Verastem Oncology Reports Third Quarter 2023 Financial Results and Highlights Recent Company Progress

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Plan to Submit Application for Accelerated Approval for Avutometinib and Defactinib in Recurrent Low-Grade Serous Ovarian Cancer (LGSOC) in H1 2024

Expects to Begin Enrollment in Phase 3 Confirmatory Trial, RAMP 301, of Avutometinib and Defactinib in LGSOC in Q4 2023

Presented Additional Patient Subgroup Data for the Combination of Avutometinib and Defactinib Showing Promising Levels of Response in LGSOC Regardless of Number and Class of Prior Therapies Including After Poor Response to Prior Therapy

Entered Into Synergistic Discovery and Development Collaboration with GenFleet Therapeutics to Advance New Programs Targeting RAS Pathway-Driven Cancers

BOSTON--(BUSINESS WIRE)--Nov. 8, 2023-- Verastem Oncology (Nasdaq: VSTM), a biopharmaceutical company committed to advancing new medicines for patients with cancer, today reported financial results for the third quarter ending September 30, 2023 and highlighted recent progress.

“In the third quarter, we presented additional data with robust levels of response from a planned subgroup analysis of the RAMP 201 study supporting the role of avutometinib and defactinib as a potential treatment option for LGSOC regardless of a patient’s prior therapy. These data continue to build on the foundational proof of concept and support our plans to submit an application for Accelerated Approval in the first half of 2024,” said Dan Paterson, President and Chief Executive Officer, Verastem Oncology. “As part of our broader development program, we were excited to share the initial, promising efficacy and safety data of the combination of avutometinib and sotorasib in G12C-mutant non-small cell lung cancer. In addition, we look forward to our synergistic collaboration with GenFleet Therapeutics that will provide us with the exclusive option to license three new programs to expand our pipeline. This collaboration along with our progress across our broader development platform, will allow us to further address the significant unmet medical needs across RAS pathway-driven cancers.”

Third Quarter 2023 and Recent Highlights

Low-Grade Serous Ovarian Cancer (LGSOC)

- The Company plans to submit an application for Accelerated Approval with the U.S. Food and Drug Administration (FDA) in the first half of 2024 for the combination of avutometinib and defactinib based on mature data from the Phase 2 registration-directed trial, RAMP 201, together with the results of the investigator-initiated FRAME trial. The Company also plans to have discussions with global regulatory authorities to bring the combination to additional regions.

- The Company finalized the design of the Phase 3 confirmatory trial (RAMP 301) of avutometinib and defactinib in LGSOC versus standard of care (SOC) chemotherapy (pegylated liposomal doxorubicin, paclitaxel, topotecan) or hormone therapy (letrozole, anastrozole). The trial will enroll approximately 270 patients randomized to either the combination of avutometinib and defactinib or SOC. RAMP 301 is an international collaboration between The GOG Foundation, Inc. (GOG) and the European Network of Gynaecological Oncological Trial groups (ENGOT) sponsored by Verastem Oncology. RAMP 301 is the follow-up confirmatory study being conducted for full regulatory approval in recurrent LGSOC and is expected to begin enrollment in the fourth quarter of this year.

- The results of a planned subgroup analysis of the Phase 2 RAMP 201 trial of avutometinib and defactinib were presented as a late-breaking abstract in an oral presentation at the Annual Global Meeting of the International Gynecologic Cancer Society (IGCS) in November. The data showed the combination demonstrated promising levels of response in recurrent LGSOC regardless of number and class of prior therapies including after poor response to prior therapy. In the combination arm, the observed overall response rate (ORR) was consistent across patients who received 1-3 (45.5%, 5/11, 95% CI 17-77) and ≥4 lines of therapy (44.4%, 8/18, 95% CI 22-69). Prior to enrollment in RAMP 201, only 2/23 (8.7%) patients responded to their last prior treatment in the recurrent setting, whereas the combination of avutometinib and defactinib yielded an ORR of 43.5% (10/23) in this subgroup. The safety profiles of avutometinib and defactinib were similar in the less and more heavily pretreated subgroups and both analyses were consistent with previously reported safety data.

- The Company, in collaboration with the LGSOC Patient Impact Advisory Committee, a global collaboration among leaders in the medical community as well as patient advocacy groups including STAAR Ovarian Cancer Foundation, Cure Our Ovarian Cancer and the World Ovarian Cancer Coalition, announced results of the first-ever LGSOC Patient Impact Survey. The focus of this survey is to better understand and address the particular challenges with diagnosis, disease management and mental, physical, and emotional well-being experienced by people living with LGSOC. More information is available LetsTalkAboutLGSOC.com.

Other Programs

- The Company announced a discovery and development collaboration with GenFleet Therapeutics ("GenFleet") to advance three oncology discovery programs targeting RAS pathway-driven cancers. The collaboration, which builds on the strengths of both companies in oncology small molecule drug development, enables Verastem Oncology to partner its clinical development and regulatory expertise with GenFleet's accomplished discovery capabilities. Verastem Oncology has the exclusive rights to obtain a license to each of the compounds after successful completion of pre-determined milestones in Phase 1 trials and adds to the Company's ability to become a leader in the treatment of RAS pathway-driven cancers.

- The Company presented initial safety, pharmacokinetics and recommended Phase 2 dose (RP2D) in the RAMP 203 trial evaluating the safety, tolerability and efficacy of avutometinib in combination with sotorasib in patients with KRAS G12C-mutant non-small cell lung cancer (NSCLC) at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics in October. The confirmed ORR was 25% (3/12) across efficacy-evaluable patients and seen in both KRAS G12C inhibitor resistant (14.3%; 1/7) and naïve (40%; 2/5) patients. Avutometinib 4.0 mg PO BIW 21/28 days + sotorasib 960 mg PO QD 28/28 days was selected as RP2D based on dose limiting toxicity assessment. Enrollment of patients with KRAS G12C-mutant NSCLC who are either naïve to or previously treated with a KRAS G12C inhibitor is ongoing in the expansion phase of RAMP 203.

- Dose escalation is ongoing in the RAMP 204 Phase 1/2 clinical trial of avutometinib with Mirati's KRAZATI® (adagrasib) in KRAS G12C-mutant NSCLC. The study is in its second dose cohort after successfully completing the first dose cohort.

- Enrollment is ongoing in the RAMP 205 Phase 1b/2 clinical trial evaluating avutometinib and defactinib in combination with SOC chemotherapy (GEMZAR® (gemcitabine) and ABRAXANE®) in patients with metastatic adenocarcinoma of the pancreas. The trial is supported by the Company's receipt of the first "Therapeutic Accelerator Award" from the Pancreatic Cancer Action Network (PanCAN).

Corporate Updates

- The Company strengthened its executive team in advance of the Company's potential commercial launch of avutometinib in combination with defactinib in LGSOC and to support the advancement of its broader development programs in RAS pathway-driven cancers. The appointments include Mike Crowther to Chief Commercial and Business Strategy Officer, David Mitchell to Senior Vice President, Head of Regulatory Affairs and the promotion of Dan Calkins to Chief Financial Officer from Vice President of Finance.

Third Quarter 2023 Financial Results

Verastem Oncology ended the third quarter of 2023 with cash, cash equivalents and investments of $165.7 million. Total operating expenses for the three months ended September 30, 2023 (the "2023 Quarter") were $21.3 million, compared to $17.7 million for the three months ended September 30, 2022 (the "2022 Quarter").

Research & development expenses for the 2023 Quarter were $13.9 million, compared to $11.3 million for the 2022 Quarter. The increase of $2.6 million, or 23.0%, primarily resulted from a $2.0 million upfront payment made to GenFleet pursuant to the discovery and development collaboration agreement and increases in contract research organization costs.

Selling, general & administrative expenses for the 2023 Quarter were $7.4 million, compared to $6.4 million for the 2022 Quarter. The increase of $1.0 million, or 15.6%, was primarily related to increased personnel costs, including non-cash stock compensation, additional costs in anticipation of a potential launch of avutometinib and defactinib in LGSOC, and increased travel and other costs.

Net loss for the 2023 Quarter was $20.0 million, or $0.75 per share (basic and diluted), compared to net loss of $18.1 million, or $1.10 per share (basic and diluted, each as adjusted for the Company's reverse stock split) for the 2022 Quarter.

For the 2023 Quarter, non-GAAP adjusted net loss was $19.0 million, or $0.71 per share (diluted), compared to non-GAAP adjusted net loss of $16.6 million, or $1.01 per share (diluted, as adjusted for the Company's reverse stock split) for the 2022 Quarter. Please refer to the GAAP to Non-GAAP Reconciliation accompanying this press release.

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 adjusted 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 and nine months ended September 30, 2023, and 2022 are included in the tables accompanying this press release, after the unaudited condensed consolidated financial statements.

About Avutometinib (VS-6766)

Avutometinib is a RAF/MEK clamp that induces inactive complexes of MEK with ARAF, BRAF and CRAF potentially creating a more complete and durable anti-tumor response through maximal RAS pathway inhibition. Avutometinib is currently in late-stage development.

In contrast to other MEK inhibitors, avutometinib blocks both MEK kinase activity and the ability of RAF to phosphorylate MEK. This unique mechanism allows avutometinib to block MEK signaling without the compensatory activation of MEK that appears to limit the efficacy of other inhibitors. The U.S. Food and Drug Administration granted Breakthrough Therapy designation for the combination of Verastem Oncology's investigational RAF/MEK clamp avutometinib, with defactinib, its FAK inhibitor, for the treatment of all patients with recurrent low-grade serous ovarian cancer (LGSOC) regardless of KRAS status after one or more prior lines of therapy, including platinum-based chemotherapy.

Verastem Oncology is currently conducting clinical trials with its RAF/MEK clamp avutometinib in RAS- driven tumors as part of its (**R**af **A**nd **M**ek **P**rogram). RAMP 201 is a registration-directed trial of avutometinib in combination with defactinib in patients with recurrent LGSOC. Verastem Oncology has established clinical collaborations with Amgen and Mirati to evaluate LUMAKRAS® (sotorasib) and KRAZATI® (adagrasib) in combination with avutometinib in KRAS G12C mutant NSCLC as part of the RAMP 203 and RAMP 204 trials, respectively. As part of the "Therapeutic Accelerator Award" Verastem Oncology received from PanCAN, Verastem Oncology is conducting RAMP 205, a Phase 1b/2 clinical trial evaluating avutometinib and defactinib with gemcitabine/nab-paclitaxel in patients with front-line metastatic pancreatic cancer.

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 its financial condition, the potential clinical value of various of its clinical trials, the timing of commencing and completing trials, including topline data reports, the potential commercial launch of avutometinib in combination with defactinib in LGSOC, the potential benefits of the collaboration with Genfleet and interactions with regulators. 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 avutometinib in combination with other compounds, including defactinib, LUMAKRAS® and others; 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 trial design, labeling and other matters that could affect the timing, 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 we may not attract and retain high quality personnel; 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, including as a result of conducting additional studies; that our target market for our product candidates might be smaller than we are presently estimating; that we or Chugai Pharmaceutical Co., Ltd. will fail to fully perform under the avutometinib license agreement; that we will not see a return on investment on the payments we have and may continue to make pursuant to the collaboration and option agreement with Genfleet or that Genfleet will fail to fully perform under the agreement; that we or our other collaboration partners may fail to perform under our collaboration agreements; that any of our third party contract research organizations, contract manufacturing organizations, clinical sites, or contractors, among others, who we rely on fail to fully perform; 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 Secura will achieve the milestones that result in payments to us under our asset purchase agreement with Secura; that we will be unable to execute on our partnering strategies for avutometinib in combination with other compounds; 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 Verastem Oncology's Annual Report on Form 10-K for the year ended December 31, 2022 as filed with the Securities and Exchange Commission (SEC) on March 14, 2023 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 Verastem Oncology 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.

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Condensed Consolidated Balance Sheets

(in thousands)

(unaudited)

	September 30, 2023	December 31, 2022
Cash, cash equivalents, & investments	$ 165,663	$ 87,894
Accounts receivable, net	—	31
Prepaid expenses and other current assets	8,822	4,945
Property and equipment, net	35	92
Right-of-use asset, net	1,336	1,789
Restricted cash and other assets	297	299
Total assets	**$ 176,153**	**$ 95,050**
Current Liabilities	$ 23,812	$ 21,663
Long term debt	39,911	24,526
Lease liability, long-term	780	1,470
Other long-term liabilities	51	—
Preferred stock tranche liability	7,260	—
Convertible preferred stock	21,159	—
Stockholders' equity	83,180	47,391
Total liabilities, convertible preferred stock and stockholders' equity	**$ 176,153**	**$ 95,050**

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Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

(unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2023	**2022**	**2023**	**2022**
Revenue:				

Sale of COPIKTRA license and related assets revenue	$ —	$ —	$ —	$ 2,596
Total revenue	—	—	—	2,596
Operating expenses:				
Research and development	13,946	11,288	38,854	39,818
Selling, general and administrative	7,363	6,421	22,091	18,869
Total operating expenses	21,309	17,709	60,945	58,687
Loss from operations	(21,309)	(17,709)	(60,945)	(56,091)
Other income (expense)	(13)	20	(60)	54
Interest income	2,247	316	4,345	446
Interest expense	(1,129)	(717)	(3,019)	(1,413)
Change in fair value of preferred stock tranche liability	200	—	(320)	—
Net loss	$ (20,004)	$ (18,090)	$ (59,999)	$ (57,004)
Net loss per share—basic and diluted	$ (0.75)	$ (1.10)[1]	$ (2.93)[1]	$ (3.60)[1]
Weighted average common shares outstanding used in computing:				
Net loss per share – basic and diluted	26,790	16,430 [1]	20,452 [1]	15,834 [1]

(1) Amounts have been retroactively restated to reflect the 1-for-12 reverse stock split effected on May 31, 2023

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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,	
	2023	**2022**	**2023**	**2022**
Net loss reconciliation				
Net loss (GAAP basis)	$ (20,004)	$ (18,090)	$ (59,999)	$ (57,004)
Adjust:				
Stock-based compensation expense	1,517	1,356	4,262	4,760

Non-cash interest, net	(371)	120	(295)	231
Change in fair value of preferred stock tranche liability	(200)	—	320	—
Severance and Other	47	—	85	—
Adjusted net loss (non-GAAP basis)	$ (19,011)	$ (16,614)	$ (55,627)	$ (52,013)
Reconciliation of net loss per share				
Net loss per share – diluted (GAAP Basis)	$ (0.75)	$ (1.10)[1]	$ (2.93)[1]	$ (3.60)[1]
Adjust per diluted share:				
Stock-based compensation expense	0.06	0.08 [1]	0.21 [1]	0.31 [1]
Non-cash interest, net	(0.01)	0.01 [1]	(0.02)[1]	0.01 [1]
Change in fair value of preferred stock tranche liability	(0.01)	—	0.02 [1]	—
Severance and Other	—	—	— [1]	—
Adjusted net loss per share – diluted (non-GAAP basis)	$ (0.71)	$ (1.01)[1]	$ (2.72)[1]	$ (3.28)[1]
Weighted average common shares outstanding used in computing net loss per share—diluted	26,790	16,430 [1]	20,452 [1]	15,834 [1]

(1) Amounts have been retroactively restated to reflect the 1-for-12 reverse stock split effected on May 31, 2023

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