Agios Announces First Patient Dosed with MAT2A Inhibitor AG-270 in Phase 1 Study in Patients with Advanced Solid Tumors or Lymphoma with an MTAP Deletion

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MTAP Deletion Present in Approximately 15 Percent of All Cancers

CAMBRIDGE, Mass., March 19, 2018 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, today announced dosing of the first patient in a Phase 1 study of AG-270, a first-in-class methionine adenosyltransferase 2a (MAT2A) inhibitor. This open-label, dose-escalation and expansion study investigates AG-270 in patients with solid tumors or lymphoma with deletion of the metabolic gene methylthioadenosine phosphorylase (*MTAP*).

“In addition to significant milestones for our late-stage portfolio this year, we are pleased to demonstrate the continued productivity of our research engine by advancing our sixth internally discovered molecule into the clinic,” said Scott Biller, Ph.D., chief scientific officer of Agios. “As a first-in-class MAT2A inhibitor, AG-270 has the potential to benefit the large number of patients whose cancer is characterized by the loss of *MTAP*. We look forward to conducting the early clinical work that will explore the pharmacology and clinical activity of MAT2A inhibition in tumors carrying this deletion.”

“We are excited to participate in the development of this novel and targeted approach to cancer treatment,” said Howard (Skip) Burris, M.D., chief medical officer and president of clinical operations at Sarah Cannon. “Bringing AG-270 into the clinic represents an opportunity for Sarah Cannon and Agios to continue a partnership that began with the IDH inhibitors and remains focused on transforming cancer care and personalizing treatment.”

AG-270 Phase 1 Study Design

The purpose of this Phase 1 multi-center, open-label study is to evaluate the safety, pharmacokinetics, pharmacodynamics and clinical activity of AG-270 in approximately 50 patients with advanced solid tumors or lymphoma with *MTAP* deletion. AG-270 will be administered as a single agent dosed orally once daily in 28-day cycles. The first part of the study is a dose-escalation phase in which cohorts of patients will receive ascending doses of AG-270 to determine the maximum tolerated dose (MTD) or optimal dose. The second part of the study is a dose expansion phase where additional patients will receive AG-270 at the MTD or optimal dose to further evaluate its safety, tolerability and clinical activity as a potential dose for future studies. Patients must have evidence of loss of the *MTAP* protein from their tumor tissue, or evidence of loss of the *CDKN2A* tumor suppressor gene (commonly co-deleted with the *MTAP* gene), in order to be eligible for the study. Please refer to www.clinicaltrials.gov for additional clinical trial information.

About the MAT2A Inhibitor AG-270
AG-270 is part of a 2016 global research collaboration agreement with Celgene Corporation. Through Phase 1 dose escalation, Celgene has the option, for a fee of at least $30 million, to participate in a worldwide cost and profit share with Agios. Upon exercise of the option the parties will share all development costs, subject to specified exceptions, and any profits on net sales and Agios will be 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 that MAT2A is a component of a novel pathway in *MTAP*-deleted tumors which, when inhibited, results in robust anti-tumor activity. Preclinical data presented at the 2017 Keystone Tumor Metabolism meeting demonstrated that small molecule inhibitors of MAT2A are efficacious in *MTAP*-deleted tumor xenograft models.

About Agios Pharmaceuticals, Inc.
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.

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 the clinical development of its drug development programs, including AG-270; the potential benefits of Agios' product candidates, including AG-270; and the potential benefit of its strategic plans and focus. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “would,” “could,” “potential,” “possible,” “hope,” “strategy,” “milestone,” “will,” 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’ 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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