration-directed clinical trials in both recurrent LGSOC and KRAS mutant non-small cell lung cancer by the end of 2020. Assuming a positive outcome from these registration-directed trials, Verastem expects to submit New Drug Applications to the FDA requesting accelerated approval for VS-6766 alone or in combination with defactinib in both LGSOC and KRAS mutant NSCLC.
- **Closed COPIKTRA Sale to Secura Bio in a Deal Totaling \$311 Million, Plus Royalties.** Verastem recently announced the closing of a strategic transaction selling 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, provides Verastem 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 and defactinib in KRAS mutant solid tumors.
- **Presented New Preclinical Research Demonstrating Synergy and Tumor Regression with VS-6766 in Combination with G12C Inhibitors.** In a virtual poster presentation, also at the RTDD Summit, Verastem highlighted new preclinical research where VS-6766 showed synergy with KRAS-G12C inhibitors in reducing cancer cell viability across a panel of KRAS-G12C mutant NSCLC and colorectal cancer (CRC) cell lines. This enhanced cellular anti-cancer activity of the

combination correlated with deeper and more durable inhibition of ERK pathway signaling compared to G12C inhibition alone. The anti-tumor effects of VS-6766 were stronger than the effects of trametinib at a comparable dose.

Third Quarter 2020 Financial Results

Total Revenue for the three months ending September 30, 2020 (2020 Quarter) was \$78.6 million, compared to \$9.0 million for the three months ending September 30, 2019 (2019 Quarter).

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

License and collaboration revenue for the 2020 Quarter was \$2.8 million, compared to \$5.0 million for the 2019 Quarter. The 2019 Quarter included a \$5.0 million upfront payment received pursuant to a license and collaboration agreement executed between Verastem Oncology and Sanofi in July 2019. The 2020 Quarter was primarily comprised of \$2.5 million for Sanofi achieving two development milestones under the license and collaboration agreement.

Net product revenue for the 2020 Quarter was \$5.8 million, compared to \$4.0 million for the 2019 Quarter.

Cost of sales as a result of the sale of COPIKTRA license and related assets for the 2020 Quarter was \$31.2 million, compared to \$0.0 million for the 2019 Quarter. The 2020 Quarter comprised of the intangible asset, certain duvelisib inventory, net duvelisib contract prepaid balances and certain manufacturing equipment for the amounts of \$19.2 million, \$6.0 million, \$5.8 million, and \$0.2 million, respectively, delivered to Secura Bio Inc. as part of the COPIKTRA sale.

Total research and development (R&D) and selling, general and administrative (SG&A) expenses for the 2020 Quarter were \$31.6 million, compared to \$34.4 million for the 2019 Quarter.

R&D expense for the 2020 Quarter was \$11.0 million, compared to \$12.2 million for the 2019 Quarter. The decrease of \$1.2 million, or 10%, was primarily related to a decrease in contract research organization costs and lower employee related expense.

SG&A expense for the 2020 Quarter was \$20.6 million, compared to \$22.2 million for the 2019 Quarter. The decrease of \$1.6 million, or 7%, primarily resulted from the company's shift in strategic direction which led to lower commercial program and employee related expense. The 2020 Quarter includes \$3.5 million of nonrecurring transaction expenses directly attributable to the COPIKTRA sale to Secura Bio Inc.

Net income (loss) for the 2020 Quarter was \$13.1 million, or \$0.08 per share (basic and diluted), compared to \$(30.1) million, or \$(0.41) per share (basic and diluted), for the 2019 Quarter.

For the 2020 Quarter, non-GAAP adjusted net income was \$18.8 million, or \$0.11 per share (diluted), compared to non-GAAP adjusted net loss of \$26.2 million, or \$0.35 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 third quarter of 2020 with cash, cash equivalents and short-term investments of \$205.7 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 KRAS mutant 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 sale of the COPIKTRA franchise during the third quarter 2020 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, 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, KRASmt NSCLC and colorectal cancer (CRC). Updated interim data from this study presented at the 2nd Annual RAS-Targeted Drug Development Summit in September 2020 demonstrated a 56% overall response rate and long duration of therapy among patients with KRAS-G12 mt LGSOC. Based on an observation of higher response rates seen in NSCLC 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 was expanded in August 2020 to include new cohorts in pancreatic cancer, KRASmt endometrial cancer and KRAS-G12V NSCLC.

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, 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)

	September 30, December 31,	
	2020	2019
Cash, cash equivalents, & investments	\$ 170,470	\$ 75,506
Accounts receivable, net	5,685	2,524
Inventory	—	3,096
Restricted cash, prepaid expenses and other current assets	12,400	3,835
Property and equipment, net	497	947
Intangible assets, net	—	20,008
Right-of-use asset, net	2,820	3,077
Restricted cash and other assets	25,898	36,053
Total assets	\$ 217,770	\$ 145,046
Current Liabilities	\$ 37,678	\$ 29,890
Long-term debt	26,397	35,067
Convertible senior notes	20,841	68,556
Lease Liability, long-term	3,081	3,489
Other liabilities	—	870
Stockholders' equity	129,773	7,174
Total liabilities and stockholders' equity	\$ 217,770	\$ 145,046

Verastem, Inc.

Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

(unaudited)

Three months ended September 30, Nine months ended September 30,

2020	2019	2020	2019
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Revenue:

Product revenue, net	\$ 5,829	\$ 4,032	\$ 15,098	\$ 8,722
License and collaboration revenue	2,818	5,000	2,912	5,118
Sale of COPIKTRA license and related assets revenue	70,000	—	70,000	—
Total revenue	78,647	9,032	88,010	13,840

Operating expenses:

Cost of sales - product	866	371	1,753	906
Cost of sales - intangible amortization	8	392	793	1,177
Cost of sales – sale of COPIKTRA license and related assets	31,187	—	31,187	—
Research and development	10,955	12,219	31,223	33,322
Selling, general and administrative	20,614	22,153	55,660	77,484
Total operating expenses	63,630	35,135	120,616	112,889
Income (Loss) from operations	15,017	(26,103)	(32,606)	(99,049)
Other expense	—	—	(1,313)	—
Interest income	19	1,005	497	3,770
Interest expense	(1,898)	(5,041)	(14,440)	(15,156)
Net income (loss)	\$ 13,138	\$ (30,139)	\$ (47,862)	\$ (110,435)
Net income (loss) per share—basic	\$ 0.08	\$ (0.41)	\$ (0.32)	\$ (1.49)
Net income (loss) per share—diluted	\$ 0.08	\$ (0.41)	\$ (0.32)	\$ (1.49)

Weighted average common shares outstanding used in computing:

Net income (loss) per share – basic	169,510	74,228	147,766	73,988
Net income (loss) per share - diluted	169,760	74,228	147,766	73,988

Verastem, Inc.

Reconciliation of GAAP to Non-GAAP Financial Information

(in thousands, except per share amounts)

(unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2020	2019	2020	2019
Net income (loss) Reconciliation				
Net income (loss) (GAAP basis)	\$ 13,138	\$ (30,139)	\$ (47,862)	\$ (110,435)
Adjust:				
Amortization of acquired intangible asset	8	392	793	1,177
Stock-based compensation expense	2,156	1,915	5,185	7,228
Non-cash interest, net	506	1,611	9,765	4,426
Severance and Other	2,993	40	4,781	2,034
Change in fair value of derivative	—	—	1,313	—
Chugai license payment	—	—	3,000	—
Adjusted Net income (loss) (non-GAAP basis)	\$ 18,801	\$ (26,181)	\$ (23,025)	\$ (95,570)
Reconciliation of Net Loss Per Share				
Net income (loss) per share – diluted (GAAP Basis)	\$ 0.08	\$ (0.41)	\$ (0.32)	\$ (1.49)
Adjust per diluted share				
Amortization of acquired intangible asset	—	0.01	—	0.01
Stock-based compensation expense	0.01	0.03	0.03	0.10
Non-cash interest, net	—	0.02	0.07	0.06
Severance and Other	0.02	—	0.03	0.03
Change in fair value of derivative	—	—	0.01	—
Chugai license payment	—	—	0.02	—
Adjusted Net income(loss) per share – diluted (non-GAAP Basis)	\$ 0.11	\$ (0.35)	\$ (0.16)	\$ (1.29)
Weighted average common shares outstanding used in computing net loss per share—diluted	169,760	74,228	147,766	73,988

References

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.

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