Agios Pharmaceuticals Initiates Multiple Ascending Dose Trial in Healthy Volunteers of AG-348 for the Potential Treatment of PK Deficiency, a Rare, Hemolytic Anemia

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Initial Safety and Pharmacokinetic Findings From Single Ascending Dose Trial Support Early Advancement

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 9, 2014-- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the fields of cancer metabolism and inborn errors of metabolism (IEMs), today announced the initiation of a multiple ascending dose (MAD) Phase 1 trial of AG-348 in healthy volunteers. This study is designed to characterize the safety, tolerability, pharmacokinetics and pharmacodynamics of increasing doses of AG-348 for 14 days. AG-348 is a novel, first-in-class, orally available activator of pyruvate kinase-R (PKR) for the treatment of pyruvate kinase (PK) deficiency, a rare, hemolytic anemia. This trial was initiated based on data from the ongoing single ascending dose (SAD) Phase 1 trial, in which AG-348 has been determined to be well tolerated to date. The SAD study began in April 2014 and has completed dosing of more than half of the planned cohorts. Data from both studies are expected to be presented at a medical conference in 2015.

“AG-348 represents a completely new approach to treating patients with PK deficiency, a serious form of inherited hemolytic anemia with no approved therapy that targets the metabolic defect responsible for the underlying disease,” said David Schenkein, M.D., chief executive officer of Agios. “We are pleased to be moving forward with the MAD study that will allow us to further characterize the product candidate and establish the dose ranges we will study in patients with PK deficiency.”

Preclinical studies have demonstrated that AG-348 activates a broad spectrum of PKR mutant proteins, and corrects the metabolic defects found in patient-derived blood samples. AG-348 is currently the only drug candidate being evaluated for the potential of correcting metabolic defects found in patients with PK deficiency.

About the Study

The Phase 1, single-center, randomized, double-blind, placebo-controlled clinical trial will assess the safety and tolerability of AG-348 through multiple ascending doses in healthy adult men and women. Key objectives of the trial include characterizing the safety, pharmacokinetic and pharmacodynamic relationships of AG-348 and select metabolic biomarkers. Please refer to www.clinicaltrials.gov for additional clinical trial details.

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

Pyruvate kinase (PK) deficiency, a rare, inherited hemolytic anemia affecting children and adults, 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. 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. AG-348 is a first-in-class orally available, potent, selective small molecule activator of PKR, which, when mutated, leads to PK deficiency. AG-348 was discovered in the laboratory of Agios, and the company retains worldwide development and commercialization rights.

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 Agios’ expectations and beliefs about: the potential of pyruvate kinase R as a therapeutic target; the potential benefits of Agios’ product candidate AG-348; its plans and timelines for the clinical development of 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, 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.

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