Agios Highlights 2022 Anticipated Milestones and Priorities to Drive Company's Strategic Vision in Genetically Defined Diseases

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– Company Expects to Receive FDA Regulatory Decision for Mitapivat as a Potential Treatment for Adults with PK Deficiency in February –

– Five Pivotal Clinical Trials are Planned and Ongoing in Thalassemia, Sickle Cell Disease and Pediatric PK Deficiency –

– Agios is Expanding Clinical Portfolio with Trials of Novel PK Activator AG-946 –

– Strong Cash Position Expected to Enable Execution of Robust Operating Plan to Cash-Flow Positivity –

CAMBRIDGE, Mass., Jan. 10, 2022 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism pioneering therapies for genetically defined diseases, today announced its anticipated 2022 key milestones and priorities that will drive its transition to a commercial-stage genetically defined disease company and the expansion of its robust clinical and research portfolio. Agios will present at the virtual 40th Annual J.P. Morgan Healthcare Conference on Wednesday, January 12 at 7:30 a.m. ET, and a live webcast will be available at investor.agios.com.

"We are beginning an exciting and defining year for Agios as we prepare for our first genetically defined disease product launch, execute on five pivotal clinical trials, continue to expand our PK activation clinical portfolio and foster our innovative research engine," said Jackie Fouse, Ph.D., chief executive officer of Agios. "We are already making strong progress with mitapivat being evaluated under FDA Priority Review in the U.S. as a potential treatment for adults with PK deficiency and executing our Phase 3 ENERGIZE and ENERGIZE-T studies in thalassemia and Phase 2/3 RISE UP study in sickle cell disease. In 2022, we expect to further drive our PK activation expansion strategy by initiating our pivotal trials in pediatric PK deficiency, completing enrollment in the Phase 2 portion of the RISE UP study and advancing our novel PK activator AG-946 in sickle cell disease and low to intermediate risk myelodysplastic syndrome. At Agios, we are fueled by connections – with patient communities, a world-class network of healthcare providers and researchers, and each other – and these collaborative partnerships will drive our key priorities and advance care for people with genetically defined diseases."

Anticipated 2022 Key Milestones & Priorities

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

Pyruvate Kinase (PK) Deficiency

- Receive U.S. Food and Drug Administration (FDA) regulatory decision for mitapivat in adults with PK deficiency in Q1
- Receive European Medicines Agency (EMA) regulatory decision for mitapivat in adults with PK deficiency by year-end
- Initiate Phase 3 ACTIVATE-kids and ACTIVATE-kidsT studies of mitapivat 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 mitapivat 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 mitapivat 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

Agios Five-Year Strategic Vision

The Agios five-year strategic vision reflects the company's expected evolution as a leader in developing genetically defined disease therapies based on its scientific expertise in cellular metabolism. As part of this vision, Agios expects to achieve the following milestones by the end of 2026:

- Receive regulatory approvals globally for mitapivat in three initial indications: adult PK deficiency, thalassemia and sickle cell disease
- Advance a broad clinical pipeline of at least 5 molecules exploring at least 10 indications
- Foster a robust research pipeline poised to deliver an investigational new drug (IND) every 12-24 months
- Achieve cash-flow positivity

Presentation at 40th Annual J.P. Morgan Healthcare Conference

Agios will webcast its corporate presentation from the virtual 40th Annual J.P. Morgan Healthcare Conference on Wednesday, January 12 at 7:30 a.m. ET. A live webcast of the presentation can be accessed under “Events & Presentations” in the Investors section of the company’s website at agios.com. A replay of the webcast will be archived on the Agios website for at least two weeks following the presentation.

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. Building on the company's leadership in the field of cellular metabolism, Agios is advancing a robust pipeline of investigational medicines, including a first-in-class pyruvate kinase (PK) activator that is being evaluated for the treatment of three distinct hemolytic anemias – pyruvate kinase deficiency, alpha- and beta-thalassemia and sickle cell disease. In addition to its active late-stage clinical pipeline, Agios has multiple novel investigational therapies in clinical and preclinical development. 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 mitapivat and AG-946; the potential benefits of Agios’ products and product candidates; Agios’ key milestones and guidance for 2022 and its strategic vision for 2026; 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 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 communication 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.

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