



Verastem Oncology Reports Second Quarter 2024 Financial Results and Highlights Recent Business Updates

August 8, 2024 at 4:05 PM EDT

Initiated rolling NDA submission for avutometinib and defactinib combination in recurrent low-grade serous ovarian cancer in Q2 2024 with plans to complete the submission with mature data from all patients in RAMP 201 trial in H2 2024

Presented positive initial interim safety and efficacy results from the RAMP 205 trial of avutometinib and defactinib in combination with current standard of care in first-line metastatic pancreatic cancer at the 2024 ASCO Annual Meeting

Company cash, cash equivalents, and investments of \$83.4 million as of June 30, 2024; pro forma \$144.5 million including the sale of common stock, pre-funded warrants, and warrants, and achievement of COPIKTRA net sales milestone by Secura

BOSTON--(BUSINESS WIRE)--Aug. 8, 2024-- Verastem Oncology (Nasdaq: VSTM), a biopharmaceutical company committed to advancing new medicines for patients with cancer, today announced business updates and reported financial results for the second quarter ended June 30, 2024.

"We have made tremendous progress in the second quarter as we initiated our rolling NDA submission for avutometinib and defactinib combination in recurrent KRAS mutant low-grade serous ovarian cancer, shared updated topline results from our RAMP 201 trial with a minimum of five months of follow-up, and presented positive initial interim results from our ongoing first-line metastatic pancreatic cancer trial," said Dan Paterson, president and chief executive officer of Verastem Oncology. "In the second half of the year, we plan to present mature data from RAMP 201, complete our rolling NDA submission with the mature data from RAMP 201 trial, and share interim topline data from our lung cancer programs."

Second Quarter 2024 and Recent Updates

Avutometinib and Defactinib Combination in Low-Grade Serous Ovarian Cancer (LGSOC)

- Announced the initiation of a rolling New Drug Application (NDA) submission seeking accelerated approval of the combination of avutometinib and defactinib for adult patients with recurrent KRAS mutant LGSOC who received at least one prior systemic therapy. Read the press release [here](#).
- Announced topline interim data from the RAMP 201 trial, with a February 2024 data cutoff, that continued to show robust overall response rates and durable responses with low discontinuation rates due to adverse events in patients with KRAS mutant or KRAS wild-type LGSOC who had a minimum follow-up of five months, in May 2024.
- Expect to complete the rolling NDA submission after obtaining and submitting to the FDA the mature safety and efficacy data from the RAMP 201 trial, including 12 months of follow-up, to inform the final indication in the second half of 2024.
- Enrollment and site activations are underway in the U.S., Australia, Europe, and the UK, for the international confirmatory Phase 3 RAMP 301 trial evaluating the avutometinib and defactinib combination versus standard of care chemotherapy or hormonal therapy for the treatment of recurrent LGSOC.
- Preparations for a potential U.S. commercial launch in 2025 are ongoing and plans to initiate discussions with European and Japanese regulatory authorities to address patient needs outside the U.S. continue to advance.

Avutometinib in Combination with KRAS G12C Inhibitors in Non-Small Cell Lung Cancer (NSCLC)

- In the RAMP 203 trial, patients who previously progressed on KRAS G12C inhibitors were initiated on the triplet combination of avutometinib, defactinib and sotorasib.
- Expect to report updated interim data from patients with KRAS G12C-mutant NSCLC in the Phase 1/2 RAMP 203 trial evaluating avutometinib plus defactinib and sotorasib are planned for H2 2024.
- Expect to report initial interim data from patients with KRAS G12C-mutant NSCLC in the Phase 1/2 RAMP 204 trial evaluating avutometinib and adagrasib are planned for H2 2024.

Avutometinib and Defactinib Combination in First-Line Metastatic Pancreatic Cancer

- Presented initial interim safety and efficacy results from the ongoing RAMP 205 trial of avutometinib and defactinib in combination with current standard of care gemcitabine and nab-paclitaxel in first-line metastatic pancreatic cancer on June 1, 2024, at the American Society of Clinical Oncology (ASCO) Annual Meeting. As of May 14, 2024, 41 patients had been treated in one of four dose cohort regimens and only patients in dose cohort 1 had a minimum follow up of six months. In the dose level 1 cohort, 83% (5/6) of patients achieved a confirmed partial response with more than six months of follow up at the time of data cutoff. One dose-limiting toxicity (DLT) was observed in the dose level 1 cohort, and the dose level

was subsequently cleared after additional patients were enrolled. Of the 26 patients in all cohorts who have had the opportunity to have their first scan while on treatment, 21 have experienced a reduction of the change in target lesion sum of diameters. Read the press release [here](#).

- FDA granted Orphan Drug Designation to avutometinib and defactinib combination for the treatment of pancreatic cancer in July 2024.
- Expect to report updated data from the ongoing RAMP 205 trial in Q1 2025.

GFH375 (VS-7375): Oral KRAS G12D (ON/OFF) Inhibitor

- GenFleet Therapeutics investigational new drug (IND) application for GFH375 (VS-7375) in China was cleared in June 2024.
- GenFleet dosed the first patient in the Phase 1/2 trial for GFH375 (VS-7375) in China in patients with KRAS G12D-mutated advanced solid tumors in July 2024.
- Plan to initiate development studies outside of China after evaluating initial dose escalation data from the Phase 1 study of GFH375 (VS-7375) in China to accelerate a path forward in the U.S. and rest of world.
- Discovery/lead optimization continues for second and third programs with GenFleet collaboration.

Corporate Updates

- Verastem strengthened its executive team with the appointment of John Hayslip, M.D., as chief medical officer in April 2024 and the promotion of Nate Sanburn to chief business officer in June 2024.
- In July 2024, Verastem strengthened its balance sheet by raising net proceeds of approximately \$51.1 million in a public offering of 13.3 million shares of its common stock and accompanying warrants to purchase up to 13.3 million shares of its common stock, and pre-funded warrants to purchase up to 5.0 million shares of its common and accompany warrants to purchase up to 5.0 million shares of its common stock.
- Secura Bio, Inc. ("Secura") achieved \$100 million of cumulative worldwide net sales of COPIKTRA during Q2 2024, entitling Verastem to a \$10 million milestone payment. Verastem received the \$10 million milestone payment in July 2024.

Second Quarter 2024 Financial Results

Verastem Oncology ended the second quarter of 2024 with cash, cash equivalents and investments of \$83.4 million. On a pro forma basis, inclusive of the \$51.1 million net proceeds raised through issuance of common stock, pre-funded warrants, and warrants in July 2024, and the \$10 million net sales milestone from Secura, cash, cash equivalents and investments were \$144.5 million as of June 30, 2024. These additional sources of capital along with the existing cash, cash equivalents, and investments through the second quarter of 2024 provides an expected cash runway through the potential approval of avutometinib and defactinib for recurrent LGSOC in the first half of 2025.

Total revenue for the three months ended June 30, 2024 (the "2024 Quarter") was \$10.0 million, compared to \$0.0 million for the three months ended June 30, 2023 (the "2023 Quarter"). Revenue for the 2024 Quarter was comprised of one sales milestone payment of \$10.0 million due upon Secura achieving total worldwide net sales of COPIKTRA exceeding \$100.0 million during the 2024 Quarter. The \$10.0 million milestone payment was received by Verastem in July 2024.

Total operating expenses for the 2024 Quarter were \$28.3 million, compared to \$20.3 million for the three months ended June 30, 2023.

Research & development expenses for the 2024 Quarter were \$18.1 million, compared to \$12.9 million for the 2023 Quarter. The increase of \$5.2 million, or 40.3%, was primarily related to increased contract research organization costs, increased consulting costs, and increased investigator fees.

Selling, general & administrative expenses for the 2024 Quarter were \$10.2 million, compared to \$7.4 million for the 2023 Quarter. The increase of \$2.8 million, or 37.8%, was primarily related to additional costs in anticipation of a potential launch of avutometinib and defactinib in LGSOC, and increased personnel costs, including non-cash stock compensation.

Net loss for the 2024 Quarter was \$8.3 million, or \$0.31 per share (basic and diluted), compared to \$24.3 million, or \$1.37 per share (basic and diluted), each as adjusted for the Company's reverse stock split) for the 2023 Quarter.

For the 2024 Quarter, non-GAAP adjusted net loss was \$16.5 million, or \$0.61 per share (diluted) compared to non-GAAP adjusted net loss of \$18.8 million, or \$1.06 per share (diluted, as adjusted for the Company's reverse stock split), for the 2023 Quarter. Please refer to the GAAP to non-GAAP Reconciliation attached to 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 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 six months ended June 30, 2024 and 2023 are included in the tables accompanying this press release after the unaudited condensed consolidated financial statements.

About the Avutometinib and Defactinib Combination

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/MAPK pathway inhibition. In contrast to currently available MEK-only 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 MEK-only inhibitors. The U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy Designation of the investigational combination of avutometinib and defactinib, a selective 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. Avutometinib alone or in combination with defactinib was also granted Orphan Drug Designation by the FDA for the treatment of LGSOC.

Verastem Oncology is currently conducting clinical trials with avutometinib in RAS/MAPK driven tumors as part of its **Raf And Mek Program** or RAMP. RAMP 301 (NCT06072781) is an international Phase 3 confirmatory trial evaluating the combination of avutometinib and defactinib versus standard chemotherapy or hormonal therapy for the treatment of recurrent LGSOC. RAMP 201 (NCT04625270) is a Phase 2 registration-directed trial of avutometinib in combination with defactinib in patients with recurrent LGSOC and enrollment has been completed in each of the dose optimization and expansion phases and the low-dose evaluation. Verastem has initiated a rolling NDA submission for avutometinib and defactinib combination in adults with recurrent LGSOC and expects to complete its NDA submission in the second half of 2024 with a potential FDA decision in the first half of 2025.

Verastem Oncology has established clinical collaborations with Amgen and Mirati to evaluate LUMAKRAS™ (sotorasib) in combination with avutometinib and defactinib and KRAZATI™ (adagrasib) in combination with avutometinib in KRAS G12C mutant NSCLC as part of the RAMP 203 (NCT05074810) and RAMP 204 (NCT05375994) trials, respectively. The RAMP 205 (NCT05669482), a Phase 1b/2 clinical trial evaluating avutometinib and defactinib with gemcitabine/nab-paclitaxel in patients with front-line metastatic pancreatic cancer, is supported by the PanCAN Therapeutic Accelerator Award. FDA granted Orphan Drug Designation to avutometinib and defactinib combination for the treatment of pancreatic cancer.

About GFH375 (VS-7375)

GFH375/VS-7375 is a potential best-in-class, potent and selective oral KRAS G12D (ON/OFF) inhibitor, identified as the lead discovery program from the Verastem Oncology discovery and development collaboration with GenFleet Therapeutics. GenFleet's IND for GFH375/VS-7375 was approved in China in June 2024 and the Phase 1/2 trial in KRAS G12D-mutant solid tumors was subsequently initiated and the first patient has been dosed in July 2024. The collaboration includes three discovery programs, the first being the KRAS G12D inhibitor, and provides Verastem Oncology with exclusive options to license three compounds selected for collaboration after successful completion of pre-determined milestones in Phase 1 trials. The licenses would give Verastem Oncology development and commercialization rights outside of the GenFleet territories of mainland China, Hong Kong, Macau, and Taiwan.

About Verastem Oncology

Verastem Oncology (Nasdaq: VSTM) is a late-stage development 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 RAS/MAPK-driven cancers, specifically novel small molecule drugs that inhibit critical signaling pathways in cancer that promote cancer cell survival and tumor growth, including RAF/MEK inhibition and FAK inhibition. For more information, please visit www.verastem.com and follow us on [LinkedIn](https://www.linkedin.com/company/verastem).

Forward-Looking Statements

This press release includes forward-looking statements about, among other things, Verastem Oncology's programs and product candidates, strategy, future plans and prospects, including statements related to the timing, scope and progress of the rolling NDA submission for the avutometinib and defactinib combination in LGSOC, the structure of our planned and pending clinical trials, the potential clinical value of various of the Company's clinical trials, including the RAMP 201, 205 and 301 trials, the timing of commencing and completing trials, including topline data reports, interactions with regulators, the timeline and indications for clinical development, regulatory submissions and the potential for and timing of commercialization of product candidates and potential for additional development programs involving Verastem Oncology's lead compound, the expected outcome and benefits of our collaboration with GenFleet Therapeutics and the estimated addressable markets of our drug candidates. 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.

Forward-looking statements are not guarantees of future performance and are subject to risks and uncertainties that could cause our actual results to differ materially from those expressed or implied in the forward-looking statements we make. 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 uncertainties inherent in research and development, such as negative or unexpected results of clinical trials, the occurrence or timing of applications for our product candidates that may be filed with regulatory authorities in any jurisdictions; whether and when regulatory authorities in any jurisdictions may approve any such applications that may be filed for our product candidates, and, if approved, whether our product candidates will be commercially successful in such jurisdictions; 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 the market opportunities of our drug candidates are based on internal and third-party estimates which may prove to be incorrect; 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, which may delay our development programs, including delays in submission or review by the FDA of our NDA submission in recurrent KRAS mutant LGSOC if enrollment in our

confirmatory trial is not well underway at the time of submission, or that the FDA may require the Company to enroll additional patients in the Company's ongoing RAMP-301 confirmatory Phase 3 clinical trial prior to Verastem submitting or the FDA taking action on our NDA seeking accelerated approval; risks associated with preliminary and interim data, which may not be representative of more mature data, including with respect to interim duration of therapy data; that our product candidates will cause adverse safety events and/or unexpected concerns may arise from additional data or analysis, or result in unmanageable safety profiles as compared to their levels of efficacy; that we may be unable to successfully validate, develop and obtain regulatory approval for companion diagnostic tests for our product candidates that require or would commercially benefit from such tests, or experience significant delays in doing so; that the mature RAMP 201 data and associated discussions with the FDA may not support the scope of our rolling NDA submission for the avutometinib and defactinib combination in LGSOC, including with respect to KRAS wild type LGSOC; that our product candidates may experience manufacturing or supply interruptions or failures; 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 face substantial competition, which may result in others developing or commercializing products before or more successfully than we do which could result in reduced market share or market potential for our product candidates; 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 or our decisions regarding execution of such commercialization; that we may not have sufficient cash to fund our contemplated operations, including certain of our product development programs; that we may not attract and retain high quality personnel; that we or Chugai Pharmaceutical Co., Ltd. will fail to fully perform under the avutometinib license agreement; that the total addressable and target markets for our product candidates might be smaller than we are presently estimating; that we or Secura Bio, Inc. (Secura) will fail to fully perform under the asset purchase agreement with Secura, including in relation to milestone payments; 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 Therapeutics (Shanghai), Inc. (GenFleet), or that GenFleet will fail to fully perform under the agreement; 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 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, 2023 as filed with the Securities and Exchange Commission (SEC) on March 14, 2024 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 Oncology

Condensed Consolidated Balance Sheets

(in thousands)

(unaudited)

	June 30, 2024	December 31, 2023
Cash, cash equivalents, & investments	\$ 83,371	\$ 137,129
Accounts receivable, net	10,000	—
Grant receivable	825	—
Prepaid expenses and other current assets	5,450	6,553
Property and equipment, net	46	37
Right-of-use asset, net	816	1,171
Restricted cash and other assets	5,239	4,828
Total assets	\$ 105,747	\$ 149,718
Current Liabilities	\$ 30,347	\$ 26,380

Long term debt	35,390	40,086
Lease liability, long-term	—	530
Preferred stock tranche liability	—	4,189
Convertible preferred stock	21,159	21,159
Stockholders' equity	18,851	57,374
Total liabilities, convertible preferred stock and stockholders' equity	\$ 105,747	\$ 149,718

Verastem Oncology

Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

(unaudited)

	Three months ended June 30,		Six months ended June 30,	
	2024	2023	2024	2023
Revenue				
Sale of COPIKTRA license and related assets	\$ 10,000	\$ —	\$ 10,000	\$ —
Total revenue	10,000	—	10,000	—
Operating expenses:				
Research and development	18,062	12,893	35,769	24,908
Selling, general and administrative	10,215	7,399	20,567	14,728
Total operating expenses	28,277	20,292	56,336	39,636
Loss from operations	(18,277)	(20,292)	(46,336)	(39,636)
Other expense	(24)	(40)	(54)	(47)
Interest income	983	1,122	2,350	2,098
Interest expense	(1,138)	(1,121)	(2,268)	(1,890)
Change in fair value of preferred stock tranche liability	10,200	(3,950)	4,189	(520)
Net loss	\$ (8,256)	\$ (24,281)	\$ (42,119)	\$ (39,995)
Net loss per share—basic and diluted	\$ (0.31)	\$ (1.37) ⁽¹⁾	\$ (1.57)	\$ (2.32) ⁽¹⁾

Weighted average common shares outstanding used in computing:

Net loss per share – basic and diluted	26,861	17,732 ⁽¹⁾)	26,846	17,231 ⁽¹⁾)
--	--------	-------------------------	--------	-------------------------

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

Verastem Oncology

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,	
	2024	2023	2024	2023
Net loss reconciliation				
Net loss (GAAP basis)	\$ (8,256)	\$ (24,281)	\$ (42,119)	\$ (39,995)
Adjust:				
Stock-based compensation expense	1,905	1,432	3,388	2,745
Non-cash interest, net	6	112	(413)	76
Change in fair value of preferred stock tranche liability	(10,200)	3,950	(4,189)	520
Severance and Other	56	—	609	38
Adjusted net loss (non-GAAP basis)	\$ (16,489)	\$ (18,787)	\$ (42,724)	\$ (36,616)
Reconciliation of net loss per share				
Net loss per share – diluted (GAAP Basis)	(0.31)	(1.37) ⁽¹⁾)	(1.57)	(2.32) ⁽¹⁾)
Adjust per diluted share:				
Stock-based compensation expense	0.08	0.08 ⁽¹⁾)	0.13	0.16 ⁽¹⁾)
Non-cash interest, net	—	0.01 ⁽¹⁾)	(0.01)	—
Change in fair value of preferred stock tranche liability	(0.38)	0.22 ⁽¹⁾)	(0.16)	0.03 ⁽¹⁾)
Severance and Other	—	—	0.02	—
Adjusted net loss per share – diluted (non-GAAP basis)	\$ (0.61)	\$ (1.06)⁽¹⁾)	\$ (1.59)	\$ (2.13)⁽¹⁾)

Weighted average common shares outstanding used in computing net loss per share—diluted	26,861	17,732 ⁽¹⁾)	26,846	17,231 ⁽¹⁾)
---	--------	-----------------------	---	--------	-----------------------	---

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

View source version on [businesswire.com](https://www.businesswire.com/news/home/20240808951114/en/): <https://www.businesswire.com/news/home/20240808951114/en/>

For Investor and Media Inquiries:

Julissa Viana

Vice President, Corporate Communications and Investor Relations

investors@verastem.com or

media@verastem.com

Source: Verastem Oncology