# Agios Outlines Key 2018 Priorities Expanding Clinical and Research Programs to Drive Long Term Value

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*- Ivosidenib NDA for IDH1m R/R AML Submitted in December for Priority Review; Multiple Pivotal Trials Ongoing or Planned for Ivosidenib in Frontline AML and Solid Tumors Including 4Q 2018 Initiation of Phase 3 Trial with Intensive Chemotherapy -*

*- Two Pivotal Trials in Pyruvate Kinase Deficiency with AG-348, ACTIVATE-T to Initiate in 1Q 2018 and ACTIVATE in 2Q 2018; Phase 2 Proof of Concept Trial of AG-348 in Thalassemia Planned for 4Q 2018 -*

*- MAT2A Inhibitor AG-270 Expected to Enter Phase 1 Dose-Escalation Trial in MTAP-Deleted Tumors in 1Q 2018 -*

*- Seventh IND Submission for a DHODH Inhibitor for the Treatment of Hematologic Malignancies Expected in 4Q 2018 -*

*- 2017 Year End Cash, Cash Equivalents and Marketable Securities of $568M Funds Expanded Clinical and Research Programs Through the End of 3Q 2019 -*

SAN FRANCISCO, Jan. 08, 2018 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, today summarized key 2018 priorities in conjunction with its presentation at the 36th Annual J.P. Morgan Healthcare Conference in San Francisco. The presentation will outline how Agios' clinical and research programs have the potential to provide meaningful benefit to a large number of patients. The company will webcast its presentation today at 9:30 a.m. PT (12:30 p.m. ET) at investor.agios.com.

"We presented a significant amount of clinical data across our portfolio in 2017, including data supporting the approval of our first internally discovered medicine, which proves that our research and development engine has the ability to deliver important precision medicines from a discovery in the lab to patients as an approved drug," said David Schenkein, M.D., chief executive officer at Agios. "We are very pleased with the early launch performance of IDHIFA® and expect to repeat this success with ivosidenib upon FDA approval in 2018, while continuing our label expansion opportunities for frontline AML and solid tumors and bringing our next drug candidate targeting genetically defined cancers, AG-270, into the clinic. In rare genetic diseases, AG-348 has demonstrated proof of concept in pyruvate kinase deficiency and in addition to beginning pivotal trials in this disease, we are exploring the utility of PK activation in other anemias, starting with a Phase 2 study in thalassemia."

The company plans to achieve the following key milestones in 2018:

*Cancer:*

- Potential FDA approval and commercialization of ivosidenib for relapsed/refractory (R/R) acute myeloid leukemia (AML) with an isocitrate dehydrogenase-1 (IDH1) mutation in the United States in the second half of 2018.
- Plan to submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for ivosidenib for IDH1m R/R AML in the fourth quarter of 2018.
- Support with Celgene an intergroup sponsored, global, registration-enabling Phase 3 trial combining ivosidenib or enasidenib and standard induction (7+3) and consolidation chemotherapy with a primary endpoint of event free survival (EFS) in frontline AML patients with an IDH1 or IDH2 mutation in the fourth quarter of 2018.
- Initiate a perioperative 'window' trial with ivosidenib and AG-881 in low-grade glioma to further investigate their effects on brain tumor tissue in the first half of 2018.
- Initiate a Phase 1 dose-escalation trial for AG-270, a first-in-class methionine adenosyltransferase 2a (MAT2A) inhibitor, in methylthioadenosine phosphorylase (MTAP)-deleted tumors in the first quarter of 2018.

*Rare Genetic Diseases:*

- Initiate two global pivotal trials for AG-348 in PK deficiency:
  - ACTIVATE-T: A single arm trial of approximately 20 regularly transfused patients is expected to initiate in the first quarter of 2018.
  - ACTIVATE: A 1:1 randomized, placebo-controlled trial of 80 patients who do not receive regular transfusions is expected to initiate in the second quarter of 2018.
- Initiate a global registry for adult and pediatric patients with PK deficiency (PEAK) in the first quarter of 2018 to increase understanding of the long-term disease burden of this chronic anemia.
- Initiate a Phase 2 proof of concept trial of AG-348 in thalassemia in the fourth quarter of 2018.

*Research:*

- Submit an investigational new drug (IND) application for our latest development candidate, an inhibitor of the metabolic enzyme dihydroorotate dehydrogenase (DHODH) for the treatment of hematologic malignancies in the fourth quarter of

2018.

The company plans to host an analyst day in the first half of 2018 to review Agios' commercial readiness and broad clinical development programs and highlight the depth of the research portfolio across oncology, rare genetic disease and metabolic immuno-oncology.

The company also highlighted key 2017 achievements:

- Collaborated with Celgene to achieve the U.S. Food and Drug Administration (FDA) full approval and subsequent launch of IDHIFA® (enasidenib) for the treatment of adult patients with R/R AML with an isocitrate dehydrogenase-2 (IDH2) mutation as detected by an FDA approved diagnostic test.
- Submitted a new drug application (NDA) to the FDA for ivosidenib for the treatment of patients with R/R AML with an IDH1 mutation.
- Initiated a global, registration-enabling Phase 3 study (AGILE) combining ivosidenib and VIDAZA® in newly diagnosed AML patients with an IDH1 mutation ineligible for intensive chemotherapy.
- Finalized two global, pivotal trial designs evaluating AG-348 in adults with pyruvate kinase (PK) deficiency.
- Achieved FDA clearance of an IND application for AG-270, a MAT2A inhibitor, targeting MTAP-deleted tumors.

**2017 Year-End Cash and Updated Guidance**

Agios ended 2017 with approximately $568 million of cash, cash equivalents and marketable securities. Based on its expanded clinical and research programs announced today, the company now expects that its existing cash, cash equivalents and marketable securities as of December 31, 2017, together with anticipated interest income, anticipated expense reimbursements, and royalty payments under our collaboration agreements, but excluding any additional program-specific milestone payments, will enable the company to fund its anticipated operating expenses and capital expenditure requirements through the end of third quarter of 2019.

**Presentation at 36th Annual J.P. Morgan Healthcare Conference**

Agios will webcast its corporate presentation from the 36th Annual J.P. Morgan Healthcare Conference in San Francisco on Monday, January 8, 2018 at 9:30 a.m. PT (12:30 p.m. ET). A live webcast of the presentation can be accessed under "Events & Presentations" in the Investors section of the company's website at agios.com. A replay of the webcast will be archived on the Agios website for at least two weeks following the presentation.

**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 Agios/Celgene Collaboration**
IDHIFA® (enasidenib), AG-881 and AG-270 are part of Agios' global strategic collaboration with Celgene Corporation. Under the terms of the 2010 collaboration agreement, Celgene has worldwide development and commercialization rights for IDHIFA® (enasidenib). Agios continues to conduct certain clinical development activities within the IDHIFA® (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 any net sales. Celgene and Agios are currently co-commercializing IDHIFA® (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. AG-270 is part of a 2016 global research collaboration agreement with Celgene. Celgene has the option to participate in a worldwide cost and profit share with Agios, under which 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.

**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 IDHIFA®, ivosidenib, AG-881, AG-348 and AG-270; the potential benefits of Agios' product candidates; its key milestones for 2018; its estimates regarding its balance of cash, cash equivalents and marketable securities for the year ended December 31, 2017; its financial guidance regarding the period in which it will have capital available to fund its operations; 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' 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.

**Contacts**

Investors:
Renee Leck, 617-649-8299
Senior Manager, Investor & Public Relations
Renee.Leck@agios.com

Media:
Holly Manning, 617-844-6630
Associate Director, Corporate Communications
Holly.Manning@agios.com


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