Agios Reports Fourth Quarter and Full Year 2022 Financial Results

February 23, 2023

– Completed Enrollment in Phase 2 RISE UP Study of PYRUKYND® (mitapivat) in Sickle Cell Disease –

– On Track to Complete Enrollment in Phase 3 ENERGIZE and ENERGIZE-T Studies of PYRUKYND® in Thalassemia by Mid-2023 –

– U.S. PYRUKYND® Launch Provides Capability Building Platform to Support Expected Future Product Growth and Expansion; Net Revenue of $4.3 Million in Q4 –

– $1.1 Billion of Cash, Cash Equivalents and Marketable Securities as of Dec. 31, 2022 –

CAMBRIDGE, Mass., Feb. 23, 2023 (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 Dec. 31, 2022.

"Over the past year, Agios has made significant progress toward our vision of transforming the lives of patients with rare diseases as we build a hematology franchise focused on diseases that share a common underlying pathophysiology, limited treatment options and profound unmet need," said Brian Goff, chief executive officer at Agios. "In 2022, we received regulatory approvals in the U.S., EU and Great Britain for PYRUKYND® as the first and only disease-modifying treatment for adults with pyruvate kinase (PK) deficiency. We achieved our ambitious enrollment targets for our pivotal trials in thalassemia and sickle cell disease. We strengthened our company leadership with the appointments of new management team and Board members with deep expertise in rare diseases and global commercial strategy. We are poised for significant near- and long-term growth and look forward to a productive 2023, anticipating the readout of our Phase 2 sickle cell disease study and the completion of enrollment in our Phase 3 thalassemia studies, driving toward two additional PYRUKYND® indications by 2026."

Fourth Quarter 2022 & Recent Highlights

- *PYRUKYND® U.S. Launch:* Continued to execute launch, generating $4.3 million in U.S. net revenue for the fourth quarter of 2022, the third full quarter following FDA approval. A total of 105 unique patients have completed prescription enrollment forms, representing an increase of 25 percent over the third quarter. A total of 78 patients are on PYRUKYND® therapy, representing a 39 percent increase over the third quarter.
- *PYRUKYND® Global Approvals:* Received marketing authorization for adults with PK deficiency in the EU and Great Britain.
- *Sickle Cell Disease:* Completed enrollment in the Phase 2 portion of the RISE UP study of PYRUKYND® in adults with sickle cell disease.
- *Thalassemia:* Enrolled more than half of patients in the Phase 3 ENERGIZE and ENERGIZE-T studies of PYRUKYND® in not regularly transfused and regularly transfused adults with thalassemia, respectively.
- *Data Presentations:* Presented broad set of clinical and translational data at the 64th American Society of Hematology (ASH) Annual Meeting & Exposition, including long-term PYRUKYND® data in adults with non-transfusion-dependent thalassemia and in adults with PK deficiency.
- *Leadership:* Appointed Tsveta Milanova to the role of chief commercial officer, bringing two decades of experience in rare disease commercial strategy and global market access.

Anticipated 2023 Milestones

- *Thalassemia:* Complete enrollment of the Phase 3 ENERGIZE and ENERGIZE-T studies of PYRUKYND® by mid-year.
- *Sickle Cell Disease:* Announce data readout from the Phase 2 portion of the RISE UP study of PYRUKYND® and go/no-go to Phase 3 decision by mid-year.
- *Pediatric PK Deficiency:* Enroll more than half of patients in the Phase 3 ACTIVATE-kids and ACTIVATE-kidsT studies of PYRUKYND® by year-end.
- *Lower-risk Myelodysplastic Syndromes (LR-MDS):* Complete enrollment of the Phase 2a study of novel PK activator AG-946 by year-end.
- *Pipeline:* File investigational new drug (IND) application for phenylalanine hydroxylase (PAH) stabilizer for the treatment of phenylketonuria (PKU) by year-end.

Fourth Quarter 2022 Financial Results

The financial results discussion compares Agios' continuing operations. All periods have been adjusted to exclude discontinued operations related to the divested oncology business.

Revenue: Net U.S. product revenue from sales of PYRUKYND® was $4.3 million for the fourth quarter of 2022, and $11.7 million for the full year

ended Dec. 31, 2022. PYRUKYND® was approved by the FDA on February 17, 2022.

Cost of Sales: Cost of sales was $0.4 million for the fourth quarter of 2022 and $1.7 million for the full year ended Dec. 31, 2022.

Non-Operating Income: Non-operating income included $127.9 million as gain on sale of contingent payments from the sale of TIBSOVO® royalty rights to Sagard Healthcare Partners. Non-operating income also included approximately $9.9 million from TIBSOVO® royalties for the full year ended Dec. 31, 2022, with royalty income ceasing after the third quarter of 2022 due to the sale of these rights to Sagard.

Research and Development (R&D) Expenses: R&D expenses were $70.3 million for the fourth quarter of 2022 compared to $73.3 million for the fourth quarter of 2021, and $279.9 million for the year ended Dec. 31, 2022 compared to $257.0 million for the year ended Dec. 31, 2021. The year-over-year increase in R&D expense was primarily driven by increased costs for PYRUKYND® and AG-946 studies and increased workforce spend across R&D.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses were $32.8 million for the fourth quarter of 2022 compared to $31.5 million for the fourth quarter of 2021, and $121.7 million for the year ended Dec. 31, 2022 compared to $121.4 million for the year ended Dec. 31, 2021.

Net Income (Loss) from Continuing Operations: Net income was $36.5 million for the fourth quarter of 2022 compared to a net loss of $98.6 million for the fourth quarter of 2021, and net loss was $231.8 million for the year ended Dec. 31, 2022 compared to $356.5 million for the year ended Dec. 31, 2021.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of Dec. 31, 2022, were $1.1 billion compared to $1.3 billion as of Dec. 31, 2021. This cash position includes the receipt of a one-time payment of $131.8 million associated with the sale of our rights to 5% royalties on U.S. net sales of Servier's TIBSOVO ®. Agios expects that its cash, cash equivalents and marketable securities together with anticipated product revenue and interest income will enable the company to execute its operating plan, including funding the currently planned development programs for mitapivat, AG-946 and PAH stabilization and commercializing mitapivat outside of the U.S. through one or more partnerships, to cash-flow positivity without the need to raise additional equity.

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 year end 2022 financial results and recent business activities. 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 a biopharmaceutical company that is fueled by connections. The Agios team cultivates strong bonds with patient communities, healthcare professionals, partners and colleagues to discover, develop and deliver therapies for 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 leadership in the field of cellular metabolism, Agios is advancing a robust clinical pipeline of investigational medicines with programs in alpha- and beta-thalassemia, sickle cell disease, pediatric PK deficiency and MDS-associated anemia. In addition to its clinical pipeline, Agios has a PAH stabilizer in preclinical development as a potential treatment for phenylketonuria (PKU) and deep scientific expertise in classical hematology. 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 and its PAH stabilizer; Agios' plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®, AG-946 and its PAH stabilizer; Agios' strategic vision and goals, including its key milestones for 2023 and potential catalysts through 2026; 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 the COVID-19 pandemic 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 maintain key collaborations; the failure of Agios to receive milestone or royalty payments related to the sale of its oncology business, the uncertainty of the timing of any receipt of any such payments, and the uncertainty of the results and effectiveness of the use of proceeds from the transaction with Servier; 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, 2022	December 31, 2021
Cash, cash equivalents, and marketable securities	$ 1,096,993	$ 1,286,393
Accounts receivable, net	2,206	—
Inventory	8,492	—
Total assets	1,238,718	1,437,736
Stockholders' equity	1,100,814	1,291,975

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

	Years Ended December 31,		
	2022	2021	2020
Revenues:			
Product revenue, net	$ 11,740	$ —	$ —
Milestone revenue	2,500	—	—
Total revenue	14,240	—	—
Operating expenses			
Cost of sales	$ 1,704	$ —	$ —
Research and development	279,910	256,973	220,811
Selling, general and administrative	121,673	121,445	115,105
Total operating expenses	403,287	378,418	335,916
Loss from operations	(389,047)	(378,418)	(335,916)
Gain on sale of contingent payments	127,853	—	—
Royalty income from gain on sale of oncology business	9,851	6,639	—
Interest income, net	12,793	836	6,611
Other income, net	6,749	14,433	—
Net loss from continuing operations	(231,801)	(356,510)	(329,305)
Net income from discontinued operations, net of tax	—	1,961,225	1,935
Net (loss) income	$ (231,801)	$ 1,604,715	$ (327,370)
Net loss from continuing operations per share - basic and diluted	$ (4.23)	$ (5.90)	$ (4.77)
Net income from discontinued operations per share - basic and diluted	$ —	$ 32.45	$ 0.03
Net (loss) income per share - basic and diluted	$ (4.23)	$ 26.55	$ (4.74)
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	54,789,435	60,447,346	68,997,879

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