Agios Reports First Quarter 2025 Financial Results and Recent Business Highlights

May 1, 2025

– U.S. Regulatory Filing Under Active Review for Approval of PYRUKYND® (mitapivat) in Thalassemia, with PDUFA Goal Date of September 7, 2025 –

– Phase 3 RISE UP Study of Mitapivat in Sickle Cell Disease On Track, with Topline Results Expected in Late 2025; Potential U.S. Commercial Launch in 2026 –

– Tebapivat Advancing in Clinical Trials for Lower-Risk Myelodysplastic Syndromes (LR-MDS) and Sickle Cell Disease –

– PYRUKYND Net Revenue of $8.7 Million in Q1; Cash, Cash Equivalents and Marketable Securities of $1.4 Billion as of March 31, 2025 –

CAMBRIDGE, Mass., May 01, 2025 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a leader in cellular metabolism and pyruvate kinase (PK) activation pioneering therapies for rare diseases, today reported business highlights and financial results for the first quarter ended March 31, 2025.

“We are pleased with our strong start to 2025, highlighted by the acceptance of our sNDA for thalassemia with a PDUFA goal date of September 7, 2025. Our engagement with the FDA is progressing as expected, and we are committed to bringing PYRUKYND to thalassemia patients, irrespective of genotype or transfusion needs,” said Brian Goff, chief executive officer at Agios. “Looking ahead, our focus is also on delivering the topline results from the Phase 3 RISE UP study in sickle cell disease, which remains on track for year-end, and continuing to advance our early and mid-stage clinical programs. Supported by our strong financial position and highly experienced team, we are driving forward PYRUKYND’s multi-billion-dollar potential while building a pipeline designed for lasting impact, with the goal of creating significant value for shareholders and delivering transformative therapies for patients.”

First Quarter 2025 and Recent Highlights

- *PYRUKYND® Revenues:* Generated $8.7 million in net revenue for the first quarter of 2025, compared to $8.2 million in the first quarter of 2024. A total of 234 unique patients have completed prescription enrollment forms, representing an increase of 5 percent over the fourth quarter of 2024. A total of 136 patients are on PYRUKYND therapy, inclusive of new prescriptions and continued therapy, as compared to 130 patients at the end of the fourth quarter 2024.
- *Thalassemia:*
  - The U.S. Food and Drug Administration (FDA) accepted the company’s supplemental New Drug Application (sNDA) for PYRUKYND for the treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia. The Prescription Drug User Fee Act (PDUFA) goal date is September 7, 2025. The FDA has communicated that at this time no advisory committee meeting is planned, and the review is ongoing.
- *Sickle Cell Disease:*
  - The Phase 3 RISE UP study evaluating mitapivat for the treatment of sickle cell disease patients who are 16 years of age or older continued to progress as anticipated. This 52-week Phase 3 study completed enrollment in October 2024, enrolling more than 200 patients worldwide.
  - Advanced final preparations to initiate a Phase 2 clinical trial of tebapivat in patients with sickle cell disease in mid-2025.
- *Pediatric Pyruvate Kinase (PK) Deficiency:*
  - Reported positive topline results from the ACTIVATE-Kids Phase 3 study of mitapivat in children aged 1 to <18 years with PK deficiency who are not regularly transfused.
  - Safety was consistent with the profile for mitapivat previously observed in adults with PK deficiency who are not regularly transfused.
  - ACTIVATE-Kids is the first study to demonstrate efficacy of an oral therapy for children with PK Deficiency who are not regularly transfused.
- *Lower-risk Myelodysplastic Syndromes (LR-MDS):*
  - Progressed patient enrollment in the Phase 2b study of tebapivat in LR-MDS.
- *Corporate:* Krishnan Viswanadhan, Pharm. D, joined Agios as Chief Corporate Development and Strategy Officer, responsible for leading the company’s corporate strategy, business development, and long-term growth initiatives.

Previously, he served as President and Chief Operating Officer of Be Biopharma and at various senior roles at both Bristol Myers Squibb and Celgene.

Key Upcoming Milestones & Priorities

Agios expects to achieve the following key milestones in 2025:

- *Thalassemia:* Receive FDA regulatory decision for PYRUKYND for the treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia (PDUFA goal date is September 7, 2025). Continue progressing the review of regulatory applications with health authorities in the European Union, Kingdom of Saudi Arabia and United Arab Emirates.
- *Sickle Cell Disease:* Announce topline results from the Phase 3 RISE UP study of mitapivat in sickle cell disease in late 2025, with a potential U.S. commercial launch in 2026. Additionally, begin patient enrollment for the Phase 2 study of tebapivat in sickle cell disease in mid-2025.
- *LR-MDS*: Complete patient enrollment in the Phase 2b study of tebapivat for LR-MDS in late 2025.
- *Early-Stage Pipeline:* File an Investigational New Drug Application for AG-236, an siRNA targeting TMPRSS6 intended for the treatment of polycythemia vera, in mid-2025.

First Quarter 2025 Financial Results

Revenue: Net product revenue from sales of PYRUKYND for the first quarter of 2025 was $8.7 million, compared to $8.2 million for the first quarter of 2024.

Cost of Sales: Cost of sales for the first quarter of 2025 was $1.1 million.

Research and Development (R&D) Expenses: R&D expenses were $72.7 million for the first quarter of 2025, compared to $68.6 million for the first quarter of 2024. The year-over-year increase was primarily attributed to an increase in workforce-related expenses and costs associated with clinical trials of tebapivat in LR-MDS and sickle cell disease, partially offset by lower costs associated with the clinical trials of mitapivat in thalassemia and pediatric PKD.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $41.5 million for the first quarter of 2025 compared to $31.0 million for the first 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.

Net Loss: Net loss was $89.3 million for the first quarter of 2025 compared to $81.5 million for the first quarter of 2024.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of March 31, 2025, were $1.4 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 to 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 first 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

Agios is the pioneering leader in PK activation and is dedicated to developing and delivering transformative therapies for patients living with rare diseases. In the U.S., Agios markets a first-in-class pyruvate kinase (PK) activator for adults with PK deficiency, the first disease-modifying therapy for this rare, lifelong, debilitating hemolytic anemia. Building on the company's deep scientific expertise in classical hematology and leadership in the field of cellular metabolism and rare hematologic diseases, Agios is advancing a robust clinical pipeline of investigational medicines with programs in alpha- and beta-thalassemia, sickle cell disease, pediatric PK deficiency, myelodysplastic syndromes (MDS)-associated anemia and phenylketonuria (PKU). In addition to its clinical pipeline, Agios is advancing a preclinical TMPRSS6 siRNA as a potential treatment for polycythemia vera. For more information, please visit the company’s website at www.agios.com.

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)

	March 31, 2025	December 31, 2024
Cash, cash equivalents, and marketable securities	$ 1,424,628	$ 1,532,031
Accounts receivable, net	3,344	4,109
Inventory	29,605	27,616
Total assets	1,555,518	1,663,199
Stockholders' equity	1,466,528	1,540,956

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

	Three Months Ended March 31,	
	2025	2024
Revenues:		
Product revenue, net	$ 8,726	$ 8,189
Total revenue	8,726	8,189
Operating expenses:		
Cost of sales	$ 1,085	$ 627
Research and development	72,743	68,620
Selling, general and administrative	41,527	31,014
Total operating expenses	115,355	100,261
Loss from operations	(106,629)	(92,072)
Interest income, net	16,087	8,889
Other income, net	1,253	1,634
Net loss	$ (89,289)	$ (81,549)
Net loss per share - basic and diluted	$ (1.55)	$ (1.45)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	57,459,195	56,383,475

Contacts:

Investor Contact
Chris Taylor, VP, Investor Relations and Corporate Communications
Agios Pharmaceuticals
IR@agios.com

Media Contact
Eamonn Nolan, Senior Director, Corporate Communications
Agios Pharmaceuticals
media@agios.com

agios

Source: Agios Pharmaceuticals, Inc.