

Agios to Present Updated Data from Clinical Trials of Mitapivat in Pyruvate Kinase Deficiency and Thalassemia in Oral Presentations at the European Hematology Association Annual Congress

May 12, 2021

- Agios to Host Investor Webcast on June 11 at 7:30 a.m. ET -

CAMBRIDGE, Mass., May 12, 2021 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the field of cellular metabolism to treat genetically defined diseases, today announced that mitapivat clinical data will be presented at the European Hematology Association (EHA) Annual Congress being held virtually June 9-17, 2021.

The accepted abstracts are listed below and are available online on the EHA meeting library website. All presentations can be accessed on demand by registered meeting attendees on the EHA Virtual Congress platform as of Friday, June 11 at 9:00 a.m. CEST / 3:00 a.m. ET.

Oral Presentations:

Title: ACTIVATE: A Phase 3, randomized, multicenter, double-blind, placebo-controlled study of mitapivat in adults with pyruvate kinase deficiency who are not regularly transfused

Live Q&A Session Date and Time: Tuesday, June 15, 2021 at 7:45 p.m. CEST / 1:45 p.m. ET

Oral Abstract Session: Changing the scene in congenital anemias

Abstract: S270

Presenter: Hanny Al-Samkari, M.D., Division of Hematology, Massachusetts General Hospital, Harvard Medical School, Boston, MA, United States

Title: ACTIVATE-T: A Phase 3, open-label, multicenter study of mitapivat in adults with pyruvate kinase deficiency who are regularly transfused

Live Q&A Session Date and Time: Tuesday, June 15, 2021 at 7:45 p.m. CEST / 1:45 p.m. ET

Oral Abstract Session: Changing the scene on congenital anemias

Abstract: S271

Presenter: Andreas Glenthøj, M.D., Department of Hematology, Rigshospitalet Copenhagen, Denmark

Title: Results from a Phase 2, open-label, multicenter study of the oral pyruvate kinase activator mitapivat in adults with non-transfusion dependent

alpha- or beta-thalassemia

Live Q&A Session Date and Time: Tuesday, June 15, 2021 at 8:45 p.m. CEST / 2:45 p.m. ET

Oral Abstract Session: Changing the scene on thalassemias

Abstract: S267

Presenter: Kevin H. M. Kuo, M.D., Division of Hematology, University of Toronto, Toronto, Canada

Poster Presentations:

Title: Bone mineral density is stable in adults with pyruvate kinase deficiency receiving long-term treatment with mitapivat

Poster Session: Enzymopathies, membranopathies and other anemias

Abstract: EP696

Lead Author: Hanny Al-Samkari, M.D., Division of Hematology, Massachusetts General Hospital, Harvard Medical School, Boston, MA, United States

Title: Early-onset osteopenia and osteoporosis in patients with pyruvate kinase deficiency (Encore)

Poster Session: Enzymopathies, membranopathies and other anemias

Abstract: EP692

Lead Author: Hanny Al-Samkari, M.D., Division of Hematology, Massachusetts General Hospital, Harvard Medical School, Boston, MA, United States

Title: Baseline characteristics by age of a global cohort of patients diagnosed with pyruvate kinase deficiency – a descriptive analysis from the Peak

Registry

Poster Session: Enzymopathies, membranopathies and other anemias

Abstract: EP691

Lead Author: Paola Bianchi, BSc, Ph.D., UOC Ematologia, Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, Milan, Italy

Title: Mortality among veterans with a diagnosis of pyruvate kinase deficiency: A real world study using U.S. veterans health administration data

(Encore)

Poster Session: Enzymopathies, membranopathies and other anemias

Abstract: EP710

Lead Author: Erin Zagadailov, PharmD, MS, Agios Pharmaceuticals, Cambridge, MA, United States

Title: The lifetime economic burden of pyruvate kinase deficiency in the United States

Poster Session: Quality of life, palliative care, ethics and health economics

Abstract: EP1194

Lead Author: Wayne Su, MSc, Xcenda, Carrollton, TX, United States

Title: Global thalassemia epidemiology: A systemic literature review

Poster Session: Thalassemias

Abstract: EP1318

Lead Author: Christina X. Chamberlain, Ph.D., Agios Pharmaceuticals, Cambridge, MA, United States

Publication Only:

Title: ENERGIZE and ENERGIZE-T: Two Phase 3, randomized, double-blind, placebo-controlled studies of mitapivat in adults with non-transfusion dependent or transfusion-dependent alpha- or beta-thalassemia

Poster Session: Thalassemias

Abstract: PB1805

Lead Author: Kevin H. M. Kuo, M.D., Division of Hematology, University of Toronto, Toronto, Canada

Conference Call Information

Agios will host a virtual investor event on June 11, 2021 at 7:30 a.m. ET to review the mitapivat clinical data. The event will be webcast live and can be accessed under "Events & Presentations" in the Investors and Media section of the company's website at www.agios.com. The archived webcast will be available on the company's website beginning approximately two hours after the event.

About Agios

Agios is focused on discovering and developing novel investigational medicines to treat genetically defined diseases through scientific leadership in the field of cellular metabolism. The company's most advanced drug candidate is a first-in-class pyruvate kinase R (PKR) activator, mitapivat, that is currently being evaluated for the treatment of three distinct hemolytic anemias. In addition to its active late-stage clinical pipeline, Agios has multiple novel, investigational therapies in clinical and/or preclinical development. 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 forwardlooking statements include those regarding the potential benefits of mitapivat; Agios' plans regarding future data presentations; 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 collaborators 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. Moreover, there can be no guarantee that any medicines ultimately commercialized by Agios will receive commercial acceptance. 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, without limitation: risks and uncertainties related to the impact of the COVID-19 pandemic to Agios' business, operations, strategy, goals and anticipated milestones, including its ongoing and planned research activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved products, and launching, marketing and selling current or future approved products; 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, the EMA or 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; 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.

Contact:

Holly Manning, 617-844-6630 Senior Director, Investor Relations Holly.Manning@agios.com



Source: Agios Pharmaceuticals, Inc.