Agios Reports Second Quarter 2025 Financial Results and Provides Business Update

July 31, 2025

- $12.5 million in second quarter PYRUKYND® (mitapivat) net revenues; ended second quarter with $1.3 billion dollars in cash, cash equivalents and marketable securities
- PYRUKYND sNDA for thalassemia under active review, with FDA PDUFA goal date of September 7, 2025
- Topline results from RISE UP Phase 3 trial of mitapivat in sickle cell disease on track by year-end with potential U.S. commercial launch in 2026
- Dosed first patient in the tebapivat Phase 2 sickle cell disease trial and received IND clearance for AG-236

CAMBRIDGE, Mass., July 31, 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 second quarter ended June 30, 2025.

“With fewer than 40 days to our PDUFA goal date, our commercial team is prepared for the potential U.S. approval of PYRUKYND for thalassemia,” said Brian Goff, Chief Executive Officer, Agios. “In the second quarter, we made progress advancing our early- and mid-stage pipeline and remain on track to deliver topline results of the RISE UP Phase 3 trial for PYRUKYND in sickle cell disease by the end of the year. Collectively, our progress reflects our continued focus on delivering innovative medicines with the potential to transform the lives of those affected by rare diseases and deliver long-term shareholder value.”

Second Quarter 2025 and Recent Corporate Highlights

Commercial Performance – PYRUKYND® (mitapivat)

- Generated $12.5 million in net revenue for the second quarter of 2025, compared to $8.6 million in the second quarter of 2024.
    - 248 unique patients completed prescription enrollment forms, representing an increase of 6 percent over the first quarter of 2025.
    - 142 patients are on PYRUKYND therapy, inclusive of new starts and continued therapy, representing an increase of 4 percent over the first quarter of 2025.
- Entered into a distribution agreement with Avanzanite Bioscience B.V., a rapidly growing European specialty pharmaceutical company focused on rare diseases, to distribute and commercialize PYRUKYND across the European Economic Area, the United Kingdom and Switzerland.

R&D Highlights

- **PYRUKYND (mitapivat)**
    - **Thalassemia** –
        - **Launch preparations underway ahead of U.S. PDUFA goal date of September 7, 2025.** The sNDA for PYRUKYND for the treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia remains under active review by the U.S. Food and Drug Administration (FDA).
        - **Other regulatory applications remain under review by health authorities in Saudi Arabia, United Arab Emirates, and the European Union.**
    - **Sickle Cell Disease –**
        - Topline results from RISE UP Phase 3 trial of mitapivat in sickle cell disease on track by year-end with potential U.S. commercial launch in 2026.
- **Tebapivat**
    - **Sickle Cell Disease –**
        - **Dosed the first patient in the Phase 2 trial investigating tebapivat in sickle cell disease.** The trial is enrolling across three dose cohorts (2.5mg, 5mg, 7.5mg) and placebo and the primary endpoint will measure hemoglobin response, defined as a ≥1g/dL increase in hemoglobin concentration from week 10 to week 12, compared to baseline.
    - **Lower-risk Myelodysplastic Syndromes (LR-MDS)** –

- Continue to progress patient enrollment in the Phase 2b trial for tebapivat in LR-MDS with target enrollment completion by the end of 2025.

- **Early Pipeline**
  - **Investigational New Drug (IND) clearance received for AG-236**, an siRNA targeting TMPRSS6 intended for the treatment of polycythemia vera (PV).

- **Presented new data on mitapivat and tebapivat at the 30th European Hematology Association Congress.** A total of 14 presentations and publications, led by Agios and external collaborators, were shared, covering sickle cell disease, thalassemia, PK deficiency, and MDS.

Second Quarter 2025 Financial Results

For the quarter ended June 30, 2025, net loss was $112.0 million dollars, compared to a net loss of $96.1 million dollars for the second quarter ended June 30, 2024.

- **Net product revenue** from sales of PYRUKYND for the second quarter of 2025 was $12.5 million, compared to $8.6 million for the second quarter of 2024.

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

- **Research and Development (R&D) Expenses** were $91.9 million for the second quarter of 2025, compared to $77.4 million for the second quarter of 2024. The year-over-year increase was primarily attributed to a $10.0 million regulatory milestone payment to Alnylam associated with our agreement to develop and commercialize AG-236, an siRNA targeting TMPRSS6, intended for the treatment of polycythemia vera.

- **Selling, General and Administrative (SG&A) Expenses** were $45.9 million for the second quarter of 2025 compared to $35.5 million for the second quarter of 2024. The year-over-year increase was primarily attributable to an increase in commercial-related activities, including headcount, as the company prepares for the potential approval of PYRUKYND in thalassemia.

- **Cash, cash equivalents and marketable securities** as of June 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 launches in thalassemia and sickle cell disease, advance existing 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 second 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' use of proceeds from the transaction with Royalty Pharma; potential U.S. net sales of vorasidenib and potential future royalty payments; 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)

	June 30, 2025	December 31, 2024
Cash, cash equivalents, and marketable securities	$ 1,339,404	$ 1,532,031
Accounts receivable, net	4,986	4,109
Inventory	30,848	27,616
Total assets	1,471,237	1,663,199
Stockholders' equity	1,369,555	1,540,956

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Revenues:				
Product revenue, net	$ 12,455	$ 8,615	$ 21,181	$ 16,804
Total revenue	12,455	8,615	21,181	16,804
Operating expenses:				
Cost of sales	$ 1,702	$ 1,495	$ 2,787	$ 2,122
Research and development	91,940	77,401	164,683	146,021
Selling, general and administrative	45,869	35,536	87,396	66,550
Total operating expenses	139,511	114,432	254,866	214,693
Loss from operations	(127,056)	(105,817)	(233,685)	(197,889)
Interest income, net	14,513	8,120	30,600	17,009
Other income, net	523	1,579	1,776	3,213
Net loss	$ (112,020)	$ (96,118)	$ (201,309)	$ (177,667)
Net loss per share - basic and diluted	$ (1.93)	$ (1.69)	$ (3.49)	$ (3.14)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	57,932,576	56,802,546	57,697,193	56,593,011

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