



AgiOS Pharmaceuticals Announces Orphan Drug Designation of AG-221 for Treatment of Acute Myelogenous Leukemia

June 16, 2014

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 16, 2014-- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the fields of cancer metabolism and inborn errors of metabolism (IEMs), today announced that the U.S. Food and Drug Administration (FDA) has granted the company orphan drug designation for AG-221 for treatment of patients with acute myelogenous leukemia (AML). AG-221 is an oral, first-in-class IDH2 mutant inhibitor being evaluated in a Phase 1 clinical trial in patients with advanced hematologic malignancies that carry an IDH2 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 and its collaborator Celgene 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-221 is another important milestone to emerge from our innovative cancer metabolism program," said Chris Bowden, M.D. chief medical officer of Agios. "We are pleased with the progress we are making in the clinic and believe that AG-221, which is expected to enter multiple expansion cohorts in the second half of this year, has the potential to play a significant role in shifting the treatment paradigm for IDH2 mutant positive hematologic cancers from the conventional chemotherapy approach."

AML is a cancer of blood and bone marrow characterized by rapid disease progression, and is the most common acute leukemia affecting adults. 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 or refractory AML. The five-year survival rate for AML is approximately 20 to 25 percent. AML prevalence is estimated to be approximately 35,000 to 40,000 patients in the U.S., with approximately 15% of patients estimated to carry an IDH2 mutation.

About Agios Pharmaceuticals, Inc.

AgiOS Pharmaceuticals is focused on discovering and developing novel drugs to treat cancer and inborn errors of metabolism, or IEMs, which are 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 lead product candidates in cancer metabolism and IEMs 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 our 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 the potential benefits of Agios' product candidates AG-221 and AG-120; and the benefit of its strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "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, 2014, 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.

Source: Agios Pharmaceuticals, Inc.

AgiOS Pharmaceuticals, Inc.
Lora Pike, 617-649-8608
Senior Director, Investor Relations and Public Relations
lora.pike@agios.com