Agios Announces Initiation of Phase 1/2 Frontline Combination Study of AG-221 or AG-120 with VIDAZA® (azacitidine for injection) in Newly Diagnosed Acute Myeloid Leukemia (AML) Patients Not Eligible for Intensive Chemotherapy

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CAMBRIDGE, Mass., March 30, 2016 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the fields of cancer metabolism and rare genetic metabolic disorders, today announced the initiation of a Phase 1/2, multicenter, international, open-label study, sponsored by Celgene Corporation, of AG-221 or AG-120 in combination with VIDAZA® (azacitidine for injection) in patients with newly diagnosed acute myeloid leukemia (AML) with an isocitrate dehydrogenase (IDH) mutation who are not eligible for intensive chemotherapy. AG-221 and AG-120 are first-in-class, oral, selective, potent inhibitors of mutant IDH2 and IDH1, respectively, and are being developed in collaboration with Celgene.

“Many newly diagnosed AML patients cannot tolerate intensive chemotherapy, which limits their available treatment options,” said Anthony S. Stein, M.D., study investigator and co-director of the leukemia program at City of Hope Cancer Center. “Based on the safety and efficacy demonstrated in clinical studies of AG-221 and AG-120 in relapsed / refractory AML, there is potential to provide a new treatment option for newly diagnosed IDH mutant AML patients by combining these therapies with VIDAZA® in the frontline setting.”

“We are rapidly executing our frontline strategy for our IDH inhibitors, having now initiated a second study in newly diagnosed AML patients,” said Chris Bowden, M.D., chief medical officer of Agios. “By combining AG-221 or AG-120 with VIDAZA ® at the onset of diagnosis, we hope to demonstrate benefit for patients with IDH mutant AML who are not eligible for intensive chemotherapy.”

About the Phase 1/2 Frontline Combination Trial of AG-221 or AG-120 with VIDAZA® in Newly Diagnosed AML Patients Not Eligible for Intensive Chemotherapy

The Phase 1/2, multicenter, international, open-label clinical trial will evaluate the safety and clinical activity of AG-221 or AG-120 in combination with VIDAZA® in patients with newly diagnosed AML with an IDH2 and/or IDH1 mutation who are not eligible for intensive chemotherapy. The study consists of a Phase 1b dose-escalation stage and a Phase 2 randomized stage.

The study will evaluate AG-221 administered at an initial oral dose of 100 mg once daily in patients with an IDH2 mutation or AG-120 administered at an initial oral dose of 500 mg once daily in patients with an IDH1 mutation. AG-221 or AG-120 will be administered continuously in a 28-day cycle with VIDAZA® at the standard 75 mg/m^2 daily dose for 7 days of each 28-day cycle.

The primary endpoint of the Phase 1b stage of the trial is to determine safety and tolerability and to establish the recommended Phase 2 dose of AG-221 or AG-120 in combination with VIDAZA®. The primary endpoint of the Phase 2 stage of the trial is to determine the efficacy of the combination of AG-221 or AG-120 with VIDAZA® compared with VIDAZA® alone. Secondary endpoints include evaluation of safety, characterization of pharmacokinetics and evaluation of effects on health-related quality-of-life outcomes. This study will enroll up to 150 patients.

Please refer to www.clinicaltrials.gov for additional clinical trial details.

About Acute Myelogenous Leukemia (AML)

AML, a cancer of blood and bone marrow characterized by rapid disease progression, is the most common acute leukemia affecting adults. Undifferentiated blast cells proliferate in the bone marrow rather than mature into normal blood cells. AML incidence significantly increases with age, and according to the American Cancer Society, the median age of onset is 66. Less than 10 percent of U.S. AML patients are eligible for bone marrow transplant, and the vast majority of patients do not respond to chemotherapy and progress to relapsed/refractory AML. The five-year survival rate for AML is approximately 20 to 25 percent. IDH1 mutations are present in about 6 to 10 percent of AML cases. IDH2 mutations are present in about 9 to 13 percent of AML cases.

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 AML. 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.

About Agios

Agios is focused on discovering and developing novel investigational medicines to treat cancer and rare genetic metabolic disorders 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.

About Agios/Celgene Collaboration

AG-221, AG-120 and AG-881 are part of Agios' global strategic collaboration with Celgene Corporation. Under the terms of the collaboration, Celgene has worldwide development and commercialization rights for AG-221 (CC-90007). Agios continues to conduct clinical development activities within the

AG-221 development program and is eligible to receive up to $120 million in payments on achievement of certain milestones and royalties on net sales. For AG-120, Agios retains U.S. development and commercialization rights and Celgene retains development and commercialization rights outside the U.S. Celgene is eligible to receive royalties on net sales in the U.S. Agios is eligible to receive royalties on net sales outside the U.S. and up to $120 million in payments on achievement of certain milestones. For AG-881, the companies have a joint worldwide development and 50/50 profit share collaboration, and Agios is eligible to receive regulatory milestone payments of up to $70 million.

VIDAZA® is a registered trademark of Celgene Corporation.

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-221 and AG-120; its plans and timelines for the clinical development of AG-221 and AG-120; 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 agreements 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' Annual Report on Form 10-K for the quarter ended December 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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