



## **AgiOS Announces MTAP Pathway Research Program as Development Program and Development Candidate Under Master Research and Collaboration Agreement with Celgene**

March 13, 2017

### **Investigational New Drug (IND) Submission Expected by Year End 2017**

CAMBRIDGE, Mass., March 13, 2017 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO) today announced that Celgene Corporation has designated the development candidate focused on MTAP (methylthioadenosine phosphorylase) deleted cancers as a development candidate under the master research and collaboration agreement (the "Agreement") dated May 17, 2016.

Under the terms of the Agreement, Celgene will pay Agios an \$8 million designation fee for the MTAP pathway program. Exploratory research, drug discovery and early development on the MTAP pathway program is led by Agios, and Celgene will have an opt-in right on the program up through Phase 1 dose escalation for at least a \$30 million fee. Upon opt-in, Celgene and Agios will have global co-development and co-commercialization rights with a worldwide 50/50 cost and profit share on the MTAP pathway program, and Agios will be eligible for up to \$169 million in clinical and regulatory milestone payments.

"We are pleased that Celgene has designated this fourth development candidate discovered and developed at Agios since the beginning of our research collaboration with them in 2010," said Scott Biller, Ph.D., chief scientific officer at Agios. "We have clearly demonstrated our ability to translate novel Agios discoveries into important precision medicines in areas of high unmet need with our IDH portfolio. We look forward to exploring the potential of our MTAP program in patients following our expected IND submission by the end of this year."

MTAP-deletions are present in approximately 15 percent of all cancers. 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 in Whistler, British Columbia on March 9, 2016. The presentation can be found under Publications in the Research section of the Agios website ([www.agios.com](http://www.agios.com)).

The \$8 million designation fee is expected to be received in the second quarter of 2017.

#### **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 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 [www.agios.com](http://www.agios.com).

#### **About Agios/Celgene Collaboration**

Enasidenib and AG-881 are part of Agios' global strategic collaboration with Celgene Corporation focused on cancer metabolism. Under the terms of the 2010 collaboration agreement, Celgene has worldwide development and commercialization rights for enasidenib. Agios continues to conduct clinical development activities within the enasidenib development program and is eligible to receive reimbursement for those development activities and up to \$95 million in remaining payments assuming achievement of certain milestones and royalties on net sales. Celgene and Agios intend to co-commercialize enasidenib in the U.S. Celgene will reimburse Agios for costs incurred for its co-commercialization efforts. 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. 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.

#### **Agios 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 Agios' plans, strategies and expectations for its and its collaborator's preclinical, clinical and commercial advancement of its drug development programs; the potential benefits of Agios' product candidates; 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 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' Annual Report on Form 10-K for the year ended December 31, 2016, 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, except as required by law.

Contacts

Investors:

Kendra Adams, 617-844-6407

Senior Director, Investor & Public Relations

[Kendra.Adams@agios.com](mailto:Kendra.Adams@agios.com)

Renee Leck, 617-649-8299

Senior Manager, Investor & Public Relations

[Renee.Leck@agios.com](mailto:Renee.Leck@agios.com)

Media:

Holly Manning, 617-844-6630

Associate Director, Corporate Communications

[Holly.Manning@agios.com](mailto:Holly.Manning@agios.com)



Agios Pharmaceuticals