Agios Pharmaceuticals Announces Initiation of Four Expansion Cohorts in Phase 1 Study of AG-221

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Once-Daily Oral Dose Selected From Ongoing Phase 1 Study of AG-221 in Hematologic Malignancies

Data From AG-120 to be Presented at EORTC-NCI-AACR 2014; AG-221 and AG-348 Data to be Presented at 2014 American Society of Hematology Annual Meeting

Company to Webcast R&D Day Today

CAMBRIDGE, Mass., Oct. 15, 2014 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq:AGIO), a leader in the fields of cancer metabolism and rare genetic disorders of metabolism, today announced the initiation of four expansion cohorts in its ongoing Phase 1 study of AG-221, a first-in-class, selective, potent IDH2 mutant inhibitor. The Phase 1 expansion cohorts will assess the safety and tolerability of AG-221 at 100 mg once daily in approximately 100 patients with IDH2-mutant hematologic malignancies, including acute myelogenous leukemia (AML). Members of Agios' management team will provide an overview of this trial expansion at its Research and Development (R&D) Day meeting today.

"AG-221 is the first targeted investigational medicine to show clinical activity in patients with an IDH2 mutation, validating IDH2 as an important target for patients with AML and potentially other cancers," said David Schenkein, M.D., chief executive officer at Agios. "Having selected the dose of AG-221 based on our ongoing Phase 1 study, we are now advancing to the next stage of development."

"At our R&D Day today, we will delve deeply into the science of Agios. We will also highlight the disease areas in which we believe our precision medicine approach has the potential to establish new treatment paradigms and deliver important medicines to patients. We are encouraged by the progress of all three of our investigational medicines and look forward to presenting data from our Phase 1 study of AG-120 at EORTC-NCI-AACR in November, as well as data from AG-221 and AG-348 studies at the 2014 American Society of Hematology Annual Meeting in December," Dr. Schenkein continued.

Webcast

A live webcast of the company's R&D Day will begin today at 9:00 a.m. EDT and can be accessed under "Events & Presentations" in the Investors and Media section of the company's website at agios.com. A replay of the webcast will be archived on the Agios website for 30 days following the presentation.

About AG-221 expansion cohorts

The four expansion cohorts of the AG-221 Phase 1 study will evaluate patients with relapsed or refractory AML who are 60 years of age and older and transplant ineligible, relapsed or refractory AML patients under age 60, untreated AML patients who decline standard of care chemotherapy, and patients with other IDH2-mutant positive hematologic malignancies. AG-221 will be administered as a single agent at a 100 mg once daily oral dose, in 28-day cycles. The study aims to enroll 25 patients in each cohort at 15 clinical trial sites in the United States and France. The primary objectives are to confirm the safety and clinical activity of AG-221 in homogenous patient populations with AML and other IDH2 mutant positive hematologic malignancies. Refer to www.clinicaltrials.gov for additional clinical trial details.

In the ongoing Phase 1 dose-escalation study, the maximum-tolerated dose has not yet been established and dose escalation continues. Agios and its partner Celgene selected the dose for the expansion cohorts based on pharmacokinetic, pharmacodynamics, and safety results across the range of doses studied in dose escalation.

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 Acute Myelogenous Leukemia (AML)

AML is a cancer of blood and bone marrow characterized by rapid disease progression, and is the most common acute leukemia affecting adults. AML incidence significantly increases with age and the median affected age is 66, according to the American Cancer Society. Treatment options are limited: less than 10 percent of U.S. patients are eligible for bone marrow transplant, and the vast majority of patients do not respond to chemotherapy and progress to relapsed or refractory AML. The five-year survival rate for AML is approximately 20 to 25 percent. Approximately 9 to 13 percent of AML patients in the U.S. carry an IDH2 mutation.

About Agios/Celgene Collaboration

AG-221 is a part of Agios' global strategic collaboration with Celgene Corporation, a leading biotechnology company. In June 2014, Celgene exercised its exclusive option to license AG-221. Under the terms of the agreement, Celgene gained worldwide development and commercialization rights for AG-221. Agios continues to conduct early clinical development and regulatory activities within the AG-221 development program in collaboration with Celgene. 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 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 cancer metabolism and rare genetic disorders of metabolism 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 IDH1/2 and pyruvate kinase R mutations as therapeutic targets; the potential benefits of Agios' drug 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," "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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