Agios Announces New Data from Ongoing Phase 1 Trial of AG-120 Showing Durable Clinical Activity in Patients with Advanced Hematologic Malignancies

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IDH1-Mutant Inhibitor Shows Durable Responses of Up to 11 Months in Patients with Advanced Acute Myeloid Leukemia (AML) and Other Blood Cancers

Three Expansion Cohorts and Global Registration-Enabling Program Remain on Track

Company to Host Conference Call and Webcast Today

CAMBRIDGE, Mass. & VIENNA--(BUSINESS WIRE)--Jun. 12, 2015-- Agios Pharmaceuticals, Inc. (Nasdaq:AGIO), a leader in the fields of cancer metabolism and rare genetic disorders of metabolism, today announced new data from the ongoing Phase 1 study evaluating single agent AG-120, a first-in-class, oral, selective, potent inhibitor of mutant isocitrate dehydrogenase-1 (IDH1), in advanced hematologic malignancies presented at the 20th Congress of the European Hematology Association (EHA) taking place June 11-14, 2015 in Vienna.

Data as of May 1, 2015 from 57 patients with advanced hematologic malignancies showed durable clinical activity and a favorable safety profile, with 25 patients on study as of the analysis. The study had an overall response rate of 31 percent (16 of 52 response-evaluable patients) and a complete remission rate of 15 percent (8 of 52 response-evaluable patients). Data continue to show durable clinical activity for AG-120, with responding patients on treatment for up to 11 months, and an estimated 79 percent of responders on treatment at three months. The overall safety profile remains consistent with 40 additional patients treated as of the last analysis.

“The durable clinical activity observed with AG-120 in such a refractory patient population is impressive,” said Stéphane de Botton, M.D., the principal investigator at the Institut de Cancérologie Gustave Roussy, Villejuif, France. “These findings provide additional evidence that AG-120 can inhibit the IDH1-mutant protein allowing for cancer cells to appropriately mature. AG-120 has the potential to improve outcomes in patients with IDH1 mutant cancers.”

“These encouraging data represent the tremendous progress to date in our AG-120 program, as this therapy is proving to be well tolerated and effective, with an objective response rate of 31 percent of treated patients and duration on study up to 11 months,” said Chris Bowden, M.D., chief medical officer of Agios. “Along with the insight gained from the AG-221 program, we are excited to move the AG-120 program forward rapidly with our partner Celgene. Our goal is to reach patients in need quickly, as evidenced by the recent announcement of our plans to initiate three expansion cohorts as part of the Phase 1 study.”

About the Ongoing Phase 1 Trial for AG-120 in Advanced Hematologic Malignancies

AG-120 is being evaluated in an ongoing Phase 1 trial in patients with AML and other IDH1-mutant positive advanced hematologic malignancies. Data reported are from patients receiving AG-120 administered from 100 mg to 1,200 mg total daily doses as of May 1, 2015. The median age of these patients is 68 (ranging from 38-89). Treatment with AG-120 showed substantial reduction in the plasma levels of the oncometabolite 2-hydroxglutarate (2HG) to the level observed in healthy volunteers.

This new data reflects responses in the evaluable population, which includes all patients with a pre-AG-120 screening assessment and day 28 or later response assessment or an earlier discontinuation for any reason. Patients with a screening assessment who were still on treatment, but had not reached the day 28 disease assessment, were excluded.

Safety Data

A safety analysis was conducted for all 57 treated patients as of May 1, 2015.

- The majority of adverse events reported by investigators were mild to moderate, with the most common being fatigue, diarrhea, pyrexia and nausea.
- 35 serious adverse events (SAEs) were reported, the majority being disease related, with four cases of leukocytosis potentially related to AG-120.
- A maximum tolerated dose (MTD) has not been reached.
- 13 deaths were reported, and all were considered unrelated to AG-120.

Efficacy Data

Sixteen out of 52 response-evaluable patients achieved investigator-assessed objective responses for an overall response rate of 31 percent as of May 1, 2015.

- Of the 16 patients who achieved an objective response, there were eight complete remissions (CR), one complete remission with incomplete platelet recovery (CRp), three marrow complete remissions (mCR) and four partial remissions (PR).

- Responses were durable, with duration on study drug as long as 11 months and ongoing. As of the analysis date, an estimated 79 percent of responders were on treatment for three months or longer, and 50 percent of responders were on treatment for six months or longer.

Upcoming Milestones for AG-120

Agios studies in IDH1-mutated solid and hematological tumors are ongoing or planned for 2015/2016 to further support the speed and breadth of development of AG-120.

- Initiate three expansion cohorts to evaluate AG-120 in 175 patients with IDH1-mutated advanced hematologic malignancies (125 in relapsed and/or refractory AML, 25 in untreated AML and 25 in basket IDH1-mutant positive cancers).
- Present first data from the Phase 1 trial in advanced solid tumors at a medical conference in the second half of 2015.
- Begin combination trials to evaluate AG-120 as a potential frontline treatment of IDH1-mutated AML and a broad range of hematologic malignancies in the second half of 2015.
- Intend to initiate a global registration-enabling Phase 3 study in AML patients that harbor an IDH1 mutation in the first half of 2016.

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/Celgene Collaboration

AG-120, the IDH2-mutant inhibitor AG-221 and the pan-IDH mutant inhibitor 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. 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. Celgene has an exclusive license outside the United States. 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.

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 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.

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 mutations, including AG-221, AG-120, and AG-881; its plans and timelines for the clinical development of AG-120; its plans regarding future data presentations for 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 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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