# Agios Pharmaceuticals Exercises Option to U.S. Development and Commercialization Rights for IDH1 Program under Celgene Collaboration

February 3, 2014

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 3, 2014-- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the fields of cancer metabolism and inborn errors of metabolism, announced today that it has elected to retain the United States development and commercial rights to the isocitrate dehydrogenase 1 (IDH1) program including the clinical candidate AG-120, in accordance with the terms of its global strategic collaboration with Celgene Corporation in the field of cancer metabolism.

“The opportunity to develop and commercialize the IDH1 program in the U.S. market is a critical component of Agios’ long term strategy and vision to transform the lives of patients with cancer,” said David Schenkein, M.D., chief executive officer of Agios. “Celgene has been an exceptional partner for our cancer metabolism research efforts, and we are looking forward to the next phase of our collaboration with the global clinical development of the IDH2 and IDH1 programs.”

By exercising Agios' option to U.S. rights for the IDH1 program, Agios will lead development and commercialization activities for AG-120 in the U.S., and Celgene retains the option to lead development and commercialization activities for AG-120 in the rest of the world. AG-120 is an orally available, selective, potent inhibitor of the mutated IDH1 protein and a highly targeted first-in-class therapeutic candidate for the treatment of patients with cancers that harbor an IDH1 mutation.

Agios and Celgene are also collaborating on the development of AG-221, an oral, selective, potent inhibitor of the mutated IDH2 protein, making it the first targeted therapeutic candidate to treat patients with cancers that harbor an IDH2 mutation.

**About IDH Mutations and Cancer**

The connection between cancer and metabolism has been the central focus of scientists at Agios, who were the first to identify the neo-activity of IDH1 mutations to produce the oncometabolite 2-HG 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, also referred to as 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 these mutations.

**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 IDH mutations as therapeutic targets; the potential benefits of its product candidates AG-221 and AG-120; the benefits of its collaboration with Celgene; and its plans and strategies. 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, including risks and uncertainties relating to: Agios’ ability to successfully commence and complete necessary preclinical and clinical development of its product candidates; Agios’ results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; Agios’ ability to maintain its collaboration with third parties on acceptable terms; 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; 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 obtain the substantial additional capital required to execute its plans and strategies; and general economic and market conditions. These and other risks are described in greater detail in filings that Agios makes with the SEC from time to time including risks described under the caption “Risk Factors” included in Agios’ Quarterly Report on Form 10-Q for the quarter ended September 30, 2013. 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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