Agios Highlights Key 2019 Initiatives to Broaden Potential for Cancer and Rare Genetic Disease Programs to Build Long-Term Value

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– Expanding the Label for TIBSOVO®: Supplemental New Drug Application (sNDA) Submitted for TIBSOVO® as Monotherapy for Patients with Newly Diagnosed AML with an IDH1 Mutation Ineligible for Standard Therapy; Approval Expected in 2019 –

– IDH1m Solid Tumor Opportunity Advancing: sNDA for TIBSOVO® in Second-line or Later Cholangiocarcinoma Expected by Year-End; Phase 3 Trial in Low-Grade Glioma with Vorasidenib (AG-881) Expected to Begin by Year-End –

– Mitapivat PK Deficiency Pivotal Program On Track to Complete Enrollment by Year-End; Clinical Program Expanded with Initiation of Phase 2 Study in Thalassemia and Plan to Pursue Development in Pediatric PK Deficiency –

– Clinical Development of MAT2A Inhibitor AG-270 Advancing into Expansion Arms in 1H 2019; Phase 1 Dose-Escalation Data to be Presented in 2H 2019 –

SAN FRANCISCO, Jan. 07, 2019 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, today summarized key 2019 initiatives in conjunction with its presentation at the 37th Annual J.P. Morgan Healthcare Conference in San Francisco. The company will webcast its presentation today at 9:30 a.m. PT (12:30 p.m. ET) at investor.agios.com.

"During 2018, just 10 years after the founding of Agios, we achieved approval of our second internally discovered oncology medicine, launched a robust registrational program in PK deficiency and successfully opened the company's seventh IND," said David Schenkein, M.D., chief executive officer at Agios. "Our validated research platform and proven drug development strategy are poised to help drive future growth across our oncology and rare genetic disease portfolios. Our priorities for 2019 include expanding the reach of our IDH inhibitors into the frontline AML and solid tumor settings, completing enrollment in two pivotal studies of mitapivat, exploring the utility of PKR activators in other hemolytic anemias, and furthering clinical development for AG-270 in MTAP deleted tumors and AG-636 in lymphoma."

The company plans to achieve the following milestones in 2019.

Cancer:

- Potential FDA approval of the supplemental new drug application (sNDA) for single agent TIBSOVO® (ivosidenib) for the treatment of patients with newly diagnosed AML with an IDH1 mutation who are not eligible for standard therapy.
- Submit a sNDA to the FDA for TIBSOVO® for second line or later IDH1m cholangiocarcinoma by year-end.
- Initiate a registration-enabling Phase 3 study of vorasidenib (AG-881) in low-grade glioma with an IDH1 mutation by year-end.
- Determine recommended dose of AG-270, a first-in-class methionine adenosyltransferase 2a (MAT2A) inhibitor, in methylthioadenosine phosphorylase (MTAP)-deleted tumors and initiate expansion arms, including a single-agent arm in a variety of MTAP-deleted cancers and a combination arm in a solid tumor in the first half of 2019.
- Initiate a Phase 1 dose-escalation trial of AG-636, an inhibitor of the metabolic enzyme dihydroorotate dehydrogenase (DHODH), in lymphoma in the first half of 2019.

Rare Genetic Diseases:

- Complete enrollment in two global pivotal trials for mitapivat in adults with pyruvate kinase (PK) deficiency by year-end 2019:
    - ACTIVATE-T: A single-arm trial of approximately 20 regularly transfused patients
    - ACTIVATE: A 1:1 randomized, placebo-controlled trial of 80 patients who do not receive regular transfusions
- Achieve proof-of-concept for mitapivat in thalassemia in the second half of 2019.

The company highlighted key data presentations expected in 2019.

- Updated data from the ongoing Phase 1 combination trial of TIBSOVO® with azacitidine in patients with newly diagnosed AML with an IDH1 mutation in the first half of 2019.
- Data from the perioperative 'window' trial with TIBSOVO® and vorasidenib in IDHm low-grade glioma in the first half of 2019.
- Topline data from the Phase 3 ClarIDHy study of TIBSOVO® in IDH1m advanced cholangiocarcinoma to be reported in the first half and full data to be presented in the second half of 2019.
- Data from the dose-escalation portion of the ongoing Phase 1 study of AG-270 in patients with MTAP-deleted tumors in the

second half of 2019.

The company also provided an update on the following 2018 milestones achieved in December.

- Submitted an sNDA to the FDA for TIBSOVO® for the treatment of patients with newly diagnosed AML with an IDH1 mutation who are not eligible for standard therapy.
- Submitted a Marketing Authorization Application to the European Medicines Agency for TIBSOVO® for the treatment of adult patients with R/R AML with an IDH1 mutation.
- Initiated a Phase 2 proof-of-concept trial of mitapivat in thalassemia.

2018 Year-End Cash and Guidance
Agios ended 2018 with approximately $805 million of cash, cash equivalents and marketable securities. The company expects that its cash, cash equivalents and marketable securities as of December 31, 2018, together with anticipated product and royalty revenue, anticipated interest income, and anticipated expense reimbursements under our collaboration agreements, but excluding any additional program-specific milestone payments, will enable the company to fund its anticipated operating expenses and capital expenditure requirements through at least the end of 2020.

Presentation at 37th Annual J.P. Morgan Healthcare Conference
Agios will webcast its corporate presentation from the 37th Annual J.P. Morgan Healthcare Conference in San Francisco on Monday, January 7, 2019 at 9:30 a.m. PT (12:30 p.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 focused on discovering and developing novel investigational medicines to treat cancer and rare genetic diseases through scientific leadership in the field of cellular metabolism. In addition to an active research and discovery pipeline across both therapeutic areas, Agios has two approved oncology precision medicines and multiple first-in-class investigational therapies in clinical and/or preclinical development. All Agios programs focus on genetically identified patient populations, leveraging our knowledge of metabolism, biology and genomics. For more information, please visit the company's website at www.agios.com.

About Agios/Celgene Collaboration
IDHIFA® (enasidenib) and AG-270 are part of our collaboration with Celgene Corporation. Under the terms of our 2010 collaboration agreement focused on cancer metabolism, Celgene has worldwide development and commercialization rights for IDHIFA®. Agios continues to conduct certain clinical development activities within the IDHIFA® development program and is eligible to receive reimbursement for those development activities and up to $80 million in remaining milestone payments, and royalties on any net sales. Celgene and Agios are currently co-commercializing IDHIFA® in the U.S. Celgene will reimburse Agios for costs incurred for its co-commercialization efforts. AG-270 is part of a 2016 global research collaboration agreement with Celgene focused on metabolic immuno-oncology. Celgene has the option to participate in a worldwide 50/50 cost and profit share with Agios, under which Agios is eligible for up to $169 million in clinical and regulatory milestone payments for the program.

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 its and its collaborator's preclinical, clinical and commercial advancement of its drug development programs including TIBSOVO® (ivosidenib), IDHIFA® (enasidenib), vorasidenib (AG-881), mitapivat, AG-270 and AG-636; the potential benefits of Agios' product candidates; its key milestones for 2019; its estimates regarding its balance of cash, cash equivalents and marketable securities for the year ended December 31, 2018; plans regarding future data presentations; its financial guidance regarding the period in which it will have capital available to fund its operations; and the potential benefit of its strategic plans and focus. The words "anticipate," "expect," "hope," "milestone," "plan," "potential," "possible," "strategy," "will," 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 or its collaborators 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: 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, such as its agreements with Celgene and CStone Pharmaceuticals; 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.

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