



AgiOS Pharmaceuticals Selects Third Novel IDH Mutant Inhibitor, AG-881, for Clinical Development

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- *Brain-penetrant, pan-IDH mutant inhibitor broadens pipeline for treatment of patients with IDH mutant positive cancers*
- *New worldwide development and profit share collaboration for AG-881 entered into by Agios and Celgene*
- *Expect to initiate clinical development for AG-881 in second quarter 2015*

CAMBRIDGE, Mass., April 29, 2015 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq:AGIO), a leader in the fields of cancer metabolism and rare genetic disorders of metabolism, today announced that it plans to advance into clinical development AG-881, a small molecule that has shown in preclinical studies to fully penetrate the blood brain barrier and inhibit isocitrate dehydrogenase-1 (IDH1) and IDH2 mutant cancer models, in collaboration with its cancer metabolism partner Celgene Corporation. The companies have entered into a new joint worldwide development and profit share collaboration for AG-881 and plan to initiate clinical development of AG-881 in the second quarter of 2015. AG-881 will be the third IDH mutant inhibitor discovered by Agios to enter clinical development.

"The addition of our third IDH mutant inhibitor to our growing pipeline is an exciting milestone for Agios and underscores our goals to lead the scientific understanding of cancer metabolism and help as many patients as possible with an IDH mutant positive cancer," said David Schenkein, M.D., chief executive officer of Agios. "AG-221 and AG-120 remain our lead medicines in clinical development and are advancing rapidly. We believe the addition of AG-881, given its unique profile, provides added flexibility to our portfolio of IDH inhibitors. Based on our preclinical findings, it has the potential to support our ongoing development effort to provide treatment options to patients with glioma, and it represents a possible second-generation molecule for both AG-221 and AG-120 in IDH mutant tumors. We look forward to generating data for AG-881 to inform our future development plans."

Under the terms of the new AG-881 collaboration, Agios will receive an initial payment of \$10 million in the second quarter of 2015 and is eligible to receive regulatory milestone payments of up to \$70 million. Agios and Celgene will jointly collaborate on the worldwide development program for AG-881, sharing development costs 50/50 worldwide. The two companies have agreed to share any worldwide profits 50/50, with Celgene booking worldwide commercial sales. Agios would lead commercialization in the U.S. with both companies sharing equally in field-based commercial activities, and Celgene would lead commercialization ex-U.S. with Agios providing one third of field-based commercial activities in the major E.U. markets.

Summary of Agios and Celgene Collaboration on IDH Mutant Inhibitors

Agios and Celgene entered a global, strategic collaboration in April 2010 and, to date, three potential new distinct investigational medicines have emerged – the IDH2 mutant inhibitor, AG-221; the IDH1 mutant inhibitor, AG-120; and the pan-IDH mutant inhibitor, AG-881, which as described above is now part of a new collaboration between the companies. These three investigational medicines aim to improve the treatment outcomes for patients whose cancers carry these IDH mutations, including difficult to treat acute myelogenous leukemia and glioma, a type of aggressive brain tumor with poor prognosis. Each of these investigational medicines carries different financial terms and rights under the collaboration, including:

- **AG-221:** Celgene has worldwide development and commercialization rights for AG-221. Agios is eligible for up to \$120 million in milestone payments and royalties on any net sales.
- **AG-120:** Agios retains U.S. development and commercialization rights, while Celgene has development and commercialization rights outside the U.S. Agios is eligible to receive royalties on any net sales outside the U.S. and up to \$120 million in milestone payments. Celgene is eligible to receive royalties on any net sales in the U.S.
- **AG-881:** Joint worldwide development and 50/50 profit share collaboration. Agios is eligible to receive regulatory milestone payments up to \$70 million.

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, and plans relating to, the collaboration with Celgene; the potential benefits of AG-221, AG-120 and AG-881; and Agios' plans to generate data from AG-881 to inform its future development plans; and the benefit of Agios' strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "goal," "intend," "may," "plan," "possible," "potential," "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 AG-881 or any other 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; 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 year ended December 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.

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