Agios Reports First Quarter 2026 Financial Results and Provides Business Update

April 29, 2026

- Mitapivat (PYRUKYND® and AQVESME™) generated worldwide net revenues of $20.7 million in the first quarter of 2026, compared to $8.7 million in the first quarter of 2025
- Strong initial U.S. commercial launch of AQVESME in thalassemia, with 242 prescriptions written as of March 31, 2026
- Company plans to submit mitapivat sNDA for sickle cell disease in the second quarter of 2026
- Pipeline advancing to multiple value-driving inflection points in 2026, including two Phase 2 readouts for next-generation PK activator tebapivat
- $1.0 billion in cash, cash equivalents, and marketable securities as of March 31, 2026

CAMBRIDGE, Mass., April 29, 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 first quarter ended March 31, 2026.

“Our first-quarter performance reflects strong execution and significant progress against our 2026 strategic objectives,” said Brian Goff, Chief Executive Officer, Agios. “The solid early momentum of our U.S. commercial launch of AQVESME in thalassemia highlights both the medicine’s clinical value and strong community reception. Additionally, following collaborative engagement with the FDA, we now plan to submit our mitapivat sNDA for sickle cell disease under the U.S. accelerated approval pathway in the second quarter. 2026 marks an important growth inflection point for Agios as we continue to build a sustainable rare disease company that is rooted in hematology and focused on delivering differentiated medicines that create meaningful long-term value for patients and shareholders.”

First Quarter 2026 and Recent Corporate Highlights

Mitapivat (PYRUKYND® and AQVESME™) Commercial Performance and Update

- **$18.8 million in U.S. net revenue** and **$1.9 million in ex-U.S. net revenue** in the first quarter of 2026.
  - U.S. net revenue was driven by the U.S. commercial launch of AQVESME™ (mitapivat) in thalassemia in late January 2026.
  - Ex-U.S. net revenue reflected demand for PYRUKYND® (mitapivat) in thalassemia in Saudi Arabia.
- **242 AQVESME prescriptions** for thalassemia were written by Risk Evaluation and Mitigation Strategy (REMS)-certified U.S. physicians as of March 31, 2026, driven by motivated prescribers and highly engaged patients.

R&D Highlights

- **Mitapivat**
  - **Thalassemia –**
    - In March 2026, the Emirates Drug Establishment of the United Arab Emirates (UAE) approved PYRUKYND for the treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia. With this approval, PYRUKYND becomes the only medicine approved in the UAE for this broad patient population.
    - Mitapivat is now approved for adults with thalassemia in the U.S., Saudi Arabia, and the UAE. A marketing application for mitapivat in thalassemia is currently under review by the European Commission.
  - **Sickle Cell Disease –**
    - Agios confirmed plans to pursue U.S. accelerated approval for mitapivat in sickle cell disease, following completion of its pre-supplemental New Drug Application (sNDA) meeting with the U.S. Food and Drug Administration (FDA).
    - The FDA’s accelerated approval pathway expedites the availability of medicines that can fill a medical need for a serious condition, with the requirement of a confirmatory clinical trial to convert to a traditional approval.
    - The company now plans to submit an sNDA for mitapivat in sickle cell disease in the second quarter of 2026.
- **Tebapivat**
  - **Lower-Risk Myelodysplastic Syndromes (LR-MDS) –**
    - Agios expects to report topline results from its Phase 2b trial in the first half of 2026. Based on findings from the Phase 2a trial, the Phase 2b open-label trial is evaluating three higher daily dose levels of tebapivat (10 mg, 15 mg, and 20 mg) over a 24-week period. The primary endpoint is the proportion of participants achieving transfusion independence, defined as being transfusion-free for at least 8 consecutive weeks during the 24-week period.

- **Sickle Cell Disease –**
  - Agios expects to report topline results from its Phase 2 trial in the second half of 2026. This double-blind, randomized, placebo-controlled trial is evaluating three daily dose levels of tebapivat (2.5 mg, 5 mg, and 7.5 mg) versus matched placebo over a 12-week period. The primary endpoint is hemoglobin response, defined as a ≥1.0 g/dL increase in average hemoglobin concentration from Week 10 through Week 12 compared with baseline.

First Quarter 2026 Financial Results

For the quarter ended March 31, 2026, net loss was $99.1 million, compared to net loss of $89.3 million for the quarter ended March 31, 2025.

- **Net product revenue from U.S. sales** of mitapivat (PYRUKYND and AQVESME) for the first quarter of 2026 was $18.8 million, compared to $8.7 million for the first quarter of 2025.
- **Net product revenue from ex-U.S. sales** of mitapivat (PYRUKYND) for the first quarter of 2026 was $1.9 million.
- **Cost of sales** for the first quarter of 2026 was $1.3 million.
- **Research and Development (R&D) Expenses** were $81.1 million for the first quarter of 2026, compared to $72.7 million for the first quarter of 2025, due to workforce-related expenses supporting pipeline advancement efforts, as well as increased mitapivat process development expenses.
- **Selling, General and Administrative (SG&A) Expenses** were $48.3 million for the first quarter of 2026, compared to $41.5 million for the first quarter of 2025, due to an increase in activities to support the U.S. commercial launch of AQVESME in thalassemia, as well as an increase in stock compensation expense.
- **Cash, cash equivalents and marketable securities** were $1.0 billion as of March 31, 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, advance the company's existing clinical programs, and opportunistically expand its pipeline through both internally- and externally-discovered assets.

First Quarter 2026 Conference Call Information

Agios will host a conference call and live webcast today at 8:00 a.m. ET to discuss the company's first 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), tebapivat, AG-236 and AG-181; Agios' plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including mitapivat, tebapivat, AG-236 and AG-181; Agios' expectations for the review of marketing applications for mitapivat by regulatory agencies, including the FDA and European Commission; 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 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)

	March 31, 2026	December 31, 2025
Cash, cash equivalents, and marketable securities	$ 1,045,492	$ 1,164,438
Accounts receivable, net	16,132	10,577
Inventory	35,087	32,920
Total assets	1,184,990	1,297,225
Stockholders' equity	1,109,115	1,193,114

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

	Three Months Ended March 31,	
	2026	2025
Revenues:		
Product revenue, net	$ 20,746	$ 8,726
Total revenue	20,746	8,726
Operating expenses:		
Cost of sales	$ 1,319	$ 1,085
Research and development	81,148	72,743
Selling, general and administrative	48,304	41,527
Total operating expenses	130,771	115,355
Loss from operations	(110,025)	(106,629)
Interest income, net	10,795	16,087
Other income, net	119	1,253
Net loss	$ (99,111)	$ (89,289)
Net loss per share - basic and diluted	$ (1.69)	$ (1.55)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	58,782,241	57,459,195

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