Agios Highlights 2021 Milestones to Accelerate and Expand Its Genetically Defined Disease Portfolio and Drive Near- and Long-Term Value Creation

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– Company Expects to File for Regulatory Approval for Mitapivat for the Treatment of Adults with Pyruvate Kinase (PK) Deficiency in the U.S. in Q2 2021 and in the EU in Mid-2021 –

– Pivotal Trials for Mitapivat in Thalassemia on Track for Initiation in 2H 2021 and in Sickle Cell Disease by Year-End –

– Sale of Oncology Portfolio to Servier Expected to Close in Q2 2021, Enabling Agios to Focus on Genetically Defined Diseases –

– Company's 2025 Strategic Vision Includes Commercialization of Mitapivat in 3 Indications, 5+ Molecules in Clinical Development across 10+ Indications, an Investigational New Drug Every 12-24 Months and a Cash-Flow Positive Business –

CAMBRIDGE, Mass., Jan. 11, 2021 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism to treat cancer and genetically defined diseases, today announced its key 2021 milestones that will drive its recently announced strategic pivot to focus on developing and commercializing innovative treatments for genetically defined diseases, as well as its five-year vision for the company. Agios will present at the virtual 39th Annual J.P. Morgan Healthcare Conference on Monday, January 11 at 10:50 a.m. ET, and a live webcast will be available at investor.agios.com.

"Agios is at an exciting inflection point as we prepare to move forward with a singular focus on genetically defined diseases," said Jackie Fouse, Ph.D., chief executive officer of Agios. "2021 will be a year of significant momentum and further evidence of our potential to meaningfully impact the lives of patients with unmet needs in pyruvate kinase deficiency, thalassemia, sickle cell disease and other genetically defined diseases. This year, we expect to file for the approval of mitapivat in pyruvate kinase deficiency, which currently has no disease-modifying treatment options, and to initiate pivotal development programs in thalassemia and sickle cell disease. We also expect to further unlock the potential of PK activation across a range of genetically defined diseases by advancing our rich and sustainable research pipeline. As we reimagine the future of Agios, we look forward to building on our core values and pioneering leadership in cellular metabolism to expand and accelerate our work on behalf of patients."

Anticipated 2021 Key Milestones

Agios expects to achieve the following key milestones in 2021:

Corporate

- Complete sale of oncology portfolio to Servier, in a transaction worth up to $2 billion plus royalties, in the second quarter of 2021 subject to shareholder approval and satisfaction of regulatory conditions, and commence return of at least $1.2 billion to shareholders post-closing

Genetically Defined Disease Program Milestones

- File for regulatory approval for mitapivat in adults with PK deficiency: submit new drug application (NDA) in the U.S. in the second quarter of 2021 and marketing authorization application (MAA) in the EU in mid-2021
- Initiate two Phase 3 studies of mitapivat, ENERGIZE and ENERGIZE-T, in not regularly transfused and regularly transfused adults with thalassemia in the second half of 2021
- Announce pivotal development plan for mitapivat in adults with sickle cell disease in the first half of 2021 and initiate pivotal development program by year-end
- Prioritize new indications for pyruvate kinase R (PKR) and pyruvate kinase M2 (PKM2) activator clinical development by year-end

Genetically Defined Disease Data Presentations

- Report topline data from the Phase 3 ACTIVATE-T study of mitapivat in adults with PK deficiency who receive regular transfusions in the first quarter of 2021
- Submit data from the following clinical studies for presentation at the European Hematology Association (EHA) Virtual Congress, which will be held June 9-17, 2021:
    - Phase 3 ACTIVATE study of mitapivat in adults with PK deficiency who do not receive regular transfusions
    - Phase 3 ACTIVATE-T study of mitapivat in adults with PK deficiency who receive regular transfusions
    - Phase 2 study of mitapivat in adults with α- and β-thalassemia who do not receive regular transfusions
- Submit data from ongoing clinical studies of mitapivat in sickle cell disease for presentation at medical meetings throughout 2021
- Present data from the single ascending dose (SAD) and multiple ascending dose (MAD) cohorts of the Phase 1 study of AG-946, the company's next-generation PKR activator, in healthy volunteers by year-end

Oncology Milestones & Data Presentations

- Present mature overall survival data from the Phase 3 ClarIDHy study of TIBSOVO® (ivosidenib tablets) in patients with previously treated IDH1-mutant cholangiocarcinoma at the American Society of Clinical Oncology Gastrointestinal Cancers Symposium (ASCO-GI), which will be held virtually January 15-17, 2021
- Submit supplemental new drug application (sNDA) in the U.S. for TIBSOVO® in patients with previously treated IDH1-mutant cholangiocarcinoma in the first quarter of 2021
- Enrollment in the Phase 3 AGILE trial of TIBSOVO® in combination with azacitidine in adult patients with previously untreated IDH1-mutant acute myeloid leukemia is expected to be complete by year-end
- Enrollment in the relapsed or refractory myelodysplastic syndrome arm of the TIBSOVO® Phase 1 study of IDH1-mutant advanced hematologic malignancies is expected to be complete by year-end
- Full-year net product revenue for TIBSOVO® is expected to be $160-170 million

Agios 2025 Strategic Vision
The Agios 2025 strategic vision reflects the company's expected evolution over the next five years in light of its singular focus on genetically defined diseases. As part of this vision, Agios expects to achieve the following milestones by the end of 2025:

- Receive regulatory approval for mitapivat in three initial indications: 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

2020 Year-End Cash and Guidance
Agios ended 2020 with approximately $670.5 million of cash, cash equivalents and marketable securities. The company expects that its cash, cash equivalents and marketable securities as of December 31, 2020, together with anticipated product and royalty revenue, interest income and expense reimbursements under our collaboration agreements, but excluding any additional program-specific milestone payments, will enable the company to fund its planned operating expenses and capital expenditure requirements to the end of 2022. Following the completion of the transaction with Servier and the subsequent shareholder returns, Agios expects its cash runway to extend to cash-flow positivity in 2025.

Presentation at 39th Annual J.P. Morgan Healthcare Conference
Agios will webcast its corporate presentation from the virtual 39th Annual J.P. Morgan Healthcare Conference on Monday, January 11, 2021 at 10:50 a.m. ET (7:50 a.m. PT). 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 focused on discovering and developing novel investigational medicines to treat malignant hematology, solid tumors and genetically defined diseases through scientific leadership in the field of cellular metabolism. In addition to an active research and discovery pipeline across these three therapeutic areas, Agios has two approved oncology precision medicines and multiple first-in-class investigational therapies in clinical and/or 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; the potential benefits of Agios' products and product candidates; Agios' key milestones and guidance for 2021 and strategic vision for 2025; its financial guidance regarding the period in which it will have capital available to fund its operations; expectations regarding the sale of Agios' oncology portfolio and associated return of capital to shareholders; and the potential benefits of Agios's 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: (i) Agios's sale of its oncology portfolio, including the occurrence of any event, change or other circumstance that could give rise to the termination of the purchase and sale agreement; the failure of Agios to obtain stockholder approval for the proposed transaction or the failure to satisfy any of the other conditions to the completion of the proposed transaction; the effect of the announcement of the proposed transaction on the ability of Agios to retain and hire key personnel and maintain relationships with its customers, suppliers, advertisers, partners and others with whom it does business, or on its operating results and businesses generally; risks associated with the disruption of management's attention from ongoing business operations due to the proposed transaction; the ability to meet expectations regarding the timing and completion of the proposed transaction, including with respect to receipt of required regulatory approvals; the failure of Agios to receive milestone or royalty payments under the purchase and sale agreement and 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 proposed transaction; (ii) 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; (iii) Agios' results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; (iv) 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; (v) Agios' ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials; (vi) unplanned cash requirements and expenditures and competitive factors; (vii) Agios' ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates it is developing; (viii) Agios' ability to maintain key collaborations; and (ix) 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.

Additional Information and Where to Find It
This communication relates to the proposed transaction involving the sale by Agios Pharmaceuticals, Inc. ("Agios") of its oncology business to Servier Pharmaceuticals, LLC. In connection with the proposed transaction, Agios will file relevant materials with the U.S. Securities and Exchange Commission (the "SEC"), including Agios' proxy statement on Schedule 14A (the "Proxy Statement"). This communication is not a substitute for the Proxy Statement or any other document that Agios may file with the SEC or send to its stockholders in connection with the proposed transaction. BEFORE MAKING ANY VOTING DECISION, STOCKHOLDERS OF AGIOS ARE URGED TO READ ALL RELEVANT DOCUMENTS FILED WITH THE SEC, INCLUDING THE PROXY STATEMENT, WHEN THEY BECOME AVAILABLE BECAUSE THEY WILL CONTAIN IMPORTANT INFORMATION ABOUT THE PROPOSED TRANSACTION. Investors and security holders will be able to obtain the documents (when available) free of charge at the SEC's website, at http://www.sec.gov, and Agios's website, at www.agios.com. In addition, the documents (when available) may be obtained free of charge by accessing Agios's website at www.agios.com under the heading "Investors" or, alternatively, directing a request to Holly Manning by email at holly.manning@agios.com or by calling 617-649-8600.

Participants in the Solicitation
Agios and its directors and executive officers may be deemed to be participants in the solicitation of proxies from the holders of Agios common stock in respect of the proposed transaction. Information about the directors and executive officers of Agios is set forth in the proxy statement for Agios' 2020 annual meeting of stockholders, which was filed with the SEC on April 16, 2020, and in other documents filed by Agios with the SEC. Other information regarding the participants in the proxy solicitation and a description of their direct and indirect interests, by security holdings or otherwise, will be contained in the Proxy Statement and other relevant materials to be filed with the SEC in respect of the proposed transaction when they become available.

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