Agios Enrolls First Patient in Phase 1 Study of AG-120 in Advanced Solid Tumors with an IDH1 Mutation

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Second Clinical Trial Investigating AG-120 in IDH1-Mutant Cancers

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 24, 2014-- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the fields of cancer metabolism and inborn errors of metabolism, today announced dose administration for the first patient in a Phase 1 study of AG-120 in patients with advanced solid tumors with an isocitrate dehydrogenase-1 (IDH1) mutation. AG-120 is an orally available, selective, potent inhibitor of the mutated IDH1 protein and a highly targeted, first-in-class therapeutic candidate.

This is the second Phase 1 study of AG-120. Agios recently announced that the first patient received treatment in a Phase 1 study of AG-120 in patients with hematologic malignancies.

“We are pleased to have rapidly initiated two studies evaluating AG-120 in a broad range of solid tumors and hematologic cancers with IDH1 mutations,” said David Schenkein, M.D., chief executive officer of Agios. “Both of these studies employ a precision medicine approach that will evaluate AG-120 among diagnostically identified patient populations, with a goal of generating valuable early data about this cancer metabolism development program. We believe the approach of targeting IDH mutations in cancer holds great promise for patients.”

“Because this study will only enroll patients whose cancers have an IDH1 mutation, it is expected to provide us with important information about how AG-120 works among the patients who are most likely to respond to it,” said Howard Burris, M.D., Sarah Cannon Research Institute, an investigator for the study. “We are hopeful that inhibiting these mutated metabolic enzymes will have a significant clinical benefit for patients with solid tumors that carry these mutations.”

AG-120 is a part of Agios’ global strategic collaboration with Celgene Corporation, a leading biotechnology company. Established in 2010, the goal of the collaboration is to discover, develop and deliver novel, disease-altering oncology therapies, based on Agios’ cancer metabolism research platform. Agios recently announced that it has elected the option to take exclusive rights on U.S. development and commercialization for AG-120, subject to and in accordance with the terms of the collaboration agreement with Celgene, with Celgene retaining its option to ex-U.S. rights. The parties are also collaborating on the development of AG-221, an oral, selective, potent inhibitor of the mutated IDH2 protein.

About the Study

The Phase 1, multi-center, open-label, dose-escalation and expansion clinical trial will evaluate the clinical activity, safety and tolerability of AG-120 among patients with advanced solid tumors that carry an IDH1 mutation. Key objectives of the trial include determining the maximum tolerated dose, evaluating the pharmacokinetics and pharmacodynamics (including the relationship of AG-120 with the oncometabolite 2-hydroxyglutarate, or 2HG), characterizing the clinical activity and describing the dose-limiting toxicities. Please refer to www.clinicaltrials.gov for additional clinical trial information.

About IDH Mutations and Cancer

The connection between cancer and metabolism has been the central focus for scientists at Agios, who were the first to identify the neo-activity of IDH1 mutations to produce 2HG in research published in *Nature* in 2009. These insights revealed the potential of IDH1 and IDH2 mutations as novel therapeutic targets in cancer. Mutations in both IDH1 and IDH2 have been linked to numerous hematologic and solid tumor malignancies.

Agios and its collaborators recently demonstrated that IDH1 and IDH2 mutations initiate and drive cancer growth by blocking differentiation, or maturation, of primitive cells. Agios believes that inhibition of these mutated proteins may lead to clinical benefit for the subset of cancer patients whose tumors carry them.

About Agios Pharmaceuticals, Inc.

Agios Pharmaceuticals is focused on discovering and developing novel drugs to treat cancer and inborn errors of metabolism (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 IDH1/IDH2 as therapeutic targets; the potential benefits of Agios’ product candidates AG-120 and AG-221; its plans and timelines for the clinical development of AG-120 and AG-221; and the benefit of its strategic plans and focus. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “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' Annual Report on Form 10-K for the year ended December 31, 2013, 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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