Agios Reports Fourth Quarter and Full Year 2021 Financial Results

February 24, 2022

– Received FDA Approval of PYRUKYND® (mitapivat) for the Treatment of Hemolytic Anemia in Adults with Pyruvate Kinase (PK) Deficiency; First Approved Therapy for this Rare Blood Disorder –

– First Patients Dosed in All Three Pivotal Trials of PYRUKYND® in Thalassemia and Sickle Cell Disease –

– Cash, Cash Equivalents and Marketable Securities $1.3 Billion as of Dec. 31; Expects Strong Cash Position to Enable Execution of Robust Operating Plan to Cash-Flow Positivity –

CAMBRIDGE, Mass., Feb. 24, 2022 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism pioneering therapies for genetically defined diseases, today reported business highlights and financial results for the fourth quarter and year ended Dec. 31, 2021.

"With last week's FDA approval of PYRUKYND ® for the treatment of hemolytic anemia in adults with PK deficiency, we have delivered the first therapy for a rare, debilitating, lifelong disease – and we have set the stage for Agios' next chapter as a leader in the genetically defined disease space," said Jackie Fouse, Ph.D., chief executive officer at Agios. "We have made incredible progress over the past year, following our transformational decision to divest our oncology business and accelerate and expand our genetically defined disease portfolio. We are poised to expand our impact to thalassemia, sickle cell disease, myelodysplastic syndrome and beyond, and I am optimistic about the future we are building together with patient communities, our clinical and research collaborators and our dedicated Agios team."

Fourth Quarter 2021 & Recent Highlights

- Received approval from the U.S. Food and Drug Administration (FDA) for PYRUKYND®, the first therapy for the treatment of hemolytic anemia in adults with PK deficiency and Agios' first genetically defined disease medicine.
- Dosed first patients in all three pivotal trials of PYRUKYND® in thalassemia and sickle cell disease.
- Hosted investor day on Nov. 17 to share updates on the company's research and development pipeline, including progress on the PKM2, BCAT2 and PAH programs, and provide insights into the commercial launch strategy.
- Presented the following key data at the 63rd American Society of Hematology (ASH) Annual Meeting & Exposition:
    - Long-term efficacy data of PYRUKYND® in adults with PK deficiency who participated in the Phase 3 ACTIVATE and ACTIVATE-T trials
    - Long-term efficacy and safety data of PYRUKYND® in adults with thalassemia who do not receive regular transfusions
    - Efficacy, safety and translational data of PYRUKYND® in sickle cell disease from ongoing collaborator-led studies
    - Phase 1 healthy volunteer study data of AG-946, the company's novel PK activator

Anticipated 2022 Key Milestones & Priorities

Agios expects to execute on the following key milestones and priorities in 2022:

Pyruvate Kinase (PK) Deficiency

- Receive European Medicines Agency (EMA) regulatory decision for PYRUKYND® in adults with PK deficiency by year-end.
- Initiate Phase 3 ACTIVATE-kids and ACTIVATE-kidsT studies of PYRUKYND® in not regularly transfused and regularly transfused pediatric patients with PK deficiency, respectively, in mid-2022.

Thalassemia

- Enroll a meaningful portion of patients in the Phase 3 ENERGIZE and ENERGIZE-T studies of PYRUKYND® in not regularly transfused and regularly transfused adults with thalassemia, respectively, by year-end.

Sickle Cell Disease

- Complete enrollment in the Phase 2 portion of the RISE UP study of PYRUKYND® in sickle cell disease by year-end.
- Initiate the sickle cell disease cohort of the Phase 1 study of novel PK activator AG-946 in the first half of 2022.

Expansion and Acceleration of PK Activation Portfolio

- Initiate Phase 2a study of AG-946 in adults with low- to intermediate-risk myelodysplastic syndrome (MDS) by year-end.
- Continue to publish clinical and translational data supporting the utility of PK activators across key disease areas and elucidating the burden of disease for PK deficiency, thalassemia and sickle cell disease.

Fourth Quarter and Full Year 2021 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.

Research and Development (R&D) Expenses: R&D expenses for continuing operations were $73.3 million for the fourth quarter of 2021 compared to $59.4 million for the fourth quarter of 2020, and $257 million for the year ended Dec. 31, 2021 compared to $220.8 million for the year ended Dec. 31, 2020. This year-over-year increase was largely driven by start-up costs for PYRUKYND® pivotal studies, including ENERGIZE, ENERGIZE-T and RISE UP, offset by closeouts of ACTIVATE and ACTIVATE-T studies; regulatory filings for PYRUKYND® in the U.S. and EU; and increased workforce spend across R&D.

Selling, General and Administrative (SG&A) Expenses: SG&A expenses for continuing operations were $31.5 million for the fourth quarter of 2021 compared to $25.9 million for the fourth quarter of 2020, and $121.4 million for the year ended Dec. 31, 2021 compared to $115.1 million for the year ended Dec. 31, 2020. This year-over-year increase was driven primarily by PYRUKYND® launch preparations and disease education, including field sales build, training and marketing.

Non-Operating Income: Non-operating income included approximately $2.6 million from TIBSOVO® (ivosidenib) royalties for the fourth quarter of 2021, and $6.6 million for the year ended Dec. 31, 2021.

Net Loss: Net loss was $98.6 million for the fourth quarter of 2021 compared to a net loss of $84.5 million for the fourth quarter of 2020, and $356.5 million for the year ended Dec. 31, 2021 compared to $329.3 million for the year ended Dec. 31, 2020.

Cash Position and Guidance: Cash, cash equivalents and marketable securities as of Dec. 31, 2021, were $1.3 billion compared to $670.5 million as of Dec. 31, 2020. The company expects that its cash, cash equivalents and marketable securities will enable the company to execute its operating plan through major catalysts and 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 2021 financial results and recent business activities. To participate in the conference call, please dial 1-877-377-7098 (domestic) or 1-631-291-4547 (international) and refer to conference ID 9873227. 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 genetically defined 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 active and planned programs in alpha- and beta-thalassemia, sickle cell disease, pediatric PK deficiency and MDS-associated anemia. In addition to its clinical pipeline, Agios has multiple investigational therapies in preclinical development and an industry-leading research team with unmatched expertise in cellular metabolism and genetics. 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 Agios' plans, strategies and expectations for the preclinical, clinical and commercial advancement of its drug development programs, including PYRUKYND® (mitapivat) and AG-946; the potential benefits of Agios' products and product candidates; Agios' key milestones and guidance for 2022; its financial guidance regarding the period in which it will have capital available to fund its operations; 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. 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 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; the impact of the COVID-19 pandemic on 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 future approved products, and launching, marketing and selling 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, including with respect to the regulatory submissions for PYRUKYND® (mitapivat), 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 and 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 collaborations; 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, or SEC, including the risks and uncertainties set forth under the heading Risk Factors in our filings with the SEC. While the list of factors presented here is considered representative, this list should not be considered to be a complete statement of all potential risks and uncertainties. Any forward-looking statements contained in this press release are made only as of the date hereof, and we undertake no obligation to update forward-looking statements to reflect developments or information obtained after the date hereof and disclaim any obligation to do so other than as may be required by law.

Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	December 31,	
	2021	2020
Cash, cash equivalents and marketable securities	$ 1,286,393	$ 670,537
Assets held for discontinued operations	-	50,460
Total assets	1,437,736	852,952
Liabilities held for discontinued operations	-	299,728
Stockholders' equity	1,291,975	399,500

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

	Three Months Ended December 31,		Years Ended December 31,	
(In thousands, except share and per share data)	2021	2020	2021	2020
Cost and expenses:				
Research and development	$ 73,299	$ 59,423	$ 256,973	$ 220,811
Selling, general and administrative	31,528	25,909	121,445	115,105
Total cost and expenses	104,827	85,332	378,418	335,916
Loss from operations	(104,827)	(85,332)	(378,418)	(335,916)
Gain on sale of oncology business	2,643	-	6,639	-
Interest income, net	332	791	836	6,611
Other income, net	3,268	-	14,433	-
Net loss from continuing operations	(98,584)	(84,541)	(356,510)	(329,305)
Net income (loss) from discontinued operations, net of tax	3,957	(13,116)	1,961,225	1,935
Net income (loss)	$ (94,627)	$ (97,657)	$ 1,604,715	$ (327,370)
Net loss from continuing operations per share - basic and diluted	$ (1.81)	$ (1.22)	$ (5.90)	$ (4.77)
Net income (loss) from discontinued operations per share - basic and diluted	$ 0.07	$ 0.19	$ 32.45	$ 0.03
Net income (loss) per share - basic and diluted	$ (1.74)	$ (1.41)	$ 26.55	$ (4.74)
Weighted-average number of common shares used in computing net loss per share from continuing operations, net income (loss) per share from discontinued operations and net income (loss) per share – basic and diluted	54,335,230	69,271,163	60,447,346	68,997,879

Contacts

Investors:
Holly Manning, 617-844-6630
Senior Director, Investor Relations
Holly.Manning@agios.com

Media:
Jessica Rennekamp, 857-209-3286
Director, Corporate Communications
Jessica.Rennekamp@agios.com

Source: Agios Pharmaceuticals, Inc.