Agios Reports Fourth Quarter and Full Year 2024 Financial Results and Recent Business Highlights

February 13, 2025

– Filed for Regulatory Approval of Mitapivat (PYRUKYND®) for the Treatment of Adult Patients with Non-Transfusion-Dependent and Transfusion-Dependent Alpha- or Beta-Thalassemia in U.S., European Union, Kingdom of Saudi Arabia and United Arab Emirates; PDUFA Goal Date of September 7, 2025 –

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

– Early- and Mid-Stage Pipeline Offers Strong Foundation for Innovation and Growth –

– PYRUKYND Net Revenue of $10.7 Million in Q4; Cash, Cash Equivalents and Marketable Securities of $1.5 Billion as of December 31, 2024 –

CAMBRIDGE, Mass., Feb. 13, 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 fourth quarter and year ended December 31, 2024.

“Agios had a transformative year in 2024, continuing to successfully deliver on all priorities. Our PYRUKYND franchise is poised for multi-billion-dollar potential, driven by the key milestones we achieved last year, including filing for regulatory approval in thalassemia across four markets and completing enrollment in our Phase 3 RISE UP study for sickle cell disease,” said Brian Goff, chief executive officer at Agios. “Backed by a strong balance sheet and a highly experienced team, Agios is focused on maximizing the potential PYRUKYND launches in thalassemia and sickle cell disease in 2025 and 2026, respectively, while advancing and diversifying our key pipeline programs and strategically deploying our capital to drive long-term growth. We are well positioned to bring significant value for shareholders, healthcare professionals and patients, as we build towards a breakout year in 2025.”

Fourth Quarter 2024 and Recent Highlights

- *PYRUKYND® Revenues:* Generated $10.7 million in net revenue for the fourth quarter of 2024, a 20 percent increase from the third quarter of 2024, primarily driven by year-end stocking and adjustments to certain revenue reserves. A total of 223 unique patients have completed prescription enrollment forms, representing an increase of 6 percent over the third quarter of 2024. A total of 130 patients are on PYRUKYND therapy, inclusive of new prescriptions and continued therapy.
- *Thalassemia:*
  - Presented positive results from the ENERGIZE-T Phase 3 randomized clinical trial evaluating mitapivat versus placebo in adults with transfusion-dependent alpha- or beta-thalassemia at the 66th American Society of Hematology Annual Meeting and Exposition (ASH 2024) in December 2024.
  - Based on the favorable benefit-risk profile observed in both the ENERGIZE and ENERGIZE-T Phase 3 studies, filed regulatory applications for mitapivat (PYRUKYND) for the treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia with the U.S., European Union, Kingdom of Saudi Arabia and United Arab Emirates health authorities.
  - 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 review classification for this application is Standard and the Prescription Drug User Fee Act (PDUFA) goal date is September 7, 2025.
- *Sickle Cell Disease:*
  - Completed enrollment of the Phase 3 RISE UP study evaluating mitapivat for the treatment of sickle cell disease patients who are 16 years of age or older. This Phase 3 study enrolled more than 200 patients worldwide.
  - European Commission adopted a positive decision for the designation of mitapivat as an orphan medicinal product for the treatment of sickle cell disease.
  - Presented Phase 1 clinical results on tebapivat (AG-946) in patients with sickle cell disease at ASH 2024.
- *Pediatric Pyruvate Kinase (PK) Deficiency:*
  - Reported in a separate press release today, 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.
- *Lower-risk myelodysplastic syndromes (LR-MDS):*

- Initiated patient enrollment in the Phase 2b study of tebapivat in LR-MDS.
- FDA granted orphan drug designation to tebapivat for the treatment of MDS.

- *Medical Congresses:* Presented 16 abstracts highlighting new data on mitapivat and tebapivat at ASH 2024.
- *Corporate:* David Schenkein, M.D., has informed the company that he will step down from Agios' Board of Directors, effective February 28, 2025, to devote more time to his other commitments. He will continue to serve as a strategic advisor to Agios' Leadership Team, concentrating on advancing the company's clinical development programs.

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).
- *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, a siRNA targeting TMPRSS6 intended for the treatment of polycythemia vera, in mid-2025.

Fourth Quarter 2024 Financial Results

Revenue: Net product revenue from sales of PYRUKYND for the fourth quarter of 2024 was $10.7 million, compared to $7.1 million for the fourth quarter of 2023, and $36.5 million for the year ended December 31, 2024, compared to $26.8 million for the year ended December 31, 2023.

Cost of Sales: Cost of sales for the fourth quarter of 2024 was $1.3 million and $4.2 million for the full year ended December 31, 2024.

Research and Development (R&D) Expenses: R&D expenses were $82.8 million for the fourth quarter of 2024, compared to $77.5 million for the fourth quarter of 2023, and $301.3 million for the full year ended December 31, 2024, compared to $295.5 million for the full year ended December 31, 2023.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $51.7 million for the fourth quarter of 2024 compared to $35.3 million for the fourth quarter of 2023, and $156.8 million for the full year ended December 31, 2024, compared to $119.9 million for the full year ended December 31, 2023. The year-over-year increase was primarily attributable to an increase in commercial-related activities as the company prepares for the potential approval of PYRUKYND in thalassemia.

Net Income (Loss): Net loss was $96.5 million for the fourth quarter of 2024 compared to a net loss of $95.9 million for the fourth quarter of 2023, and net income was $673.7 million for the year ended December 31, 2024, compared to a net loss of $352.1 million for the year ended December 31, 2023.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of December 31, 2024, were $1.5 billion compared to $806.4 million as of December 31, 2023. This increase reflects payments from the royalty monetization for vorasidenib and a milestone payment from Servier for vorasidenib approval received in the third quarter of 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 fourth quarter 2024 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-946), AG-236 and AG-181, Agios' PAH

stabilizer; 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)

	December 31, 2024	December 31, 2023
Cash, cash equivalents, and marketable securities	$ 1,532,031	$ 806,363
Accounts receivable, net	4,109	2,810
Inventory	27,616	19,076
Total assets	1,663,199	937,118
Stockholders' equity	1,540,956	811,019

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

	Years Ended Dec 31,		
	2024	2023	2022
Revenues:			
Product revenue, net	$ 36,498	$ 26,823	$ 11,740
Milestone revenue	—	—	2,500
Total revenue	36,498	26,823	14,240
Operating expenses			
Cost of sales	$ 4,165	$ 2,881	$ 1,704
Research and development	301,286	295,526	279,910
Selling, general and administrative	156,784	119,903	121,673
Total operating expenses	462,235	418,310	403,287
Loss from operations	(425,737)	(391,487)	(389,047)
Gain on sale of contingent payments	889,136	—	127,853
Milestone payment from gain on sale of oncology business	200,000	—	—
Royalty income from gain on sale of oncology business	—	—	9,851
Interest income, net	48,083	33,344	12,793
Other income, net	6,487	6,055	6,749
Net income (loss) before taxes	717,969	(352,088)	(231,801)
Income tax expense	44,244	—	—
Net income (loss)	$ 673,725	$ (352,088)	$ (231,801)
Net income (loss) per share - basic	$ 11.86	$ (6.33)	$ (4.23)
Net income (loss) per share - diluted	$ 11.64	$ (6.33)	$ (4.23)
Weighted-average number of common shares used in computing net income (loss) per share – basic	56,807,415	55,651,487	54,789,435

Weighted-average number of common shares used in computing net income (loss) per share – diluted	57,889,255	55,651,487	54,789,435

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

Source: Agios Pharmaceuticals, Inc.