Agios Pharmaceuticals Announces FDA Orphan Drug Designation Granted to AG-120 for Treatment of IDH1-Mutant Positive Acute Myelogenous Leukemia

June 10, 2015

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 10, 2015-- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the fields of cancer metabolism and rare genetic disorders of metabolism, today announced that the U.S. Food and Drug Administration (FDA) has granted the company orphan drug designation for AG-120 for treatment of patients with acute myelogenous leukemia (AML). AG-120 is an oral, first-in-class IDH1 mutant inhibitor being evaluated in a Phase 1 clinical trial in patients with advanced hematologic malignancies that carry an IDH1 mutation.

The FDA's Office of Orphan Drug Products grants orphan status to support development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the U.S. Orphan drug designation provides to Agios certain benefits, including market exclusivity upon regulatory approval if received, exemption of FDA application fees and tax credits for qualified clinical trials.

"Receiving orphan drug designation for AG-120 is an important milestone as we continue to move this program to late-stage development," said Chris Bowden, M.D. chief medical officer of Agios. "We are pleased with the progress we are making in the clinic and look forward to presenting new data from our ongoing Phase 1 study of AG-120 at the Congress of the European Hematology Association later this week. We believe that AG-120, which is on track to initiate multiple expansion cohorts in the next month, has the potential to play a significant role in shifting the treatment paradigm for IDH1-mutant positive hematologic cancers from the conventional chemotherapy approach."

AML, a cancer of blood and bone marrow characterized by rapid disease progression, is the most common acute leukemia in adults. Undifferentiated blast cells proliferate in the bone marrow rather than mature into normal blood cells. AML incidence significantly increases with age, and according to the American Cancer Society the median age is 66. Less than 10 percent of U.S. patients are eligible for bone marrow transplant, and 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 Pharmaceuticals, Inc.

Agios Pharmaceuticals is focused on discovering and developing novel investigational medicines to treat cancer and rare genetic disorders of metabolism 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 our knowledge of metabolism, biology and genomics. For more information, please visit the company's website at 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 the potential benefits of Agios' product candidate AG-120; the potential benefits of orphan drug status; and the benefit of its strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "potential," "possible," "hope," "could," "would" 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 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 agreement 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 March 31, 2015, 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.

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