Agios Reports Fourth Quarter and Full Year 2023 Financial Results and Recent Business Highlights

February 15, 2024

– Announced Positive Results from the Phase 3 ENERGIZE Study of Mitapivat in Adults with Non-Transfusion-Dependent Alpha- or Beta-Thalassemia –

– Presented Positive Results from Phase 2 Portion of the RISE UP Pivotal Study in Sickle Cell Disease at 65th ASH Annual Meeting and Exposition –

– Two Additional Phase 3 Readouts of Mitapivat Expected in 2024, with a Total of Four Phase 3 Readouts by the End of 2025 –

– U.S. PYRUKYND® (mitapivat) Net Revenue of $7.1 Million in Q4; Cash, Cash Equivalents and Marketable Securities of $806.4 Million as of December 31, 2023 –

CAMBRIDGE, Mass., Feb. 15, 2024 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a leader in the field of cellular metabolism pioneering therapies for rare diseases, today reported business highlights and financial results for the fourth quarter and year ended December 31, 2023.

"The past 12 months have been remarkable for Agios. We reported positive data across our industry-leading pipeline of PK activators, including Phase 3 data in non-transfusion-dependent thalassemia, Phase 2 data in sickle cell disease and clinical proof-of-concept in lower-risk MDS, and expanded our preclinical pipeline by in-licensing a novel siRNA program from Alnylam," said Brian Goff, chief executive officer at Agios. "In 2024, we expect two additional Phase 3 readouts, including the Phase 3 study of mitapivat in transfusion-dependent thalassemia, and are actively preparing for a potential U.S. launch in thalassemia in 2025. Together with our strong cash position, Agios is poised for significant near- and long-term growth as we progress toward of our vision of becoming a leading rare disease company."

Fourth Quarter 2023 & Recent Highlights

- *PYRUKYND® U.S. Launch:* Generated $7.1 million in U.S. net revenue for the fourth quarter of 2023, a 4 percent decrease from the third quarter of 2023, primarily driven by lower customer inventory levels at the end of the fourth quarter of 2023, partially offset by favorable gross-to-net adjustments. A total of 178 unique patients have completed prescription enrollment forms, representing an increase of 11 percent over the third quarter of 2023. A total of 109 patients are on PYRUKYND® therapy, a 9 percent increase from the third quarter of 2023.
- *Thalassemia:* Announced positive topline data from the Phase 3 ENERGIZE study of mitapivat in non-transfusion-dependent thalassemia. The study achieved its primary endpoint of hemoglobin response and achieved both key secondary endpoints associated with change from baseline in FACIT-Fatigue Score and hemoglobin concentration.
- *Sickle Cell Disease:* Presented positive results from the Phase 2 portion of the RISE UP pivotal study of mitapivat at the 65th American Society of Hematology (ASH) Annual Meeting and Exposition. The study achieved its primary endpoint of hemoglobin response and an improvement in annualized rates of sickle cell pain crises was observed.
- *Lower-risk Myelodysplastic Syndromes:* Announced clinical proof-of-concept data in the open-label Phase 2a study of AG-946 for the treatment of anemia in lower-risk myelodysplastic syndromes (LR-MDS).
- *Earlier-stage Pipeline:* Filed an Investigational New Drug Application (IND) for AG-181, Agios' PAH stabilizer for the treatment of phenylketonuria (PKU).
- *Data presentations:* Presented broad set of clinical and translational data at the 65th ASH Annual Meeting & Exposition, including positive data from the Phase 2 portion of the RISE UP study of mitapivat in sickle cell disease, as noted above.

Anticipated 2024 Milestones

- *Thalassemia:* Following the announcement of positive topline data from the Phase 3 ENERGIZE study of mitapivat in non-transfusion-dependent thalassemia in January 2024, Agios plans to report topline data from the Phase 3 ENERGIZE-T study of mitapivat in transfusion-dependent thalassemia (mid-year) and file for FDA approval of mitapivat in thalassemia (year-end)
- *Sickle Cell Disease:* Complete enrollment in the Phase 3 portion of the RISE UP study of mitapivat (year-end)
- *Pediatric PK Deficiency:* Complete enrollment in the Phase 3 ACTIVATE-kids study of mitapivat (mid-year). Report topline data from Phase 3 ACTIVATE kids-T study (year-end)
- *Lower-risk Myelodysplastic Syndromes:* Dose first patient in Phase 2b study of AG-946 (mid-year)
- *Earlier-stage Pipeline:* Dose the first patient in the Phase 1 study of AG-181 for the treatment of PKU (early 2024)

Fourth Quarter and Full Year 2023 Financial Results

Revenue: Net U.S. product revenue from sales of PYRUKYND® was $7.1 million for the fourth quarter of 2023 compared to $4.3 million for the fourth quarter of 2022, and $26.8 million for the year ended Dec. 31, 2023 compared to $11.7 million for the year ended Dec. 31, 2022.

Cost of Sales: Cost of sales was $0.6 million for the fourth quarter of 2023 and $2.9 million for the full year ended Dec. 31, 2023.

Research and Development (R&D) Expenses: R&D expenses were $77.5 million for the fourth quarter of 2023 compared to $70.3 million for the fourth quarter of 2022, and $295.5 million for the year ended Dec. 31, 2023 compared to $279.9 million for the year ended Dec. 31, 2022. These changes reflect an increase in development costs for mitapivat and the up-front payment associated with the license agreement with Alnylam, partially offset by a reduction in expenses associated with the evolution of our research organization and the sale of our oncology business to Servier.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $35.3 million for the fourth quarter of 2023 compared to $32.8 million for the fourth quarter of 2022, and $119.9 million for the year ended Dec. 31, 2023 compared to $121.7 million for the year ended Dec. 31, 2022.

Net Income (Loss): Net loss was $95.9 million for the fourth quarter of 2023 compared to a net income of $36.5 million for the fourth quarter of 2022, and net loss was $352.1 million for the year ended Dec. 31, 2023 compared to $231.8 million for the year ended Dec. 31, 2022. The increase in net loss is due to the $127.9 million sale to Sagard in the fourth quarter of 2022 of our rights to future contingent payments associated with royalties on U.S. net sales of TIBSOVO®.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of Dec. 31, 2023, were $806.4 million compared to $1.1 billion as of Dec. 31, 2022. Agios expects that its cash, cash equivalents and marketable securities together with anticipated product revenue, interest income and vorasidenib milestone will enable the company to fund its operating expenses and capital expenditures at least into 2026. This does not include cash inflows which could extend runway beyond 2026 including potential royalties or monetization of royalties from vorasidenib, commercializing mitapivat outside of the U.S. through one or more partnerships, or other potential strategic business or financial agreements.

Conference Call Information
Agios will host a conference call and live webcast with slides today at 8:00 a.m. ET to discuss fourth quarter and full year 2023 financial results and recent business highlights. The live webcast can be accessed under “Events & Presentations” in the Investors section of the company’s website at www.agios.com. The archived webcast will be available on the company's website beginning 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, 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), AG-946, TMPRSS6 siRNA and AG-181, Agios’ PAH stabilizer; Agios’ plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®, AG-946 and AG-181, its PAH stabilizer; Agios’ strategic vision and goals, including its key milestones for 2024; 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 milestone or royalty payments related to the sale of its oncology business or its in-licensing of TMPRSS6 siRNA, 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, 2023	December 31, 2022
Cash, cash equivalents, and marketable securities	$ 806,363	$ 1,096,993
Accounts receivable, net	2,810	2,206
Inventory	19,076	8,492

Total assets	937,118	1,238,718
Stockholders' equity	811,019	1,100,814

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

	Years Ended December 31,		
	2023	**2022**	**2021**
Revenues:			
Product revenue, net	$ 26,823	$ 11,740	$ —
Milestone revenue	—	2,500	—
Total revenue	26,823	14,240	—
Operating expenses			
Cost of sales	$ 2,881	$ 1,704	$ —
Research and development	295,526	279,910	256,973
Selling, general and administrative	119,903	121,673	121,445
Total operating expenses	418,310	403,287	378,418
Loss from operations	(391,487)	(389,047)	(378,418)
Gain on sale of contingent payments	—	127,853	—
Royalty income from gain on sale of oncology business	—	9,851	6,639
Interest income, net	33,344	12793	836
Other income, net	6,055	6,749	14,433
Net loss from continuing operations	(352,088)	(231,801)	(356,510)
Net income from discontinued operations, net of tax	—	—	1,961,225
Net (loss) income	$ (352,088)	$ (231,801)	$ 1,604,715
Net loss from continuing operations per share - basic and diluted	$ (6.33)	$ (4.23)	$ (5.90)
Net income from discontinued operations per share - basic and diluted	$ —	$ —	$ 32.45
Net (loss) income per share - basic and diluted	$ (6.33)	$ (4.23)	$ 26.55
Weighted-average number of common shares used in computing net loss per share from continuing operations, net income per share from discontinued operations and net (loss) income per share – basic and diluted	55,651,487	54,789,435	60,447,346

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Source: Agios Pharmaceuticals, Inc.