



AgiOS to Present Updated Clinical Data from PKR Activator AG-348 at EHA

May 18, 2017

Data from IDHIFA® (Enasidenib) Phase 1 Trial in IDH2m R/R AML to be Presented

CAMBRIDGE, Mass., May 18, 2017 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the fields of cancer metabolism and rare genetic diseases, today announced that updated clinical data from the fully enrolled, ongoing Phase 2 study (DRIVE PK) of AG-348 in adults with pyruvate kinase (PK) deficiency, a rare, potentially debilitating, congenital anemia, will be presented at the 22nd Congress of the European Hematology Association (EHA) taking place June 22-25, 2017 in Madrid, Spain. AG-348 is a wholly owned, novel, first-in-class, oral activator of both wild-type (normal) and mutated pyruvate kinase-R (PKR) enzymes.

The accepted abstracts are listed below and available online on the EHA conference website: http://learningcenter.ehaweb.org/eha/#?listing=3*browseby=2*sortby=1*media=3*ce_id=1181*label=15531

Oral presentation by Agios:

Title: Effects of AG-348, a pyruvate kinase activator, in patients with Pyruvate Kinase Deficiency: updated results from the DRIVE-PK study

Date & Time: Saturday, June 24, 2017 from 11:30-11:45 a.m. CET

Session Title: Sickle cell disease, enzymes

Abstract Code: S451

Location: Room N109

Presenter: Rachael Grace, M.D., Dana-Farber Boston Children's Cancer and Blood Disorder Center

Updated data from the DRIVE PK study will be presented at the time of the meeting.

Poster presentation by Agios collaborator:

Title: Ex-vivo treatment of red blood cells from 15 Pyruvate Kinase (PK)-deficient patients with AG-348, an allosteric activator of PK-R, increases enzymatic activity, protein stability and ATP levels

Date & Time: Saturday, June 24, 2017 from 5:30-7:00 p.m. CET

Session Title: Enzymes and sickle cell disease

Abstract Code: P614

Location: Poster area (Hall 7)

Author: Richard van Wijk, Ph.D., University Medical Center Utrecht

Encore presentations by Agios and Celgene:

Title: Enasidenib in mutant-IDH2 relapsed or refractory acute myeloid leukemia (R/R AML): Results of a phase 1 dose-escalation and expansion study

Date & Time: Saturday, June 24, 2017 from 4:00-4:15 p.m. CET

Oral Abstract Session: Targeted treatment of AML

Abstract Code: S471

Location: Hall D

Presenter: Eytan Stein, M.D., Memorial Sloan-Kettering Cancer Center and Weill Cornell Medical College

Title: Differentiation syndrome associated with enasidenib, a selective inhibitor of mutant isocitrate dehydrogenase 2 (mIDH2)

Poster Session Date & Time: Friday, June 23, 2017 from 5:15-6:45 p.m. CET

Session Title: Acute myeloid leukemia - Clinical 3

Abstract Code: P215

Location: Poster area (Hall 7)

Author: Amir Tahmasb Fathi, M.D., Massachusetts General Hospital and Harvard Medical School

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.

About Agios/Celgene Collaboration

IDHIFA® (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 IDHIFA® (enasidenib). Agios continues to conduct 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 net sales. Celgene and Agios intend to co-commercialize IDHIFA® (enasidenib) in the U.S. Celgene will reimburse Agios for costs incurred for its co-commercialization efforts.

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 collaborators' preclinical, clinical and commercial advancement of its drug development programs including AG-348 and IDHIFA® (enasidenib); the potential benefits of Agios' product candidates; its

plans regarding future data presentations; and the potential benefit of its strategic plans and focus. The words "intend," "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.

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Agios Pharmaceuticals