Agios Submits New Drug Application to the FDA for Ivosidenib for the Treatment of Patients with Relapsed/Refractory AML and an IDH1 Mutation

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- Ivosidenib Has Potential to be a First-in-Class Therapy for Patients with R/R AML and an IDH1 mutation -

- Company Also Announces FDA Clearance of Investigational New Drug Application (IND) for AG-270 Targeting MTAP-Deleted Tumors; Phase 1 Study to Initiate in First Quarter 2018 -

CAMBRIDGE, Mass., Dec. 26, 2017 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, today announced that it has submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for ivosidenib (AG-120), an investigational oral treatment for patients with relapsed or refractory acute myeloid leukemia (R/R AML) and an isocitrate dehydrogenase-1 (IDH1) mutation. Agios has requested priority review for the application, which, if granted, could result in a six-month review process.

“People with AML who have relapsed or refractory disease have limited treatment options available to them, and it is our hope that we can change that,” said Chris Bowden, M.D., chief medical officer at Agios. “Earlier this month at the ASH Annual Meeting, we presented compelling single agent ivosidenib data demonstrating durable responses in high-risk relapsed or refractory AML patients with an IDH1 mutation. These data highlight the potential for ivosidenib to be a first-in-class therapy for patients with R/R AML and an IDH1 mutation.”

The NDA is supported by data from the ongoing Phase 1 dose-escalation and expansion study of ivosidenib in patients with advanced hematologic malignancies and an IDH1 mutation. Ivosidenib is wholly owned by Agios. The FDA has a 60-day filing review period to determine whether the NDA is complete and acceptable for filing.

AG-270 IND Clearance

In addition, Agios announced that the FDA has completed their 30-day safety review of the investigational new drug (IND) application for AG-270, the development candidate targeting MTAP-deleted tumors, and has granted IND clearance to proceed with the proposed Phase 1 dose-escalation trial in multiple tumor types carrying an MTAP deletion. Agios expects to initiate the Phase 1 trial in the first quarter of 2018.

About Agios
Agios is focused on discovering and developing novel investigational medicines to treat cancer and rare genetic 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 an approved oncology precision medicine and multiple first-in-class investigational therapies 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 www.agios.com.

About the MTAP Pathway Program
The program focused on MTAP deleted cancers is part of a 2016 global co-development and co-commercialization agreement with Celgene focused on metabolic immuno-oncology. Celgene has the option to participate in a worldwide 50/50 cost and profit share with Agios, under which Agios is eligible for up to $169 million in clinical and regulatory milestone payments for the program. As described in a 2016 Cell Reports publication, Agios discovered a novel pathway in MTAP-deleted tumors which, when inhibited, results in robust anti-tumor activity in animal models. This pathway can be modulated by small molecule inhibitors, as demonstrated in a preclinical data presentation at the Keystone Tumor Metabolism meeting.

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’ plans, strategies and expectations for its and its collaborator’s preclinical, clinical and commercial advancement of its drug development programs including ivosidenib and AG-270; the potential benefits of its product candidates; its plans for regulatory submissions; and the potential benefit of its strategic plans and focus. The words “anticipate,” “expect,” “intend,” “potential,” “milestone,” “goal,” “will,” “on track,” “upcoming,” 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 or its collaborator, Celgene, 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’ public filings with the Securities and Exchange Commission. 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, except as required by law.

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