Agios Reports Third Quarter 2025 Financial Results and Provides Business Update

October 30, 2025

- $12.9 million in third quarter PYRUKYND® (mitapivat) net revenues
- PDUFA goal date for PYRUKYND U.S. sNDA in thalassemia set for December 7, 2025
- CHMP adopted positive opinion for PYRUKYND in thalassemia; EC decision expected by early 2026
- RISE UP Phase 3 trial topline results in sickle cell disease by year-end; potential U.S. commercial launch in 2026
- Phase 2b tebapivat trial in lower-risk MDS fully enrolled; topline results expected in early 2026
- $1.3 billion dollars in cash, cash equivalents and marketable securities as of September 30, 2025

CAMBRIDGE, Mass., Oct. 30, 2025 (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, 2025.

“As we approach year-end, we remain focused on our two key PYRUKYND milestones – the potential U.S. approval in thalassemia and the topline results from the RISE UP Phase 3 trial in sickle cell disease. Our recent engagements with these communities have underscored the urgent need for innovation and PYRUKYND’s potential to address critical gaps in care for these serious and life-threatening diseases,” said Brian Goff, Chief Executive Officer, Agios. “We continued strong execution across our rare disease portfolio in the third quarter, including the completion of enrollment in our Phase 2b tebapivat trial for lower-risk MDS. We look forward to building on this strong momentum and remain steadfast in our commitment to delivering meaningful progress for the patients we serve.”

Third Quarter 2025 and Recent Corporate Highlights
Commercial Performance – PYRUKYND® (mitapivat)

- **Generated $12.9 million in net revenue** for the third quarter of 2025, representing an increase of 44 percent from $9.0 million in the third quarter of 2024 and a 3 percent increase from $12.5 million in the second quarter of 2025.
  - **262 unique patients** completed prescription enrollment forms, representing an increase of 6 percent over the second quarter of 2025.
  - **149 patients** are on therapy in the U.S., inclusive of new starts and continued therapy, representing an increase of 5 percent over the second quarter of 2025.

R&D Highlights
PYRUKYND (mitapivat)

- **Thalassemia –**
  - **United States –**
    - U.S. Food and Drug Administration (FDA) **extended the Prescription Drug User Fee Act (PDUFA) goal date** for the supplemental New Drug Application (sNDA) of PYRUKYND for the treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia by three months, to **December 7, 2025.**
      - Extension was triggered by FDA request for a **Risk Evaluation and Mitigation Strategy (REMS)** to address the potential risk of hepatocellular injury described in the original application.
      - Extension was not the result of new or additional efficacy or safety data requested by the FDA or submitted by Agios.
      - **U.S. commercial launch preparations remain underway**, and the application remains under active FDA review.
  - **Europe –**
    - **Committee for Medicinal Products for Human Use (CHMP)** of the **European Medicines Agency (EMA)** has **adopted a positive opinion** recommending approval of PYRUKYND in adults for the treatment of anemia associated with transfusion-dependent and non-transfusion-dependent alpha- or beta-thalassemia.
      - A **final decision** from the **European Commission** is expected by **early 2026**.
  - **Gulf Cooperation Council (GCC) –**
    - PYRUKYND **received approval in Saudi Arabia** for the treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia.
      - **Commercial launch activities are underway** in Saudi Arabia in partnership with **NewBridge Pharmaceuticals**.
    - In the **United Arab Emirates**, PYRUKYND thalassemia regulatory application remains **under active review**.

- **Sickle Cell Disease –**
  - **Topline results from the RISE UP Phase 3 trial** of mitapivat in sickle cell disease are expected by **year-end**, potentially supporting a **U.S. commercial launch in 2026.**

Tebapivat

- **Lower-risk Myelodysplastic Syndromes (LR-MDS) –**
  - **Completed patient enrollment in the Phase 2b trial** of tebapivat in LR-MDS. Following findings from the Phase 2a trial, the Phase 2b trial is evaluating three higher daily doses (10 mg, 15 mg, and 20 mg) versus placebo over 24 weeks. **Topline results** from this trial are **expected in early 2026**.

Third Quarter 2025 Financial Results

For the quarter ended September 30, 2025, net loss was $103.4 million dollars, compared to net income of $947.9 million dollars for the quarter ended September 30, 2024. The net income in the third quarter of 2024 was due to the milestone payment from Servier and the sale of royalty rights to Royalty Pharma, both of which were recorded in that quarter.

- **Net product revenue** from sales of PYRUKYND for the third quarter of 2025 was $12.9 million, compared to $9.0 million for the third quarter of 2024.

- **Cost of sales** for the third quarter of 2025 was $1.7 million.

- **Research and Development (R&D) Expenses** were $86.8 million for the third quarter of 2025, an increase of $14.3 million compared to the third quarter of 2024. The year-over-year increase was primarily driven by increased clinical trial costs associated with the PK activation franchise.

- **Selling, General and Administrative (SG&A) Expenses** were $41.3 million for the third quarter of 2025, representing an increase of $2.7 million compared to the third quarter of 2024, primarily driven by disciplined investment in preparation for the potential U.S. commercial launch of PYRUKYND in thalassemia.

- **Cash, cash equivalents and marketable securities** as of September 30, 2025, were $1.3 billion compared to $1.5 billion as of December 31, 2024. Agios expects that its cash, cash equivalents and marketable securities, together with anticipated product revenue and interest income, will provide the financial independence to prepare for potential PYRUKYND commercial launches in thalassemia and sickle cell disease, advance existing clinical programs, and opportunistically expand its pipeline through both internally and externally discovered assets.

Conference Call Information

Agios will host a conference call and live webcast today at 8:00 a.m. ET to discuss the company's third quarter 2025 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.

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), tebapivat, AG-236 and AG-181; Agios' plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®, tebapivat, AG-236 and AG-181; Agios' expectations for the review of marketing applications for PYRUKYND by regulatory agencies, including the FDA and European Commission; Agios' strategic vision and goals, including its key milestones for 2025; 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)

	September 30, 2025	December 31, 2024
Cash, cash equivalents, and marketable securities	$ 1,257,201	$ 1,532,031
Accounts receivable, net	5,029	4,109
Inventory	32,034	27,616
Total assets	1,385,705	1,663,199
Stockholders' equity	1,284,330	1,540,956

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenues:				
Product revenue, net	$ 12,880	$ 8,964	$ 34,061	$ 25,768
Total revenue	12,880	8,964	34,061	25,768
Operating expenses:				
Cost of sales	$ 1,679	$ 783	$ 4,466	$ 2,905
Research and development	86,796	72,455	251,479	218,476
Selling, general and administrative	41,274	38,537	128,670	105,087
Total operating expenses	129,749	111,775	384,615	326,468
Loss from operations	(116,869)	(102,811)	(350,554)	(300,700)
Gain on sale of contingent payments	—	889,136	—	889,136
Milestone payment from gain on sale of oncology business	—	200,000	—	200,000
Interest income, net	13,369	13,059	43,969	30,068
Other income, net	67	1,651	1,843	4,864
Net (loss) income before taxes	(103,433)	1,001,035	(304,742)	823,368
Income tax expense	—	53,120	—	53,120
Net (loss) income	$ (103,433)	$ 947,915	$ (304,742)	$ 770,248
Net (loss) income per share - basic	$ (1.78)	$ 16.65	$ (5.27)	$ 13.58
Net (loss) income per share - diluted	$ (1.78)	$ 16.22	$ (5.27)	$ 13.38
Weighted-average number of common shares used in computing net (loss) income per share – basic	58,139,277	56,939,403	57,846,173	56,709,318
Weighted-average number of common shares used in computing net (loss) income per share – diluted	58,139,277	58,432,796	57,846,173	57,581,382

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