Agios Pharmaceuticals Announces the U.S. FDA Grants Fast Track Designation to AG-221 for Treatment of Patients With Acute Myelogenous Leukemia That Harbor an IDH2 Mutation

August 13, 2014

CAMBRIDGE, Mass., Aug. 13, 2014 (GLOBE NEWSWIRE) -- 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 Fast Track designation to AG-221 for the treatment of patients with acute myelogenous leukemia (AML) that harbor an isocitrate dehydrogenase-2 (IDH2) mutation. AG-221 is a first-in-class, oral, selective, potent IDH2 mutant inhibitor being evaluated in a Phase 1 clinical trial in patients with advanced hematologic malignancies.

"We believe this designation is an important recognition by the FDA of the nonclinical and clinical data reported to date and the potential for AG-221 to address a significant unmet need for patients diagnosed with AML," said Chris Bowden, M.D., chief medical officer of Agios. "We remain on track to initiate the planned expansion cohorts for patients with IDH2 mutant positive AML and other IDH2 mutant positive hematologic malignancies in the second half of this year. We are committed to working with our partner Celgene Corporation to get this medicine to patients as soon as possible."

The Fast Track Drug Development Program was established under the FDA Modernization Act of 1997. The program is designed to facilitate frequent interactions with the FDA review team to expedite clinical development and submission of a New Drug Application (NDA) for medicines with the potential to treat serious or life-threatening conditions and address unmet medical needs. Specifically, Fast Track designation facilitates meetings to discuss all aspects of development to support approval. It also provides the opportunity to submit sections of an NDA on a rolling basis as data become available. This permits the FDA to review portions of the NDA as they are received instead of waiting for the entire NDA submission.

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 115,000 to 160,000 patients worldwide, with approximately 20 percent of patients carrying an IDH mutation.

About Agios Pharmaceuticals, Inc.

Agios Pharmaceuticals is focused on discovering and developing novel drugs 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 lead product candidates in cancer metabolism and rare genetic disorders of metabolism 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' drug candidate AG-221; 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 June 30, 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.

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