



AgiOS Pharmaceuticals to Present Clinical and Translational Data in Rare Blood Disorders at European Hematology Association 2024 Hybrid Congress

May 14, 2024

– Data from Phase 3 ENERGIZE Study of Mitapivat in Non-Transfusion-Dependent Thalassemia to be Presented in Plenary Session –

– Additional Presentations to Highlight Quality of Life Data from ENERGIZE and Design of the Phase 3 RISE UP Study of Mitapivat in Sickle Cell Disease –

– Agios to Webcast Virtual Investor Event on June 16, 2024, at 10 a.m. Eastern Time or 4 p.m. Central European Summer Time –

CAMBRIDGE, Mass., May 14, 2024 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in cellular metabolism and PK activation pioneering therapies for rare diseases, today announced that data from its programs will be presented at the European Hematology Association 2024 (EHA2024) Hybrid Congress, to be held June 13-16, 2024, in Madrid, Spain.

Data from the Phase 3 ENERGIZE study of mitapivat in non-transfusion-dependent thalassemia will be presented in a plenary session on June 15, 14:45-15:15 CEST (Abstract #S104) and in a poster session on June 14, 18-19:00 CEST (Abstract #P1529). Additional presentations will highlight data across Agios' pipeline in rare blood disorders, including sickle cell disease and pyruvate kinase deficiency.

In total, seven abstracts led by Agios and external collaborators will be presented or published. The accepted abstracts are listed below and are available online on the EHA congress website at www.ehaweb.org.

Thalassemia

Data from the global Phase 3 ENERGIZE study of mitapivat in alpha- or beta- non-transfusion-dependent thalassemia.

Plenary Abstracts Session:

Title: ENERGIZE: A Global Phase 3 Study of Mitapivat Demonstrating Efficacy and Safety in Adults with Alpha- or Beta- Non-Transfusion-Dependent Thalassemia

Abstract: S104

Session Date and Time: Saturday, June 15, 14:45-15:15 CEST

Presenter: Ali T. Taher, M.D., Ph.D.; Naef K. Basile Cancer Institute, American University of Beirut Medical Center in Beirut, Lebanon

Poster Presentation:

Title: Improvements in Fatigue and 6-minute Walk Test in Adults with Alpha- and Beta-Non-Transfusion-Dependent Thalassemia: The Phase 3 ENERGIZE Trial of Mitapivat

Abstract: P1529

Session Date and Time: Friday, June 14, 18-19:00 CEST

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

Sickle Cell Disease

A look at the design of the Phase 3 portion of the RISE UP study, as well as information about Phase 2 open-label renal study.

e-Poster Presentations:

Title: Study Design of the Phase 3 Portion of RISE UP: A Phase 2/3, Randomized, Double-blind, Placebo-controlled Study of Mitapivat in Patients with Sickle Cell Disease

Abstract: P2193

Time: Friday, June 14, 9:00 CEST

Lead Author: Biree Andemariam, M.D.; New England Sickle Cell Institute, University of Connecticut Health, Farmington, CT

Title: The Launch of a Global, Phase 2, Open-label, Multicenter, Single-arm Study of Mitapivat in Patients with Sickle Cell Disease and Nephropathy

Abstract: P2194

Time: Friday, June 14, 9:00 CEST

Lead Author: Fuad El Rassi, M.D.; Department of Hematology and Medical Oncology, Winship Cancer Institute, Emory University School of Medicine, Atlanta, GA, USA; Georgia Comprehensive Sickle Cell Center at Grady Health System, Grady Memorial Hospital, Atlanta, GA, USA

Title: Cost Analysis of Care Expenditures of Patients with Sickle Cell Disease of Hydroxyurea Therapy in a Specialized Blood Center in Rio de Janeiro, Brazil

Abstract: P2306

Time: Friday, June 14, 9:00 CEST

Lead Author: La'Ron Browne, M.D.; Pediatric Hematology Oncology Clinical Fellow, St. Jude Children's Research Hospital, Memphis, TN

Pyruvate Kinase Deficiency

Real-world data describing the characteristics of pediatric patients facing iron overload as part of their disease.

Poster Presentation:

Title: The Characteristics of Pediatric Patients with Pyruvate Kinase Deficiency and Iron Overload

Session Date and Time: Friday, June 14, 18-19:00 CEST

Abstract: P1564

Lead Author: Rachael F. Grace, M.D., MMSc; Dana-Farber/Boston Children's Cancer and Blood Disorder Center, Harvard Medical School, Boston, MA

Other

Agios' collaborators present new data evaluating mitapivat as a potential treatment for other rare hemolytic anemias.

Oral Presentation:

Title: Ex Vivo Pyruvate Kinase Activation in Hereditary Spherocytosis and Xerocytosis: Improved Enzyme Function and Red Cell Properties

Presentation Time: Sunday, June 16, 11:30-12:45 CEST

Abstract: S299

Presenter: Jonathan R.A. de Wilde, Ph.D. Candidate; Red Blood Cell Research Group, Central Diagnostic Laboratory-Research, University Medical Center Utrecht, Utrecht University, Utrecht, the Netherlands

Conference Call Information

Agios will host a virtual investor breakout session on June 16, 2024, at 10:00 a.m. ET (4 p.m. CEST) to review the key clinical oral and poster presentations from this year's EHA meeting. 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 the pioneering leader in PK activation and is dedicated to developing and delivering transformative therapies for patients living with rare diseases. In the U.S., Agios markets a first-in-class pyruvate kinase (PK) activator for adults with PK deficiency, the first disease-modifying therapy for this rare, lifelong, debilitating hemolytic anemia. Building on the company's deep scientific expertise in classical hematology and leadership in the field of cellular metabolism and rare hematologic diseases, Agios is advancing a robust clinical pipeline of investigational medicines with programs in alpha- and beta-thalassemia, sickle cell disease, pediatric PK deficiency, MDS-associated anemia and phenylketonuria (PKU). In addition to its clinical pipeline, Agios is advancing a preclinical TMPRSS6 siRNA as a potential treatment for polycythemia vera. 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 the potential benefits of PYRUKYND® (mitapivat); plans regarding future data presentations; and the potential benefits of Agios' strategic plans and focus. The words "anticipate," "expect," "goal," "hope," "milestone," "plan," "potential," "possible," "strategy," "will," "vision," 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, without limitation: risks and uncertainties related to the impact of pandemics or other public health emergencies 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 establish and maintain key collaborations; uncertainty regarding any milestone or royalty payments related to the sale of its oncology business or its in-licensing of TMPRSS6 siRNA, and the uncertainty of the timing of any such payments; uncertainty of the results and effectiveness of the use of Agios' cash and cash equivalents; 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:

Investor Contact

Chris Taylor, VP, Investor Relations and Corporate Communications

Agios Pharmaceuticals

IR@agios.com

Media Contact

Dan Budwick

1AB Media

dan@1abmedia.com



Source: Agios Pharmaceuticals, Inc.