Verastem Oncology Reports Fourth Quarter and Full Year 2024 Financial Results and Highlights Recent Business Updates

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Avutometinib plus defactinib granted priority review by FDA in December 2024, under the accelerated approval pathway, for KRAS mutant recurrent LGSOC; PDUFA action date set for June 30, 2025

Filed an investigational new drug application in the U.S. for VS-7375, an oral KRAS G12D (ON/OFF) inhibitor

RAMP 205 trial in 1L metastatic pancreatic cancer continues to progress with an additional dose cohort added and enrollment across all dose-level cohorts on track to complete in Q1

Company cash, cash equivalents, and investments of $88.8 million as of December 31, 2024; pro forma $151.3 million including debt refinancing and equity issuance with Oberland, and equity issuance under at-the-market facility

BOSTON--(BUSINESS WIRE)--Mar. 20, 2025-- Verastem Oncology (Nasdaq: VSTM), a biopharmaceutical company committed to advancing new medicines for patients with cancer, today reported financial results for the three months and full year ended December 31, 2024, and highlighted recent progress.

“In 2024, we made tremendous progress across our pipeline programs, most notably the NDA acceptance of our novel-novel combination of avutometinib plus defactinib for Priority Review under the accelerated approval pathway for KRAS mutant recurrent low-grade serous ovarian cancer,” said Dan Paterson, president and chief executive officer of Verastem Oncology. “2025 is expected to be a transformational year with our potential to launch the first FDA-approved treatment specifically for KRAS mutant recurrent low-grade serous ovarian cancer and become a fully integrated commercial-stage company. In addition, we anticipate advancing our pipeline programs in pancreatic cancer and non-small cell lung cancer and expect to initiate a Phase 1/2a study for VS-7375, our recently licensed KRAS G12D (ON/OFF) inhibitor.”

Fourth Quarter 2024 and Recent Highlights

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

- On December 30, 2024, the U.S. Food and Drug Administration (FDA) accepted the Company’s New Drug Application (NDA) under the accelerated approval pathway and granted Priority Review for avutometinib in combination with defactinib in adult patients with KRAS mutant recurrent LGSOC and designated June 30, 2025, as the Prescription Drug User Fee Act (PDUFA) action date.
- The NDA was based on the positive, mature safety and efficacy data from the RAMP 201 trial as presented at the International Gynecologic Cancer Society (IGCS) 2024 Annual Meeting in October 2024. The NDA also includes supportive data from the FRAME Phase 1 trial, the first study conducted with the combination therapy in recurrent LGSOC.
- The Company continued its commercial preparation activities for a potential U.S. launch in mid-2025.
- Presented the RAMP 201 primary analysis with additional subgroup analysis by KRAS mutational status at the Society of Gynecologic Oncology 2025 Annual Meeting on Women’s Cancer on March 17, 2025. The subgroup analysis showed clinically meaningful responses were observed in patients with and without prior MEK inhibitor treatment, with and without prior bevacizumab treatment, as well as patients receiving multiple lines of therapy (1-3 and >3 prior lines).
- RAMP 301, which is currently enrolling patients with recurrent LGSOC regardless of KRAS mutation status across the U.S., UK, EU, Canada, Korea, and Australia, will serve as a confirmatory study for the initial indication and has potential to expand the indication regardless of KRAS mutation status. The Company plans to complete enrollment in RAMP 301 by the end of 2025.
- The Japanese Gynecologic Oncology Group (JGOG) dosed the first patient in a Phase 2 Verastem sponsored clinical trial, called RAMP201J, evaluating the safety and efficacy of avutometinib in combination with defactinib for recurrent LGSOC in Japan in October 2024.

Key Milestones Expected for 2025:

- Plan for FDA decision on NDA submitted for the combination of avutometinib plus defactinib in KRAS mutant recurrent LGSOC, expected by June 30, 2025.
- Plan to submit for NCCN guideline inclusion upon FDA approval.
- Primary analysis from both the FRAME and RAMP 201 clinical trials anticipated to be published in H1 2025.
- Complete enrollment for the international Phase 3 confirmatory RAMP 301 clinical trial for patients with recurrent LGSOC

regardless of KRAS mutation status by the end of 2025.
- Report initial data from the RAMP 201J Phase 2 clinical trial being conducted in Japan with JGOG in H2 2025.
- Continue to advance the regulatory pathway in Japan and Europe.

RAMP 205: Avutometinib Plus Defactinib in Combination with Chemotherapy in First-Line Metastatic Pancreatic Cancer

- Today, Verastem announced an interim update on RAMP 205:
  - A new dose level "0" was added to evaluate the doses of avutometinib and defactinib used in LGSOC, 3.2 mg of avutometinib, 200 mg of defactinib, in combination with 800 mg/m^2 of gemcitabine and 100 mg/m^2 of Nab-paclitaxel on a schedule of day 1, 8, and 15.
  - All dose levels have been expanded to 12 patients each, including six additional patients recently enrolled to dose level 1, where 5/6 patients reported an objective response at the ASCO 2024 annual meeting. In dose level 1, of the six additional patients, 5 remain on therapy and continue to be monitored for response given the initial length of time to respond.
  - 59 of 60 patients have been treated and enrollment is on track to be completed in Q1.
  - Based on the initial safety and efficacy data from these cohorts, dose level 1 or 0 is anticipated to be chosen for expansion.
  - Adverse events across all dose cohorts remained generally consistent with the previously announced safety and tolerability profile, and no new safety signals have emerged.

Key Milestones Expected for 2025:

- Plan to present additional data at a medical meeting mid-year 2025.
- Select the recommended Phase 2 Dose (RP2D) for trial expansion in H1 2025.

RAMP 203: Avutometinib Plus Defactinib in Combination with a KRAS G12C Inhibitor in Non-Small Cell Lung Cancer (NSCLC)

- Enrollment to the KRAS G12C inhibitor, prior-treated Stage 1 Part B doublet cohort on track to complete in Q1 2025. Patients enrolled in the doublet cohorts continue to be followed for safety and efficacy results (both the prior-treated and treatment-naïve cohorts).
- Enrollment in the triplet combination continues in the dose evaluation cohort.
- In December 2024, the Company announced preliminary clinical data for the triplet combination cohort of avutometinib and LUMAKRAS™ (sotorasib) plus defactinib in the RAMP 203 Phase 1/2 study in KRAS G12C mutant advanced NSCLC. No dose-limiting toxicities (DLTs) have been observed in the triplet combination.

Key Milestones Expected for 2025:

- Present an interim update of both doublet and triplet data at a medical meeting in H2 2025.

VS-7375, an Oral KRAS G12D (ON/OFF) Inhibitor, in Advanced Solid Tumors

- Verastem filed an investigational new drug (IND) application in the U.S. for VS-7375 in the first quarter of 2025.
- Verastem announced on January 14, 2025, that it has exercised its option early to license GFH375 (VS-7375) from GenFleet. In addition, the Company announced preliminary clinical data from the Phase 1 dose-escalation study conducted by GenFleet in China. In the study, VS-7375, demonstrated oral bioavailability, no DLTs across six dose levels, and several partial responses, including multiple patients with pancreatic and lung cancers. Enrollment in the Phase 1 dose-escalation cohort is ongoing.

Key Milestones Expected for 2025:

- Initiate a Phase 1/2a trial in the U.S. by mid-2025.
- Share preclinical and clinical data from the Phase 1 study of VS-7375 in China in H1 2025.

Corporate Updates

- Strengthened the executive leadership team with the appointment of Matthew E. Ros to Chief Operating Officer on January 15, 2025.
- Verastem announced on January 14, 2025, that it has exercised its option early to license VS-7375 from GenFleet.
- Verastem announced on January 13, 2025, agreements with Oberland Capital and IQVIA. The agreements with Oberland Capital include a debt refinancing and an equity investment, strengthening the Company's cash position and will help fund commercialization past FDA approval and other pipeline programs. The strategic collaboration with IQVIA leverages IQVIA's world-class infrastructure and commercialization solutions to complement the Company's launch strategy in recurrent LGSOC.

Fourth Quarter 2024 Financial Results

Verastem Oncology ended the fourth quarter of 2024 with cash, cash equivalents and investments of $88.8 million. On a pro forma basis, taking into account the initial $75.0 million of notes and $7.5 million of equity to be purchased by Oberland Capital at closing, repayment of amounts owed under the Company's existing loan with Oxford Finance of $42.7 million, and net proceeds from equity issuance under the Company's at-the-market facility in January 2025 of $22.7 million, cash, cash equivalents and investments were $151.3 million as of December 31, 2024. These additional sources of capital along with the existing cash, cash equivalents, and investments provide an expected cash runway through a potential launch of avutometinib and defactinib for recurrent LGSOC into Q4 2025.

Total operating expenses for the three months ended December 31, 2024 (the "2024 Quarter") were $31.6 million, compared to $31.1 million for the three months ended December 31, 2023 (the "2023 Quarter").

Research & development expenses for the 2024 Quarter were $20.8 million, compared to $22.5 million for the 2023 Quarter. The decrease of $1.7 million, or 7.6%, primarily resulted from decreased contract research organization costs and decreased drug substance and drug product costs.

Selling, general & administrative expenses for the 2024 Quarter were $10.8 million, compared to $8.6 million for the 2023 Quarter. The increase of $2.2 million, or 25.6%, was primarily related to increased personnel costs, including non-cash stock compensation and increased consulting and professional fees.

Net loss for the 2024 Quarter was $64.6 million, or $1.33 per share (basic and diluted), compared to a net loss of $27.4 million, or $1.02 per share (basic and diluted) for the 2023 Quarter.

For the 2024 Quarter, non-GAAP adjusted net loss was $29.3 million, or $0.60 per share (diluted), compared to non-GAAP adjusted net loss of $29.6 million, or $1.10 per share (diluted) for the 2023 Quarter. Please refer to the GAAP to Non-GAAP Reconciliation attached to this press release.

Full-Year 2024 Financial Results

Total operating expenses for the year ended December 31, 2024 (the "2024 Period") were $125.0 million, compared to $92.1 million for the year ended December 31, 2023 (the "2023 Period").

Research & development expenses for the 2024 Period were $81.3 million, compared to $61.4 million for the 2023 Period. The increase of $19.9 million, or 32.4%, was primarily related to increased contract research organization costs, increased investigator fee costs, increased consulting fees and increased personnel costs, including non-cash stock compensation.

Selling, general & administrative expenses for the 2024 Period were $43.6 million, compared to $30.7 million for the 2023 Period. The increase of $12.9 million, or 42.0%, 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 a one-time cost associated with July 2024 financing activities.

Net loss for the 2024 Period was $130.6 million, or $3.66 per share (basic and diluted), compared to $87.4 million, or $3.96 per share (basic and diluted, each as adjusted for the Company's reverse stock split) for the 2023 Period.

For the 2024 Period, non-GAAP adjusted net loss was $107.4 million, or $3.01 per share (diluted) compared to non-GAAP adjusted net loss of $85.2 million, or $3.86 per share (diluted, as adjusted for the Company's reverse stock split), for the 2023 Period. 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 months and year ended December 31, 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 an oral RAF/MEK clamp that potently inhibits MEK1/2 kinase activities and 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 the MEK-only inhibitors.

Defactinib is an oral, selective inhibitor of focal adhesion kinase (FAK) and proline-rich tyrosine kinase-2 (Pyk2), the two members of the focal adhesion kinase family of non-receptor protein tyrosine kinases. FAK and Pyk2 integrate signals from integrin and growth factor receptors to regulate cell proliferation, survival, migration, and invasion. FAK activation has been shown to mediate resistance to multiple anti-cancer agents, including RAF and MEK inhibitors.

Verastem Oncology is currently conducting clinical trials with avutometinib with and without defactinib in RAS/MAPK-driven tumors as part of its Raf And Mek Program or RAMP. Verastem is currently enrolling patients and activating sites for RAMP 301 (GOG-3097/ENGOT-ov81/NCRI)

(NCT06072781), an international Phase 3 confirmatory trial evaluating the combination of avutometinib and defactinib versus standard chemotherapy or hormonal therapy for the treatment of recurrent low-grade serous ovarian cancer (LGSOC).

Verastem was granted Priority Review and a Prescription Drug User Fee Act (PDUFA) date of June 30, 2025, for its New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA), for the investigational combination of avutometinib and defactinib in adults with recurrent KRAS mutant LGSOC who received at least one prior systemic therapy. Verastem initiated a rolling NDA in May 2024 to the FDA and completed its NDA submission in October 2024. The FDA granted Breakthrough Therapy Designation for the treatment of patients with recurrent LGSOC after one or more prior lines of therapy, including platinum-based chemotherapy, in May 2021. Avutometinib alone or in combination with defactinib was also granted Orphan Drug Designation by the FDA for the treatment of LGSOC.

Verastem Oncology has established a clinical collaboration with Amgen to evaluate LUMAKRAS™ (sotorasib) in combination with avutometinib and defactinib in both treatment-naïve patients and in patients whose KRAS G12C mutant non-small cell lung cancer progressed on a G12C inhibitor as part of the RAMP 203 trial (NCT05074810). Verastem has received Fast Track Designation from the FDA for the triplet combination in April 2024. 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 the avutometinib and defactinib combination for the treatment of pancreatic cancer.

About VS-7375, an Oral KRAS G12D (ON/OFF) Inhibitor

VS-7375 is a potential best-in-class, potent, and selective oral KRAS G12D dual ON/OFF inhibitor. VS-7375 is the lead program from the Verastem Oncology discovery and development collaboration with GenFleet Therapeutics. GenFleet's IND for VS-7375 (known as GFH375 in China) was approved in China in June 2024, and the first patient was dosed in a Phase 1/2 study in July 2024.

About the GenFleet Therapeutics Collaboration

The collaboration with GenFleet Therapeutics aims to advance three oncology discovery programs related to RAS/MAPK pathway-driven cancers. The collaboration provides Verastem with an exclusive option to obtain a license for each of the three compounds in the collaboration after the successful completion of pre-determined milestones in a Phase 1 trial. Verastem selected VS-7375 (also known as GFH375), an oral KRAS G12D (ON/OFF) inhibitor, as its lead program in December 2023 and the license for VS-7375 that was exercised in January 2025 is the first one from this collaboration. The licenses would give Verastem development and commercialization rights outside the GenFleet markets 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 RAS/MAPK pathway-driven cancers. 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, FAK inhibition and KRAS G12D inhibition. For more information, please visit www.verastem.com and follow us on LinkedIn.

Forward-Looking Statements

This press release includes forward-looking statements. These forward-looking statements generally can be identified by the use of words such as "anticipate," "expect," "plan," "could," "may," "believe," "estimate," "forecast," "goal," "project," and other words of similar meaning. Such forward-looking statements address various matters about, among other things, Verastem Oncology's programs and product candidates, strategy, future plans and prospects, including statements related to the potential for and timing of commercialization of product candidates, the anticipated timing for the IND application for VS-7375/GFH375, the expected outcome and benefits of the Company's collaboration with GenFleet Therapeutics (Shanghai), Inc., the timing of commencing and completing trials and compiling data, the expected timing of the presentation of data by the Company and the potential clinical value of various of the Company's clinical trials. Each forward-looking statement contained in this press release is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statement. Applicable risks and uncertainties include, among others: the uncertainties inherent in research and development, such as the possibility of negative or unexpected results of clinical trials; that we may 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 may fail to fully perform under the agreement; that the development and commercialization of our product candidates may take longer or cost more than planned, including as a result of conducting additional studies or our decisions regarding execution of such commercialization; that data may not be available when expected; risks associated with preliminary and interim data, which may not be representative of more mature data; that our product candidates may not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients; and the risks identified under the heading "Risk Factors" as detailed in the Company's Annual Report on Form 10-K for the year ended December 31, 2024, as filed with the Securities and Exchange Commission (SEC) on March 20, 2025, as well as the other information we file with the SEC, are possibly realized. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. You are encouraged to read our filings with the SEC, available at www.sec.gov, for a discussion of these and other risks and uncertainties. The forward-looking statements in this press release speak only as of the date of this press release, and we undertake no obligation to update or revise any of these statements. Our business is subject to substantial risks and uncertainties, including those referenced above. Investors, potential investors, and others should give careful consideration to these risks and uncertainties.

Verastem Oncology

Condensed Consolidated Balance Sheets

(in thousands)

(unaudited)

	December 31, 2024	December 31, 2023

Cash, cash equivalents, & investments	$ 88,818	$ 137,129
Grants receivable	200	—
Prepaid expenses and other current assets	5,943	6,553
Property and equipment, net	32	37
Right-of-use asset, net	1,405	1,171
Restricted cash and other assets	5,140	4,828
Total assets	**$ 101,538**	**$ 149,718**
Current Liabilities	30,973	$ 26,380
Long term debt	40,724	40,086
Lease liability, long-term	535	530
Preferred stock tranche liability	—	4,189
Warrant Liability	58,199	—
Convertible preferred stock	—	21,159
Stockholders' equity	(28,893)	57,374
Total liabilities, convertible preferred stock and stockholders' equity	**$ 101,538**	**$ 149,718**

Verastem Oncology

Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

(unaudited)

	Three months ended December 31,		**Year ended December 31,**	
	2024	**2023**	**2024**	**2023**
Revenue				
Sale of COPIKTRA license and related assets	$ —	$ —	$ 10,000	$ —
Total revenue	—	—	10,000	—
Operating expenses:				

Research and development	20,811	22,502	81,334	61,356
Selling, general and administrative	10,779	8,637	43,622	30,728
Total operating expenses	31,590	31,139	124,956	92,084
Loss from operations	(31,590)	(31,139)	(114,956)	(92,084)
Other income (expense)	9	(49)	(123)	(109)
Interest income	968	1,869	4,149	6,214
Interest expense	(1,146)	(1,120)	(4,562)	(4,139)
Change in fair value of preferred stock tranche liability	—	3,071	4,189	2,751
Change in fair value of warrant liability	(32,606)	—	(19,149)	—
Net loss before taxes	(64,365)	(27,368)	(130,452)	(87,367)
Income tax expense	(185)	—	(185)	—
Net Loss	$ (64,550)	$ (27,368)	$ (130,637)	$ (87,367)
Net loss per share—basic and diluted	$ (1.33)	$ (1.02)	$ (3.66)	$ (3.96)[(1)]
Weighted average common shares outstanding used in computing:				
Net loss per share – basic and diluted	48,709	26,808	35,713	22,054[(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 December 31,		Year ended December 31,	
	2024	**2023**	**2024**	**2023**
Net loss reconciliation:				
Net loss (GAAP basis)	$ (64,550)	$ (27,368)	$ (130,637)	$ (87,367)
Adjust:				
Stock-based compensation expense	2,019	1,598	7,342	5,860
Non-cash interest, net	207	(837)	(5)	(1,132)

Change in fair value of preferred stock tranche liability	—	(3,071)	(4,189)	(2,751)
Change in fair value of warrant liability	32,606	—	19,149	—
Severance and other	371	113	990	199
Adjusted net loss (non-GAAP basis)	$ (29,347)	$ (29,565)	$ (107,350)	$ (85,191)
Reconciliation of net loss per share				
Net loss per share – diluted (GAAP basis)	$ (1.33)	$ (1.02)	$ (3.66)	$ (3.96)[1]
Adjust per diluted share				
Stock-based compensation expense	0.04	0.06	0.21	0.26[1]
Non-cash interest, net	0.01	(0.03)	—	(0.05)[1]
Change in fair value of preferred stock tranche liability	—	(0.11)	(0.12)	(0.12)[1]
Change in fair value of warrant liability	0.67	—	0.53	—
Severance and other	0.01	—	0.03	0.01[1]
Adjusted net loss per share – diluted (non-GAAP basis)	$ (0.60)	$ (1.10)	$ (3.01)	$ (3.86)[1]
Weighted average common shares outstanding used in computing net loss per share—diluted	$ 48,709	$ 26,808	$ 35,713	$ 22,054[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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For Investor and Media Inquiries:
Julissa Viana
Vice President, Corporate Communications,
Investor Relations & Patient Advocacy
investors@verastem.com or
media@verastem.com

Source: Verastem Oncology