Agios Pharmaceuticals Announces Orphan Drug Designation of AG-348 for Treatment of Pyruvate Kinase Deficiency

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- PK deficiency is a severe and rare genetic disease with currently no approved or disease modifying treatments -

CAMBRIDGE, Mass., March 24, 2015 (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 United States Food and Drug Administration (FDA) has granted the company orphan drug designation for its investigational medicine AG-348 for the treatment of pyruvate kinase (PK) deficiency, a rare form of hemolytic anemia. AG-348, a first-in-class, orally available activator of pyruvate kinase-R (PKR) enzymes, met its primary endpoints in two Phase 1 healthy volunteer studies – a single ascending dose study and multiple ascending dose study. In addition, data presented in December 2014 at the 54th Annual Meeting of the American Society of Hematology (ASH) provided early proof-of-mechanism for AG-348. Based on these findings, Agios plans to initiate a Phase 2 clinical trial in patients with PK deficiency in the first half of 2015.

"We are pleased to achieve another milestone in the clinical program for AG-348, the first medicine in development designed to treat the underlying cause of PK deficiency," said Chris Bowden, M.D., chief medical officer of Agios. "PK deficiency can result in lifelong medical problems and is an example of our focus on underserved diseases with significant medical needs."

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. This designation provides certain benefits, including market exclusivity upon regulatory approval, if received, exemption of FDA application fees and tax credits for qualified clinical trials.

About AG-348 Clinical Development Plans and Upcoming Milestones

Based on findings presented at ASH, Agios expects to initiate a Phase 2 study of AG-348 in patients with PK deficiency in the first half of 2015. The company plans to provide final results from the Phase 1 multiple ascending dose study at a medical conference in mid-2015. A natural history study of PK deficiency is also ongoing and Agios expects to report initial data from this study at a medical conference in mid-2015.

About Pyruvate Kinase (PK) Deficiency, a Rare, Inherited Hemolytic Anemia

Pyruvate kinase (PK) deficiency, a rare, inherited hemolytic anemia affecting children and adults. It is caused by mutations that affect the activity of the metabolic enzyme pyruvate kinase-R (PKR), the form of pyruvate kinase that is present in red blood cells. When this enzyme (or protein) is deficient, people with PK deficiency have fewer healthy red blood cells. The current standard of care for PK deficiency is supportive, including blood transfusions, splenectomy, chelation therapy to address iron overload and/or interventions for other treatment, and disease-related morbidities. Currently, there is no approved therapy to treat the underlying cause of PK deficiency. Agios retains worldwide development and commercialization rights for AG-348.

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' drug candidate AG-348; 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; and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in Agios' Annual Report on Form 10-K for the year ended December 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.

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