Agios Announces First Patient Dosed with AG-881 in Phase 1 Study in Patients with Advanced Solid Tumors with an IDH Mutation

June 24, 2015

– Third IDH Mutant Inhibitor to Enter Clinical Trials –

– Brain Penetration and Pan-IDHm Inhibition Broaden Development Options for IDH Mutant-Positive Cancers, Including Gliomas –

– Second Phase 1 Study in Hematologic Malignancies on Track to Begin Dosing in Coming Weeks –

CAMBRIDGE, Mass., June 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 dose administration for the first patient in a Phase 1, open-label, dose-escalation and expansion study of single agent AG-881, a small molecule that has shown in preclinical studies to fully penetrate the blood-brain barrier and inhibit isocitrate dehydrogenase-1 (IDH1) and IDH2 mutations in cancer models.

"The initiation of this study represents a significant milestone for Agios, as it marks the third program from our portfolio of IDH inhibitors to enter the clinic in less than two years," said Chris Bowden, M.D., chief medical officer of Agios. "We look forward to producing important early data to guide our future development plans and continuing to demonstrate Agios' leadership in cancer metabolism and drug development for IDH inhibitors."

"We are eager to explore the profile of AG-881 as we continue to investigate the role of IDH inhibitors for the treatment of patients with IDH mutant-positive tumors," said Howard Burris, M.D., Sarah Cannon Research Institute, an investigator for the study. "The Phase 1 study of this second-generation IDH inhibitor expands the opportunities for clinical development in the genetically defined spectrum of IDH1 or IDH2 mutant-positive tumors."

About the AG-881 Phase 1 Study in Advanced Solid Tumors, including Gliomas, with an IDH1 or IDH2 Mutation

The purpose of the Phase 1 multi-center, open-label study is to evaluate the safety, pharmacokinetics, pharmacodynamics and clinical activity of AG-881 in advanced solid tumors. AG-881 will be administered continuously as a single agent dosed orally in a 28-day cycle. The first portion of the study includes a dose-escalation phase in which cohorts of patients will receive ascending oral doses of AG-881 to determine the maximum tolerated dose (MTD) and/or the recommended Phase 2 dose based on safety and tolerability. The second portion of the study is a dose expansion phase where patients will receive AG-881 to further evaluate the safety, tolerability and clinical activity of the recommended Phase 2 dose. Please refer to www.clinicaltrials.gov for additional clinical trial information.

Upcoming Milestones for AG-881

A second dose-escalating and expansion trial, for patients with advanced IDH1 or IDH2 mutant-positive hematologic malignancies whose cancer has progressed on a prior IDH inhibitor therapy, is expected to begin shortly.

About IDH Mutations and Cancer

IDH1 and IDH2 are two metabolic enzymes that are mutated in a wide range of hematologic and solid tumor malignancies, including acute myeloid leukemia (AML) and gliomas. Normally, IDH enzymes help to break down nutrients and generate energy for cells. When mutated, IDH increases production of an oncometabolite 2-hydroxyglutarate (2HG) that alters the cells' epigenetic programming, thereby promoting cancer. 2HG has been found to be elevated in several tumor types. Agios believes that inhibition of the mutated IDH proteins may lead to clinical benefit for the subset of cancer patients whose tumors carry them.

Summary of Agios and Celgene Collaboration on IDH Mutant Inhibitors

Agios and Celgene entered a global, strategic collaboration in April 2010, and to date, three potential new distinct investigational medicines have emerged – the IDH2 mutant inhibitor, AG-221; the IDH1 mutant inhibitor, AG-120; and the pan-IDH mutant inhibitor, AG-881, which was recently announced as part of a new collaboration between the companies. These three investigational medicines aim to improve treatment outcomes for patients whose cancers carry IDH mutations, including difficult-to-treat AML and glioma, a type of aggressive brain tumor with a poor prognosis.

Each of these investigational medicines carries different financial terms and rights under the collaboration:

- **AG-221:** Celgene has worldwide development and commercialization rights for AG-221. Agios is eligible for up to $120 million in milestone payments and royalties on any net sales.
- **AG-120:** Agios retains U.S. development and commercialization rights, while Celgene has development and commercialization rights outside the U.S. Agios is eligible to receive royalties on any net sales outside the U.S. and up to $120 million in milestone payments. Celgene is eligible to receive royalties on any net sales in the U.S.
- **AG-881:** Joint worldwide development and 50/50 profit share collaboration. Agios is eligible to receive regulatory milestone payments up to $70 million.

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' product candidates targeting IDH mutations, including AG-881; its plans and timelines for the clinical development of AG-881; and the benefit of its strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "potential," "possible," "hope," "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, 2015, 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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