FDA Accepts New Drug Application and Grants Priority Review for Ivosidenib in Relapsed or Refractory AML with an IDH1 Mutation

February 15, 2018

– PDUFA date set for August 21, 2018 –

CAMBRIDGE, Mass., Feb. 15, 2018 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, today announced that the U.S. Food and Drug Administration (FDA) has accepted the company's New Drug Application (NDA) for ivosidenib (AG-120) for the treatment of patients with relapsed or refractory (R/R) acute myeloid leukemia (AML) with an isocitrate dehydrogenase 1 (IDH1) mutation. The NDA was granted Priority Review and has been given a Prescription Drug User Fee Act (PDUFA) action date of August 21, 2018. The FDA's Priority Review status accelerates the review time from 10 months to a goal of six months from the day of filing acceptance and is given to drugs that may offer major advances in treatment or may provide a treatment where no adequate therapy exists. Agios completed the NDA submission in late December 2017.

"After decades of little change, treatment of AML has begun to shift dramatically as result of new therapies, and IDHm inhibitors will play an important role in how we treat this terrible disease," said David Schenkein, M.D., chief executive officer of Agios. "Today marks an important milestone in our efforts to rapidly advance what could be the first targeted treatment for R/R AML patients with an IDH1 mutation. We appreciate the FDA's collaboration during the application process, and we look forward to continuing our productive dialogue."

Ivosidenib is a first-in-class, oral, targeted inhibitor of mutant IDH1. The NDA submission is based on results from AG120-C-001, a Phase 1 dose-escalation and expansion study of ivosidenib in patients with advanced hematologic malignancies and an IDH1 mutation. Data from the R/R AML patients in this study were presented at the 2017 American Society of Hematology (ASH) Annual Meeting.

Additionally, Abbott has submitted a Premarket Approval (PMA) application for the FDA review of an IDH1 assay on the Abbott m2000 RealTi*m*e System, an automated sample preparation and batch analyzer system for nucleic acid amplification and detection. In 2014, Abbott and Agios entered into an exclusive agreement under which Abbott is responsible for development and commercialization of a RealTime PCR assay for detection of the IDH1 mutation in bone marrow and blood. The Abbott assay will serve as a companion diagnostic for ivosidenib.

IDH1 mutations occur in about 6 to 10 percent of AML patients. Recent publications have highlighted the advances in the understanding of the genetics underlying AML and the need for routine mutational analysis at diagnosis and relapse.

Ivosidenib is an investigational drug that has not been approved for any use in any country.

About Acute Myelogenous Leukemia (AML)
AML, a cancer of blood and bone marrow characterized by rapid disease progression, is the most common acute leukemia affecting adults. Undifferentiated blast cells proliferate in the bone marrow rather than mature into normal blood cells. AML incidence significantly increases with age, and the median age at diagnosis is 68. The vast majority of patients do not respond to chemotherapy and progress to relapsed/refractory AML. The five-year survival rate for AML is approximately 20 to 25 percent. IDH1 mutations are present in about 6 to 10 percent of AML cases.

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 an approved oncology precision medicine 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.

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' expectations for the FDA's review of its NDA for ivosidenib. The words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" 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, the FDA's acceptance of Agios's NDA for ivosidenib does not represent evaluation of the efficacy and safety of ivosidenib, and is not a guarantee of approval. 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: risks associated with the regulatory review process generally; the risk that the FDA may determine that the data included in the NDA are insufficient for approval and that the Company must conduct additional clinical trials, or nonclinical or other studies, before ivosidenib can be approved; the risk that the results of previously conducted studies involving ivosidenib will not be repeated or observed in ongoing or future studies or following commercial launch, if ivosidenib is approved; and risks associated with the Company's dependence on third parties with respect to regulatory matters for ivosidenib. 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.

Contacts

Investors:
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

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