Verastem Oncology Announces Two-Year Median Follow-Up Data on AVMAPKI® FAKZYNJA® Combination Therapy (avutometinib capsules; defactinib tablets) in Recurrent Low-Grade Serous Ovarian Cancer at the SGO 2026 Annual Meeting on Women's Cancers

April 10, 2026 at 7:00 AM EDT

Patients in the Phase 2 RAMP 201 clinical trial demonstrated sustained clinical benefits across multiple efficacy measures highlighting durability of response with a median follow-up of two years

52% of patients with a KRAS mutation and 30% of patients with KRAS wild-type recurrent LGSOC remained on therapy for more than one year

After two years of follow-up, the combination therapy continued to demonstrate a well-tolerated safety profile, with no new safety signals and a low discontinuation rate due to adverse events

Poster presentation of new exposure response analysis demonstrates that the best therapeutic effect of avutometinib plus defactinib can be achieved when using the approved dose and schedule

BOSTON--(BUSINESS WIRE)--Apr. 10, 2026-- Verastem Oncology (Nasdaq: VSTM), a biopharmaceutical company committed to advancing new medicines for patients with RAS/MAPK pathway-driven cancers, today announced two-year median follow-up data from the Phase 2 RAMP 201 clinical trial that evaluated AVMAPKI® FAKZYNJA® combination therapy (avutometinib capsules; defactinib tablets) in patients with recurrent low-grade serous ovarian cancer (LGSOC) will be presented today during an oral plenary session at the Society of Gynecologic Oncology (SGO) 2026 Annual Meeting on Women's Cancers taking place in San Juan, Puerto Rico, April 10-13, 2026.

"Patients who remained on treatment with avutometinib plus defactinib for two years were able to maintain the same level of response and duration of therapy as seen in the primary analysis, suggesting that patients can stay on the combination for a long period of time, derive benefit and have manageable toxicity during long-term administration of these medications," said Rachel Grisham, M.D., RAMP 201 Presenting Investigator, Section Head, Ovarian Cancer at Memorial Sloan Kettering Cancer Center (MSK) in New York, NY, and Global Lead Principal Investigator of GOG-3097/ENGOT-ov81/GTG-UK/RAMP 301. "As we approach the one-year FDA approval anniversary of avutometinib in combination with defactinib, this analysis reinforces progress in bringing a durable and clinically meaningful option to patients."

In the updated analysis, with ongoing patients presenting a median follow-up of 24.9 months, efficacy measures, including median duration of response (mDOR) and median progression free survival (mPFS), and safety, are consistent with the primary analysis, which was conducted more than 13 months prior:

	Long-Term Median Follow-Up *(data cutoff of August 12, 2025)*			**Primary Analysis*** *(data cutoff of June 30, 2024)*		
	(N=17)			(N=109)		
	Total (N=17)	**KRAS mt (N=12)**	**KRAS wt (N=5)**	**Total (N=109)**	**KRAS mt (N=57)**	**KRAS wt (N=52)**
mDoR	31.1 months	31.1 months	12 months	31.1 months	31.1 months	9.2 months
mPFS	12.9 months	19.6 months	12.7 months	12.9 months	22 months	12.8 months

**The primary analysis was published in the Journal of Clinical Oncology (Banerjee et al, July 11, 2025).*

Fifty percent of patients with KRAS-mutated and 30 percent with KRAS wild-type LGSOC remained on therapy for more than one year. Adverse events (AEs) were consistent with the primary analysis with no new safety signals observed, and a 12 percent discontinuation rate due to adverse events.

"With two years of follow-up demonstrating durable benefit, these analyses reinforce confidence in avutometinib plus defactinib in the real-world setting for patients living with recurrent LGSOC," said Bradley Monk, M.D., FACOG, FACS, RAMP 201 Investigator and Gynecologic Oncologist with Florida Cancer Specialists & Research Institute and Director GOG-Partners. "Notably, seeing one-third of patients without KRAS mutations remain on therapy for over a year is clinically meaningful, especially in a patient population with limited treatment options. The data also underscore the combination's manageable safety profile and how dose holds can be used effectively to maintain dose intensity, helping distinguish this treatment strategy from prior MEK-based approaches."

Exposure-Response Analysis for Avutometinib in Combination with Defactinib in LGSOC

Efficacy analyses included patients with LGSOC (N=158) from the FRAME and RAMP 201 studies, while safety analyses included a broader population (N=303) from the FRAME, RAMP 201, and RAMP 202 studies.

All efficacy endpoints, including overall response rates, duration of response, and best target lesion response, suggest the best therapeutic effect is achieved with the FDA approved dose of avutometinib 3.2 mg twice weekly plus defactinib 200 mg twice daily. While a lower avutometinib dose may mitigate treatment emergent adverse events (TEAEs) (the most frequent being grade ≥2 skin disorders (39.9%; n=121), followed by grade ≥2 gastrointestinal toxicity (34.0%; n=103), grade ≥2 liver function tests (25.1%; n=76), and grade ≥3 creatine phosphokinase elevation (14.5%; n=44)), it may also compromise the efficacy. Importantly, these analyses demonstrated that TEAEs can be monitored and managed with dose interruptions, and subsequently resume treatment at the approved dose level, to allow patients to stay on treatment.

Verastem has an exhibition booth (#608) at the meeting to provide an overview of its approved therapy and ongoing cancer research. The full schedule and poster titles are available online at the SGO 2026 Annual Meeting on Women's Cancer website .

About RAMP 201

RAMP 201 (ENGOTov60/GOG3052/NCRI) (NCT04625270) was an adaptive, two-part multicenter, parallel cohort, randomized, open-label Phase 2 registration-directed trial evaluating the efficacy and safety of avutometinib alone and in combination with defactinib in patients with recurrent low-grade serous ovarian cancer (LGSOC). The first part of the trial (Part A) determined the selection of the go-forward regimen, which was the combination of avutometinib and defactinib versus avutometinib alone, based on overall response rates. The expansion phases of the trial (Parts B and C) evaluated the safety and efficacy of the go-forward regimen of avutometinib 3.2 mg twice weekly and defactinib 200 mg twice daily. The Part D portion of the trial evaluated a low dose of the combination to inform individualized dose reduction.

About Low-Grade Serous Ovarian Cancer (LGSOC)

LGSOC is a rare ovarian cancer that is insidious and persistent. LGSOC is distinct and different from high-grade serous ovarian cancer (HGSOC) and requires different treatment. LGSOC is highly recurrent and less sensitive to chemotherapy compared to HGSOC. Approximately 6,000-8,000 women in the U.S. and 80,000 worldwide are living with this disease. LGSOC affects younger women with bimodal peaks of diagnosis at ages between 20-30 and 50-60 and has a median survival of approximately ten years. Approximately 70 percent of LGSOC shows RAS pathway-associated mutations, and 30 percent of people with LGSOC have a KRAS mutation. The majority of patients report a negative impact of LGSOC on their mental and physical health, fertility, and long-term quality of life.

About AVMAPKI and FAKZYNJA Combination Therapy

AVMAPKI (avutometinib) inhibits MEK kinase activity while also blocking the compensatory reactivation of MEK by upstream RAF. RAF and MEK proteins are regulators of the RAS/RAF/MEK/ERK (MAPK) pathway. Blocking RAF and/or MEK activates FAK, a key mediator of drug resistance. FAKZYNJA (defactinib) is a FAK inhibitor and together, the avutometinib and defactinib combination was designed to provide a more complete blockade of the signaling that drives the growth and drug resistance of RAS/MAPK pathway-dependent tumors.

The U.S. Food and Drug Administration (FDA) approved AVMAPKI® FAKZYNJA® CO-PACK (avutometinib capsules; defactinib tablets) for the treatment of adult patients with KRAS-mutated recurrent LGSOC who have received prior systemic therapy on May 8, 2025. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial. Verastem is conducting RAMP 301 (GOG-3097/ENGOT-ov81/GTG-UK) (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) with and without a KRAS mutation. Verastem is also evaluating avutometinib plus defactinib with standard-of-care chemotherapy as a potential treatment in the first-line for patients with advanced pancreatic cancer (RAMP 205; NCT05669482). Avutometinib and defactinib are not approved by the FDA or any other regulatory authority, either in combination or with other therapies, for any of these investigative uses. Neither avutometinib nor defactinib are approved by the FDA or any other regulatory authority on a stand-alone basis for any use.

AVMAPKI FAKZYNJA CO-PACK U.S. Indication

Indication

AVMAPKI FAKZYNJA CO-PACK is indicated for the treatment of adult patients with *KRAS*-mutated recurrent low-grade serous ovarian cancer (LGSOC) who have received prior systemic therapy.

This indication is approved under accelerated approval based on tumor response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

Important Safety Information

Warnings and Precautions

- **Ocular Toxicities:** Ocular toxicities, including visual impairment and vitreoretinal disorders, occurred. Perform comprehensive ophthalmic evaluation at baseline, prior to cycle 2, every three cycles thereafter, and as clinically indicated. Withhold AVMAPKI FAKZYNJA CO-PACK for ocular toxicities until improvement at the same or reduced dose. Permanently discontinue AVMAPKI FAKZYNJA CO-PACK for any grade 4 toxicity.
- **Serious Skin Toxicities:** Skin toxicities, including photosensitivity and severe cutaneous adverse reactions (SCARSs) occurred. Adhere to concomitant medications. Monitor for skin toxicities and interrupt, reduce or permanently discontinue AVMAPKI FAKZYNJA CO-PACK based on severity, tolerability and duration.
- **Hepatotoxicity:** Monitor liver function tests prior to each cycle, on day 15 of the first 4 cycles, and as clinically indicated. Withhold, reduce or discontinue AVMAPKI FAKZYNJA CO-PACK based on severity and persistence of abnormality.
- **Rhabdomyolysis:** Monitor creatine phosphokinase prior to the start of each cycle, on day 15 of the first four cycles, and as clinically indicated. If increased CPK occurs, evaluate patients for rhabdomyolysis or other causes. Withhold, reduce or

permanently discontinue AVMAPKI FAKZYNJA CO-PACK based on severity and duration of the adverse reaction.
- **Embryo-Fetal Toxicity:** AVMAPKI FAKZYNJA CO-PACK can cause fetal harm. Advise patients of the potential risk to a fetus and to use effective contraception.

Adverse Reactions

The most common (≥ 25%) adverse reactions, including laboratory abnormalities, were increased creatine phosphokinase, nausea, fatigue, increased aspartate aminotransferase, rash, diarrhea, musculoskeletal pain, edema, decreased hemoglobin, increased alanine aminotransferase, vomiting, increased blood bilirubin, increased triglycerides, decreased lymphocyte count, abdominal pain, dyspepsia, dermatitis acneiform, vitreoretinal disorders, increased alkaline phosphatase, stomatitis, pruritus, visual impairment, decreased platelet count, constipation, dry skin, dyspnea, cough, urinary tract infection, and decreased neutrophil count.

Drug Interactions

- **Strong and moderate CYP3A4 inhibitors**: Avoid concomitant use with AVMAPKI FAKZYNJA CO-PACK.
- **Strong and moderate CYP3A4 inducers**: Avoid concomitant use with AVMAPKI FAKZYNJA CO-PACK.
- **Warfarin:** Avoid concomitant use of AVMAPKI FAKZYNJA CO-PACK with warfarin and use an alternative to warfarin.
- **Gastric acid reducing agents**: Avoid concomitant use of AVMAPKI FAKZYNJA CO-PACK with proton pump inhibitors (PPIs) or H2 receptor antagonists. If use of an acid-reducing agent cannot be avoided, administer FAKZYNJA 2 hours before or 2 hours after the administration of a locally acting antacid.

Use in Specific Populations

- **Lactation**: Advise not to breastfeed.
- **Fertility:** May impair fertility in males and females.

Click here for full Prescribing Information.

About Verastem Oncology

Verastem Oncology (Nasdaq: VSTM) is a biopharmaceutical company committed to developing and commercializing new medicines to improve the lives of patients diagnosed with RAS/MAPK pathway-driven cancers. Verastem markets AVMAPKI® FAKZYNJA® CO-PACK in the U.S. 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.

Disclosure: Dr. Grisham has financial interests related to Verastem Oncology.

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 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 we may not be successful in our continued commercialization of AVMAPKI FAKZYNJA CO-PACK; 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; risks associated with the recent changes in administration policy or actions that may create regulatory uncertainty that may adversely affect our business; risks associated with the current administration's reductions to the FDA's workforce and any subsequent reductions that may lead to disruptions and delays in the FDA's review and oversight of our product candidates and impact the FDA's ability to provide timely feedback on our development programs; 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, 2025, as filed with the Securities and Exchange Commission (SEC) on March 4, 2026, 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.

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