Agios to Present Clinical and Preclinical Data at the 20th Congress of the European Hematology Association

May 21, 2015

New Data from Three Lead Programs in IDH Mutant Positive Cancers and PK Deficiency

Company to Host Conference Call and Webcast on Friday, June 12, 2015

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 21, 2015-- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the fields of cancer metabolism and rare genetic disorders of metabolism, today announced that new clinical and preclinical data from the company's lead programs will be presented at the 20th Congress of the European Hematology Association (EHA) taking place June 11-14, 2015 in Vienna. Agios' cancer metabolism medicines are being developed in collaboration with Celgene.

"EHA is an important meeting for us this year as we share new and encouraging data for each of our three lead clinical programs," said Chris Bowden, M.D., chief medical officer of Agios. "Data from the ongoing Phase 1 studies of our mutant-IDH inhibitors in hematologic malignancies support the continued development of these molecules as we move into global registration programs. In addition, complete findings from our MAD study of AG-348, coupled with the natural history study of PK deficiency from Boston Children's Hospital, inform our understanding of the disorder and dosing as we prepare to initiate a Phase 2 study in patients in the coming weeks."

Highlights of selected data presentations include:

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

- A poster presentation will provide new safety and efficacy data from the ongoing Phase 1 study of AG-221 in advanced IDH2-mutant hematologic malignancies, including longer follow-up data, additional patients and early molecular data from the Phase 1 dose escalation study, in addition to initial data from the expansion cohorts initiated in October 2014.

AG-120: a first-in-class, orally available, selective, potent inhibitor of the mutated IDH1 protein

- A poster presentation will provide new safety and efficacy data from the ongoing Phase 1 study of AG-120 in advanced IDH1-mutant hematologic malignancies, including longer follow-up data and additional patients since the initial data presentation in November 2014 at the 26th Annual EORTC-NCI-AACR Symposium on Molecular Targets and Cancer Therapeutics.

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

- An oral presentation for AG-348 will include the final data showing safety, tolerability, pharmacokinetic data and effects on pharmacodynamics markers from the multiple ascending dose escalation (MAD) study in healthy volunteers.
- A poster presentation and an e-poster from Boston Children's Hospital will show the first data from a natural history study of PK deficiency.

The accepted abstracts are listed below and are now available online on the EHA conference website: http://www.ehaweb.org/congress-and-events/annual-congress-2/20th-congress/.

The schedule for the oral presentation by Agios is as follows:

Date & Time: Friday, June 12, 2015 at 12:00 p.m. CEST
Title: Phase 1 Multiple Ascending Dose Study of the Safety, Tolerability, and Pharmacokinetics/Pharmacodynamics of AG-348, a First-in-class Allosteric Activator of Pyruvate Kinase-R, in Healthy Subjects
Session: Red Cells: Novel Clinical Aspects
Abstract: S138
Location: Room Strauss 1
Presenter: Sam Agresta, M.D., M.P.H., T.M., Agios Pharmaceuticals

The details for poster presentations and e-posters by Agios and/or its collaborators are as follows:

Date & Time: Friday, June 12, 2015 at 5:15 p.m. CEST
Title: Categorization of Clinical Severity in Pyruvate Kinase Deficiency (PKD) in an International, Observational Cohort
Session: Red Blood Cells and Iron – Clinical 1
Abstract: P375
Location: Poster area (Hall C)
Presenter: Rachael Grace, M.D., Dana-Farber Boston Children's Cancer and Blood Disorder Center

Date & Time: Saturday, June 13, 2015 at 5:15 p.m. CEST
Title: AG-221, An Oral, Selective, First-in-class, Potent Inhibitor of the IDH2 Mutant Enzyme, Induced Durable Responses in a Phase 1 Study of IDH2

Mutation-Positive Advanced Hematologic Malignancies
Session: Acute Myeloid Leukemia – Clinical 3
Abstract: P569
Location: Poster area (Hall C)

Date & Time: Saturday, June 13, 2015 at 5:15 p.m. CEST
Title: Clinical Safety and Activity of AG-120, a First-in-class, Potent Inhibitor of the IDH1 Mutant Protein, in a Phase 1 Study of Patients with Advanced IDH1-Mutant Hematologic Malignancies
Session: Acute Myeloid Leukemia – Clinical 3
Abstract: P563
Location: Poster area (Hall C)

Date & Time: Saturday, June 13, 2015 at 5:15 p.m. CEST
Title: Pharmacokinetic/Pharmacodynamic Evaluation of AG-120, a Potent Inhibitor of the IDH1 Mutant Protein, in a Phase 1 Study of IDH1-Mutant Advanced Hematologic Malignancies
Session: Acute Myeloid Leukemia - Clinical
Abstract: P572
Location: Poster area (Hall C)

Date & Time: Saturday, June 13, 2015 at 5:15 p.m. CEST
Title: Preclinical Pharmacokinetic/Pharmacodynamic Relationships for AG-348, an Investigational Small-Molecule Activator of Pyruvate Kinase
Session: Non-malignant Hematopoietic Disorders
Abstract: P751
Location: Poster area (Hall C)

E-Poster Title: Pharmacokinetic/Pharmacodynamic (PK/PD) Evaluation of AG-221, a Potent Mutant IDH2 Inhibitor, from a Phase 1 Trial of Patients with IDH2-Mutation Positive Hematologic Malignancies

E-Poster Title: The Clinical Features and Treatment of Iron Overload in Pyruvate Kinase Deficiency (PKD): Data from the PKD Natural History Study (NHS)

Conference Call Information

Agios will host a conference call and webcast from the congress to review the data on Friday, June 12, 2015 beginning at 8:00 a.m. ET (2:00 p.m. CEST). To participate in the conference call, please dial (877) 377-7098 (domestic) or (631) 291-4547 (international) and refer to conference ID 53010830. The webcast will be accessible live or in archived form under "Events & Presentations" in the Investors and Media section of the company's website at www.agios.com.

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 IDH1/IDH2 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 regarding future data presentations; 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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