# Agios Pharmaceuticals to Present Clinical and Preclinical Data at the 2014 American Society of Hematology Annual Meeting

November 6, 2014

*New Data from Ongoing Phase 1 AG-221 Trial Accepted for Oral Presentation*

*Poster Presentation to Reveal First Clinical Data for AG-348, a Potential Treatment for Pyruvate Kinase Deficiency*

*Company to Host Investor Lunch and Webcast on Monday, December 8, 2014*

CAMBRIDGE, Mass., November 6, 2014 -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO) today announced that new data from the Phase 1 clinical trial for AG-221, a first-in-class IDH2 mutant inhibitor, will be presented at the 2014 American Society of Hematology (ASH) Annual Meeting and Exposition in San Francisco, December 6-9, 2014. In total, six abstracts led by Agios describing new clinical and preclinical data from the company's cancer metabolism and rare genetic disorders of metabolism programs have been accepted for presentation at ASH. Agios' cancer metabolism medicines are being developed in collaboration with Celgene.

"Agios has a strong presence at ASH this year, and we are very encouraged by the data we will have the opportunity to present across our programs," said David Schenkein, M.D., chief executive officer of Agios. "Data included in the abstract from our ongoing Phase 1 study of AG-221 in patients with advanced IDH2 mutant-positive hematologic malignancies show further evidence of safety and durable clinical activity among patients who desperately need new and better treatment options. In addition, the emerging data from the Phase 1 healthy volunteer studies for AG-348 allow us to advance into patients in early 2015 and lend support to the hypothesis that targeting the underlying defect of PK deficiency could potentially improve clinical outcomes."

Highlights of selected data presentations include:

AG-221: a first-in-class, oral, selective, potent inhibitor of the mutated IDH2 protein

- Data included in the AG-221 abstract released today provide new safety and efficacy data as of July 24, 2014 from the Phase 1 study of AG-221 in advanced hematologic malignancies. The oral presentation on December 7, 2014 will be updated with longer follow-up data and additional patients since the data in the submission of today's abstract.

AG-348: a novel, first-in-class, oral activator of pyruvate kinase-R (PKR) for the treatment of pyruvate kinase (PK) deficiency, a cause of hemolytic anemia

- The presentation for AG-348 will include the first data showing safety, pharmacokinetic data and effects on pharmacodynamics markers from the single ascending dose (SAD) and multiple ascending dose escalation (MAD) studies in healthy volunteers.

The accepted abstracts are listed below and are now available online on the ASH conference website: ash.confex.com/ash/2014/webprogram/start.html.

Oral Presentation

Date & Time: Sunday, December 7, 2014 at 4:30 p.m. PST (7:30 p.m. EST)
Session Title: 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: New Drugs I Session
Abstract number: 115
Presentation Title: AG-221, an Oral, Selective, First-in-Class, Potent Inhibitor of the IDH2 Mutant Metabolic Enzyme, Induces Durable Remissions in a Phase I Study in Patients with IDH2 Mutation Positive Advanced Hematologic Malignancies
Location: Moscone Center, West Building, 2001-2003-2014-2016

Poster Presentations

Date: Monday, December 8, 2014
Session Title: 101. Red Cells and Erythropoiesis, Structure and Function, Metabolism, and Survival, Excluding Iron: Poster III
Abstract number: 4007
Presentation Title: Phase I Single (SAD) and Multiple Ascending Dose (MAD) Studies of the Safety, Tolerability, Pharmacokinetics (PK) and Pharmacodynamics (PD) of AG-348, a First-in-Class Allosteric Activator of Pyruvate Kinase-R, in Healthy Subjects
Location: Moscone Center, West Building, Level 1

Date: Monday, December 8, 2014
Session Title: 101. Red Cells and Erythropoiesis, Structure and Function, Metabolism, and Survival, Excluding Iron: Poster III
Abstract number: 4010
Presentation Title: AG-348 Activation of Pyruvate Kinase in Vivo Enhances Red Cell Glycolysis in Mice
Location: Moscone Center, West Building, Level 1

Date: Monday, December 8, 2014
Session Title: 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster III Abstract number: 3737
Presentation Title: Evaluation of Pharmacokinetic-Pharmacodynamic (PKPD) Relationship of an Oral, Selective, First-in-Class, Potent IDH2 Inhibitor, AG-221, from a Phase 1 Trial in Patients with Advanced IDH2 Mutant Positive Hematologic Malignancies
Location: Moscone Center, North Building, Hall E

Date: Monday, December 8, 2014
Session Title: 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Poster III
Abstract number: 3734
Presentation Title: AG-120, an Oral, Selective, First-in-Class, Potent Inhibitor of Mutant IDH1, Reduces Intracellular 2HG and Induces Cellular Differentiation in TF-1 R132H Cells and Primary Human IDH1 Mutant AML Patient Samples Treated Ex Vivo
Location: Moscone Center, North Building, Hall E

Date: Monday, December 8, 2014
Session Title: 616. Acute Myeloid Leukemia: Novel Therapy, excludingTransplantation: Poster III
Abstract number: 3735
Presentation Title: AG-221, an Oral, Selective, First-in-Class, Potent IDH2-R140QMutant Inhibitor, Induces Differentiation in a Xenotransplant Model
Location: Moscone Center, North Building, Hall E

**Investor Lunch and Webcast Information**
Agios will host an investor lunch on Monday, December 8, 2014 beginning at 12:00 p.m. PST (3:00 p.m. EST) in San Francisco to review data presented at ASH, including new data from the ongoing Phase 1 study of AG-221 and data from AG-348's Phase 1 healthy volunteer studies. The event will be webcast live and can be accessed under "Events & Presentations" in the Investors and Media section of the company's website at www.agios.com.

**About AG-221 and IDH2**
IDH1 and IDH2 are metabolic enzymes that are mutated in a wide range of hematologic and solid tumor malignancies, including AML. Normally, IDH enzymes help to break down nutrients and generate energy for cells. When mutated, IDH creates a molecule that alters the cells' genetic programming, and instead of maturing, the cells remain primitive and proliferate quickly. Agios believes that inhibition of these mutated proteins may lead to clinical benefit for the subset of cancer patients whose tumors carry them. AG-221 was developed by Agios as a selective, potent inhibitor of the mutated IDH2 protein. While most cancer treatments attempt to destroy the cancerous cells, AG-221 uniquely attacks its target – mutated IDH2 – and aids the maturation of the cells into functioning blood cells. AG-221 has received orphan drug designation for AML and fast track designation for patients with IDH2-mutant AML.

**About Agios/Celgene Collaboration**
AG-221 is a part of Agios' global strategic collaboration with Celgene Corporation, an integrated global biopharmaceutical company. In June 2014, Celgene exercised its exclusive option to license AG-221 and gained worldwide development and commercialization rights for AG-221. Agios continues to conduct early clinical development activities within the AG-221 development program. The companies are also collaborating on the development of AG-120, which is being studied in two Phase 1 trials in patients whose hematologic malignancies and solid tumors carry an IDH1 mutation. Agios retains U.S. development and commercialization rights for AG-120, and Celgene has an exclusive option to the ex-U.S. rights.

**About AG-348 and Pyruvate Kinase (PK) Deficiency, a Rare, Inherited Hemolytic Anemia**
Pyruvate kinase 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 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 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 the potential of IDH1/2 and pyruvate kinase-R mutations as therapeutic targets; the potential benefits of Agios' drug candidate AG-221 targeting IDH1/2 or pyruvate kinase-R mutations, including AG-221, AG-120 and AG-348; its plans and timelines for the clinical development of AG-221, AG-120 and AG-348; its plans and timelines for the clinical development of AG-221, AG-120 and AG-348; its plans regarding future data presentations; 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 June 30, 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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