Agios Pharmaceuticals Announces that Celgene Exercised its Option to License AG-221 Under Global Strategic Collaboration

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Major Milestone in Landmark Collaboration Between Agios and Celgene

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 13, 2014-- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the fields of cancer metabolism and inborn errors of metabolism (IEMs), today announced that its partner Celgene Corporation has exercised its option to an exclusive worldwide license to AG-221, an oral, first-in-class, potent inhibitor of the mutant IDH2 protein. Under the terms of the agreement, the option to license extended to Celgene through the end of Phase 1, but AG-221 has been exercised early based on the Phase 1 data generated to date. AG-221 is currently in a Phase 1 dose escalation study in patients that harbor an IDH2 mutation with advanced hematologic malignancies, including acute myeloid leukemia (AML).

“We are pleased with Celgene’s decision to license AG-221, as we believe it reflects the strength of our progress with this product candidate and underscores Agios’ and Celgene’s commitment to precision medicine,” said David Schenkein, M.D., chief executive officer of Agios. “Celgene brings global reach, significant expertise and financial resources to the AG-221 program, and we look forward to our continued collaboration to increase the scope and efforts directed to IDH2 and broadly advance this important potential cancer medicine.”

“Agios’ AG-221 candidate is simultaneously advancing convergent fields, including cancer metabolism, epigenetics and precision medicine. The emerging Phase 1 clinical data validate the preclinical and mechanistic work on IDH2 mutations in AML, and most importantly, advance a highly promising drug candidate for treatment of molecularly selected patients,” said Thomas Daniel, M.D., president of research & early development at Celgene. “Celgene looks forward to deploying our worldwide development capabilities in hematological malignancies and to working with Agios to accelerate development.”

Agios and Celgene entered into a global strategic collaboration in April 2010 to develop new therapeutics targeting cancer metabolism. By exercising its exclusive option under the terms of the agreement, Celgene gains worldwide development and commercialization rights for AG-221. Agios, in addition to contributing its scientific and translational expertise, will continue to conduct early clinical development and regulatory activities within the AG-221 development program in collaboration with Celgene. Celgene is responsible for all development costs for AG-221. Agios is eligible for up to $120 million in milestone payments and a tiered royalty on any net sales. Agios also has the right to conduct a portion of any commercialization activities for AG-221 in the United States.

AG-221 is part of Agios’ IDH portfolio that also includes the IDH1 mutant inhibitor AG-120, which the company continues to develop and is in Phase 1 clinical trials in advanced solid tumors and hematologic malignancies. Agios retains U.S. rights to the IDH1 program, and Celgene has an exclusive option to ex-U.S. rights for the program. Agios continues to advance its discovery and research of cancer metabolism targets.

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. The prevalence of IDH is expected to evolve as genomic analysis of tumors increase. Agios’ research revealed the potential of IDH1 and IDH2 mutations as novel therapeutic targets in cancer, which may lead to clinical benefit for the subset of cancer patients whose tumors carry them. Patients carry either an IDH1 or IDH2 mutation, but not both.

Agios is developing two oral, first-in-class IDH mutant inhibitors: AG-221 is an IDH2 mutant inhibitor, and AG-120 is an IDH1 mutant inhibitor. AG-221 is currently being evaluated in a Phase 1 dose-escalation study in patients with advanced hematologic malignancies, including AML, one of the most common types of leukemia in adults. AG-120 is currently being evaluated in two Phase 1 trials, one in hematologic malignancies and another in solid tumors. Both compounds were discovered and developed in the laboratory of Agios.

About Cancer Metabolism

Cancer metabolism is a new and exciting field of biology that provides a novel approach to treating cancer. Cancer cell metabolism is marked by profound changes in nutrient requirements and usage to ensure cell proliferation and survival. Research in the field has demonstrated that cancer cells become addicted to certain fuel sources and metabolic pathways. In cancer, this metabolic reprogramming is coordinated with proliferative signaling and regulated by the same oncogenes and tumor suppressor genes to ensure efficient proliferation. Glycolysis (sugar metabolism), fatty acid metabolism and autophagy (self metabolism) are three pathways shown to play a critical role in cancer metabolism. Identifying and disrupting certain enzymes in these, and perhaps other, metabolic pathways provides a powerful intervention point for discovery and development of cancer therapeutics.

About Agios Pharmaceuticals, Inc.

Agios Pharmaceuticals is focused on discovering and developing novel drugs to treat cancer and inborn errors of metabolism, or IEMs, which are rare genetic metabolic diseases, 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 lead product candidates in cancer metabolism and IEMs 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 benefits of Agios' product candidates targeting IDH1 and IDH2, including 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," "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, 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.

Source: Agios Pharmaceuticals, Inc.

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