Agios Announces Key Upcoming Milestones to Support Evolution to a Commercial Stage Biopharmaceutical Company in 2017

January 9, 2017

- Enasidenib (AG-221) NDA Submitted for IDH2m Relapsed/Refractory (R/R) Acute Myeloid Leukemia (AML) -

- AG-120 NDA Submission for IDH1m R/R AML Planned by Year End 2017 -

- AG-348 Advancing to Pivotal Development in PK Deficiency -

- Development Candidate for MTAP Pathway Selected; IND Submission Expected by Year End 2017 -

- Company Ends 2016 in a Strong Financial Position with $574M in Cash, Cash Equivalents and Marketable Securities -

SAN FRANCISCO, Jan. 09, 2017 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the fields of cancer metabolism and rare genetic metabolic diseases, today outlined key 2017 milestones in conjunction with its presentation at the 35th Annual J.P. Morgan Healthcare Conference in San Francisco. The presentation will outline important milestones as Agios evolves into a commercial stage company, including potential launches for enasidenib and AG-120 in R/R AML, pivotal development for its second wholly owned asset, AG-348 in pyruvate kinase (PK) deficiency, and an investigational new drug (IND) application submission for the company's next development candidate, focused on MTAP deleted cancers. The company will webcast its presentation on Monday, January 9, 2017 at 10:00 a.m. PT (1:00 p.m. ET) at www.agios.com.

"This is the year Agios will evolve into a commercial-stage organization with the anticipated launch of enasidenib for patients with R/R AML, followed by the NDA submission of AG-120 and AG-348 preparing to enter a pivotal trial in PK deficiency," said David Schenkein, M.D., chief executive officer of Agios. "We believe these milestones will enable us to achieve our vision of delivering important medicines with the potential to transform patients' lives. Additionally, our robust research engine continues to be highly productive with an IND submission for the company's sixth development candidate in eight years anticipated by the end of 2017."

The company expects to achieve the following key milestones by the end of 2017:

- Potential approval of enasidenib in the United States for IDH2m positive R/R AML in collaboration with Celgene.
- Submit a new drug application (NDA) to the U.S. FDA for AG-120 by the end of 2017. AG-120 is a wholly owned, first-in-class, oral, selective, potent inhibitor of IDH1m, in IDH1m positive R/R AML.
- Initiate a global, registration-enabling Phase 3 study combining AG-120 and VIDAZA® in frontline AML patients with an IDH1 mutation ineligible for intensive chemotherapy in the first half of 2017.
- Finalize design and operational activities for a global pivotal trial of AG-348 to initiate in the first half of 2018. AG-348 is a wholly owned, first-in-class, oral activator of both wild-type (normal) and mutated pyruvate kinase-R (PKR) enzymes, in PK deficiency.
- File an IND application for the MTAP pathway development candidate by the end of 2017.

The company also provided an update on the following 2016 milestones achieved in December:

- Supported Celgene's submission of an NDA for enasidenib in IDH2m positive R/R AML.
- Initiated a global, registration-enabling randomized Phase 3 trial for AG-120 in IDH1m positive cholangiocarcinoma. The FDA also granted AG-120 Fast Track Designation for the treatment of patients with previously treated, unresectable or metastatic cholangiocarcinoma with an IDH1 mutation.
- Selected a development candidate focused on the MTAP pathway to enter IND-enabling studies.

2016 Year-End Cash and Guidance

Agios ended 2016 with approximately $574 million of cash, cash equivalents and marketable securities. Based on its current operating plans, the company expects that its existing cash, cash equivalents and marketable securities as of December 31, 2016, together with anticipated interest income, and anticipated expense reimbursements under its collaboration agreements with Celgene, but excluding any additional program-specific milestone payments from Celgene, will enable the company to fund its anticipated operating expenses and capital expenditure requirements through at least the end of 2018.

Presentation at 35th Annual J.P. Morgan Healthcare Conference

Agios will webcast its corporate presentation from the 35th Annual J.P. Morgan Healthcare Conference in San Francisco on Monday, January 9, 2017 at 10:00 a.m. PT (1:00 p.m. ET). A live webcast of the presentation can be accessed under "Events & Presentations" in the Investors and Media 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 metabolic 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 multiple first-in-class investigational medicines in clinical and/or preclinical development. All Agios programs focus on genetically identified patient populations, leveraging the company's knowledge of metabolism, biology and genomics. For more information, please visit the company's website at www.agios.com.

About Agios/Celgene Collaboration

Enasidenib and AG-881 are part of Agios' global strategic collaboration with Celgene Corporation focused on cancer metabolism. Under the terms of the 2010 collaboration, Celgene has worldwide development and commercialization rights for enasidenib. Agios continues to conduct clinical development activities within the enasidenib development program and is eligible to receive up to $120 million in payments assuming achievement of certain milestones and royalties on net sales. Additionally, Agios and Celgene intend to co-commercialize enasidenib in the U.S. For AG-881, the companies have a joint worldwide development and 50/50 profit share collaboration, and Agios is eligible to receive regulatory milestone payments of up to $70 million. The program focused on MTAP (methylthioadenosine phosphorylase) deleted cancers is part of a 2016 global co-development and co-commercialization agreement with Celgene focused on metabolic immuno-oncology with a worldwide 50/50 cost and profit share between Agios and Celgene, under which Agios is eligible for up to $169 million in clinical and regulatory milestone payments for the program.

Vidaza® is a registered trademark of Celgene Corporation.

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 Agios' plans, strategies and expectations for its and its collaborator's preclinical, clinical and commercial advancement of its drug development programs including enasidenib, AG-120, and AG-348; the potential benefits of Agios' product candidates; its key milestones for 2017; 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," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," "could," "potential," "possible," "hope," "strategy," "milestone," "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 collaborator, Celgene, 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 and 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 general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in Agios' Quarterly Report on Form 10-Q for the quarter ended September 30, 2016, and other filings that Agios may make with the Securities and Exchange Commission in the future. 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.

Contacts

Investors:
Kendra Adams, 617-844-6407
Senior Director, Investor & Public Relations
Kendra.Adams@agios.com

Renee Leck, 617-649-8299
Senior Manager, Investor & Public Relations
Renee.Leck@agios.com

Media:
Holly Manning, 617-844-6630
Associate Director, Corporate Communications
Holly.Manning@agios.com

Agios Pharmaceuticals