Agios Reports Third Quarter 2026 Financial Results and Provides Business Update

November 5, 2026

- Mitapivat (PYRUKYND® and AQVESME®) delivered worldwide net revenues of $55.4 million in third quarter of 2026, more than four times the $12.9 million reported in third quarter of 2025
- Strong U.S. demand for AQVESME in thalassemia continues, with 619 cumulative prescriptions with completed start forms from REMS-certified physicians, plus 48 additional prescriptions with start forms in process as of September 30, 2026
- New PDUFA goal date for mitapivat sickle cell disease sNDA set for February 1, 2027; company is launch-ready and in labeling discussions with FDA
- 14 abstracts accepted at ASH 2026, including new clinical data for mitapivat in sickle cell disease and for AG-236 in polycythemia vera, highlighting the breadth and depth of Agios' rare hematology portfolio
- $907.0 million in cash, cash equivalents, and marketable securities as of September 30, 2026, fully funding commercial launches and pipeline programs; company is targeting non-GAAP profitability in 2028

CAMBRIDGE, Mass., Nov. 05, 2026 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a commercial-stage biopharmaceutical company focused on delivering innovative medicines for patients with rare diseases, today announced financial results and updates for the third quarter ended September 30, 2026.

"The third quarter was a record for our product revenue, outperforming our entire 2025 total in just three months. This growth was driven by the U.S. commercial launch of AQVESME in thalassemia, where broadening demand is translating into a durable base of patients on therapy," said Brian Goff, Chief Executive Officer, Agios. "This launch has built the commercial foundation for our largest indication opportunity with mitapivat – sickle cell disease. Our conviction in mitapivat for patients with sickle cell disease is grounded in the robust efficacy and safety data generated to date and we are launch-ready ahead of our PDUFA goal date of February 1, 2027. With this commercial infrastructure, alongside a strong balance sheet and a disciplined operating model, Agios is well positioned for sustained growth as a focused, rare hematology company."

Third Quarter 2026 and Recent Corporate Highlights

- **Mitapivat (PYRUKYND® and AQVESME®) Commercial Performance and Update –**
  - $48.7 million in U.S. net revenue and $6.6 million in ex-U.S. net revenue in the third quarter of 2026
    - U.S. net revenue increased 20% sequentially, driven by the U.S. commercial launch of AQVESME (mitapivat) in thalassemia in late January 2026.
    - Ex-U.S. net revenue increased 74% sequentially, driven primarily by early access programs in Europe following the late May 2026 approval of PYRUKYND (mitapivat) in thalassemia in the European Union. This robust, early momentum underscores the strong global opportunity for mitapivat, which the company is advancing through capital-efficient partnerships with Avanzanite Bioscience B.V. in Europe and NewBridge Pharmaceuticals in the Gulf Cooperation Council (GCC) region.
  - AQVESME demand continues to expand following the January 2026 U.S. commercial launch in thalassemia
    - In the third quarter, 177 AQVESME prescriptions with completed Agios start forms (ASFs) were written by Risk Evaluation and Mitigation Strategy (REMS)-certified U.S. physicians, bringing cumulative prescriptions to 619 as of September 30, 2026.
    - An additional 48 cumulative prescriptions had ASFs in process as of September 30, 2026, which are not included in the reported metric above.
    - Demand continues to broaden, with deeper penetration in transfusion-dependent patients and expansion into the larger non-transfusion dependent population. Approximately 75% of patients remain on AQVESME after six months of treatment.
- **Operating Model Update –**
  - Over the course of 2026, Agios reviewed its operating model, and will have reduced its R&D headcount by over 100 roles by year-end, representing a more than 35% decrease compared with 2025. The company expects the associated cost savings of these reductions to be realized in 2027.

Research and Development (R&D) Highlights

- **Mitapivat (pyruvate kinase [PK] activator) –**
  - **Sickle Cell Disease –**
    - Agios announced that the U.S. Food and Drug Administration (FDA) has extended the Prescription Drug User Fee Act (PDUFA) goal date for its mitapivat sickle cell disease supplemental New Drug Application (sNDA) by three months, from November 1, 2026, to February 1, 2027.
    - The FDA recently requested additional information to evaluate whether a REMS is necessary for mitapivat in sickle cell disease. The FDA classified Agios' response to this information request as a major amendment to the sNDA, resulting in the three-month review extension.
    - Agios will continue its labeling discussions with the FDA as part of the ongoing sNDA review, including elements of a potential REMS.

- **American Society of Hematology (ASH) Annual Meeting and Exposition (ASH 2026) –**
  - Agios announced that 14 abstracts led by Agios and external collaborators have been accepted for presentation and publication at ASH 2026, taking place December 12-15, 2026, in New Orleans, Louisiana. These include three oral presentations featuring new data across mitapivat clinical programs.
  - New clinical data from the RISE UP Phase 3 trial of mitapivat in sickle cell disease shows durable hemoglobin improvements for up to 115 weeks (over two years) with continued treatment. Additional post-hoc RISE UP analyses show the potential for mitapivat to improve measures of kidney function and cardiac remodeling in this population, which may reflect its unique dual activation of two pyruvate kinase isoforms: PKR, expressed in red blood cells, and PKM2, expressed in organ tissues.
  - The first clinical data for AG-236 from a Phase 1 trial in healthy volunteers support every-six-month dosing in polycythemia vera, supporting a potential best-in-class profile.

Third Quarter 2026 Financial Results
For the quarter ended September 30, 2026, net loss was $65.0 million, compared to net loss of $103.4 million for the quarter ended September 30, 2025.

- **Net product revenue from U.S. sales** of mitapivat (PYRUKYND and AQVESME) for the third quarter of 2026 was $48.7 million, compared to $12.3 million for the third quarter of 2025.
- **Net product revenue from ex-U.S. sales** of mitapivat (PYRUKYND) for the third quarter of 2026 was $6.6 million, compared to $0.6 million for the third quarter of 2025.
- **Cost of sales** for the third quarter of 2026 was $2.5 million.
- **Research and Development (R&D) expenses** were $73.5 million for the third quarter of 2026, compared to $86.8 million for the third quarter of 2025, driven primarily by reductions in spend associated with mitapivat and tebapivat process development and certain clinical trials.
- **Selling, General and Administrative (SG&A) expenses** were $56.5 million for the third quarter of 2026, compared to $41.3 million for the third quarter of 2025, due to an increase in activities supporting the U.S. commercial launch of AQVESME in thalassemia and preparation for the potential U.S. commercial launch of mitapivat in sickle cell disease.
- **Cash, cash equivalents and marketable securities** were $907.0 million as of September 30, 2026, compared to $1.2 billion as of December 31, 2025. Agios expects that its cash, cash equivalents and marketable securities, together with anticipated product revenue and interest income, will provide the financial independence to execute the U.S. commercial launch of AQVESME in thalassemia, prepare for the potential U.S. commercial launch of mitapivat in sickle cell disease, and progress the company's pipeline. Agios' goal is to achieve non-Generally Accepted Accounting Principles (GAAP) profitability in 2028.

Third Quarter 2026 Conference Call Information
Agios will host a conference call and live webcast today, November 5, 2026, at 8:00 a.m. ET to discuss the company's third quarter 2026 financial results and recent business highlights. The live webcast will be accessible on the Investors section of the company's website (www.agios.com) under the "Events & Presentations" tab. A replay of the webcast will be available on the company's website approximately two hours after the event.

About Agios: Fueled by Connections to Transform Rare Diseases™
At Agios, our vision is to redefine the future of rare disease treatment. Fueled by connections, we build trusted partnerships with communities – collaborating to develop and deliver innovative medicines that have the potential to transform lives. With a foundation in hematology, we combine biological expertise with real-world insights to advance a growing pipeline of rare disease medicines that reflect the priorities of the people we serve. Agios is a commercial-stage biopharmaceutical company headquartered in Cambridge, Massachusetts. To learn more, visit www.agios.com and follow us on LinkedIn and X.

Available Information about Agios
To achieve broad dissemination, Agios may disclose information to the public through a variety of disclosure channels including press releases, SEC filings, and public conference calls and webcasts. Some of the information distributed through these disclosure channels may be considered material information. Investors and others should note that Agios plans to use its website (www.agios.com) as a distribution channel to announce and give notice of Agios' upcoming events and presentations (including, but not limited to, presentations at medical or healthcare conferences). Such information, which may be deemed material, will be available on the Investors section of the company's website under the "Events & Presentations"

tab. In addition, you may sign up to automatically receive email alerts about Agios' upcoming events and presentations ("Calendar Alerts") by visiting the "Email Alerts" option under the "IR Resources" tab of the Investors section of the company's website and submitting your email address.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding the potential benefits of PYRUKYND® (mitapivat), AQVESME® (mitapivat), and AG-236; Agios' plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including mitapivat and AG-236; Agios' expectations for the review of marketing applications for mitapivat by regulatory agencies, including the FDA; Agios' strategic vision and goals; and the potential benefits of Agios' strategic plans and focus. The words "anticipate," "expect," "goal," "hope," "milestone," "plan," "potential," "possible," "strategy," "will," "vision," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from Agios' current expectations and beliefs. For example, there can be no guarantee that any product candidate Agios is developing will successfully commence or complete necessary preclinical and clinical development phases, or that development of any of Agios' product candidates will successfully continue. There can be no guarantee that any positive developments in Agios' business will result in stock price appreciation. Management's expectations and, therefore, any forward-looking statements in this press release could also be affected by risks and uncertainties relating to a number of other important factors, including, without limitation: risks and uncertainties related to the impact of pandemics or other public health emergencies to Agios' business, operations, strategy, goals and anticipated milestones, including its ongoing and planned research activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved products, and launching, marketing and selling current or future approved products; Agios' results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the U.S. FDA, the EMA or other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies; Agios' ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials; unplanned cash requirements and expenditures; competitive factors; Agios' ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates it is developing; Agios' ability to establish and maintain key collaborations; uncertainty regarding any royalty payments related to the sale of its oncology business or any milestone or royalty payments related to its in-licensing of AG-236 and cevidoplenib, and the uncertainty of the timing of any such payments; uncertainty of the results and effectiveness of the use of Agios' cash and cash equivalents; and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in Agios' public filings with the Securities and Exchange Commission. Any forward-looking statements contained in this press release speak only as of the date hereof, and Agios expressly 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.

Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	September 30, 2026	December 31, 2025
Cash, cash equivalents, and marketable securities	$ 907,018	$ 1,164,438
Accounts receivable, net	25,254	10,577
Inventory	37,841	32,920
Total assets	1,064,040	1,297,225
Stockholders' equity	979,336	1,193,114

Consolidated Statements of Operations Data
(in thousands, except share and per share data)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenues:				
Product revenue, net	$ 55,385	$ 12,880	$ 120,876	$ 34,061
Total revenue	55,385	12,880	120,876	34,061
Operating expenses:				
Cost of sales	$ 2,525	$ 1,679	$ 6,840	$ 4,466
Research and development	73,487	86,796	255,389	251,479
Selling, general and administrative	56,533	41,274	156,384	128,670
Total operating expenses	132,545	129,749	418,613	384,615
Loss from operations	(77,160)	(116,869)	(297,737)	(350,554)
Royalty income from gain on sale of oncology business	2,969	—	2,969	—
Interest income, net	8,880	13,369	29,405	43,969
Other income, net	266	67	504	1,843
Net loss	$ (65,045)	$ (103,433)	$ (264,859)	$ (304,742)
Net loss per share - basic and diluted	$ (1.09)	$ (1.78)	$ (4.46)	$ (5.27)

Weighted-average number of common shares used in computing net loss per share – basic and diluted	59,707,904	58,139,277	59,329,729	57,846,173

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