Agios to Present Clinical and Translational Data at the European Hematology Association Annual Congress

May 12, 2022

– Agios to Present New Patient-Reported Outcomes (PRO) Data from ACTIVATE Phase 3 Study of PYRUKYND® (mitapivat) in Adults with PK Deficiency Who Do Not Receive Regular Transfusions –

– Agios to Present New Clinical Data Demonstrating the Normalization of Hemoglobin Levels with Long-term Treatment of PYRUKYND® in Adults with PK Deficiency –

– New Clinical Data from Investigator-Sponsored ESTIMATE Phase 2 Study of PYRUKYND® in Sickle Cell Disease to Be Presented –

– Agios to Host Investor Webcast on June 13 at 8 a.m. ET –

CAMBRIDGE, Mass., May 12, 2022 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism pioneering therapies for genetically defined diseases, today announced that clinical and translational data will be presented at the European Hematology Association (EHA) Annual Congress, hosted virtually and in person in Vienna on June 9-12, 2022.

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 on Friday, June 20.

Agios-led Abstracts:

Poster Presentations

Title: Long-term Efficacy and Safety of the Oral Pyruvate Kinase Activator Mitapivat in Adults With Non–transfusion-dependent Alpha- or Beta-thalassemia
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1522
Presenting Author: Kevin H. M. Kuo, M.D., Division of Hematology, University of Toronto, Toronto, Canada

Title: Comorbidities and Complications Across Genotypes in Adult Patients With Pyruvate Kinase Deficiency: Analysis From the Peak Registry
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1542
Presenting Author: Andreas Glenthøj, M.D., Department of Hematology, Rigshospitalet Copenhagen, Denmark

Title: Mitapivat Decreases the Need for Transfusions Secondary to Poorly Tolerated Anemia and Acute Events Compared to Placebo in Patients With Pyruvate Kinase Deficiency Who Are Not Regularly Transfused
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1543
Presenting Author: Hanny Al-Samkari, M.D., Division of Hematology, Massachusetts General Hospital, Harvard Medical School, Boston, MA, United States

Title: Bone Mineral Density Remains Stable in Pyruvate Kinase Deficiency Patients Receiving Long-term Treatment With Mitapivat
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1544
Presenting Author: Hanny Al-Samkari, M.D., Division of Hematology, Massachusetts General Hospital, Harvard Medical School, Boston, MA, United States

Title: Durability of Hemoglobin Response and Reduction in Transfusion Burden Is Maintained Over Time in Patients With Pyruvate Kinase Deficiency Treated With Mitapivat in a Long-term Extension Study
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1545
Presenting Author: Rachael F. Grace, M.D., Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Boston, MA, United States

Title: ACTIVATE-kidsT: Mitapivat in Children With Pyruvate Kinase Deficiency Who Are Regularly Transfused
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1546
Presenting Author: Rachael F. Grace, M.D., Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Boston, MA, United States

Title: ACTIVATE-kids: Mitapivat in Children With Pyruvate Kinase Deficiency Who Are Not Regularly Transfused
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1547
Presenting Author: Rachael F. Grace, M.D., Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Boston, MA, United States

Title: Long-term Treatment With Oral Mitapivat Is Associated With Normalization of Hemoglobin Levels in Patients With Pyruvate Kinase Deficiency
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1548

Presenting Author: Wilma Barcellini, M.D., Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, Milan, Italy

Title: Characterizing Iron Overload by Age in Patients Diagnosed with Pyruvate Kinase Deficiency – a Descriptive Analysis from the Peak Registry
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1562
Presenting Author: Paola Bianchi, BSc, Ph.D., Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico di Milano, Milan, Italy

Title: Mitapivat Improves Ineffective Erythropoiesis and Reduces Iron Overload in Patients With Pyruvate Kinase Deficiency
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1565
Presenting Author: Eduard J. van Beers, M.D., Van Creveldkliniek, Department of Internal Medicine, University Medical Center Utrecht, Utrecht, Netherlands

Title: Improvements in Patient-Reported Outcomes in Mitapivat-treated Patients With Pyruvate Kinase Deficiency: A Descriptive Analysis From the Phase 3 ACTIVATE Trial
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1735
Presenting Author: Kevin H. M. Kuo, M.D., Division of Hematology, University of Toronto, Toronto, Canada

Publication Only

Title: Systematic Literature Review of Health-related Quality of Life Burden in Patients Across the Spectrum of Thalassemia
Abstract: PB2233
Lead Author: Khaled M. Musallam, M.D., Ph.D., Thalassemia Center, Burjeel Medical City, Abu Dhabi, United Arab Emirates

Title: Pharmacokinetic Modeling and Simulation to Support Mitapivat Dose Selection Used in Pediatric Phase III Studies
Abstract: PB2247
Lead Author: Ophelia Yin, Ph.D., Agios Pharmaceuticals, Cambridge, MA, United States

Title: Healthcare Resource Utilization of Adult Patients With Pyruvate Kinase Deficiency: A Real World Study Using US Veterans Health Administration Data
Abstract: PB2359
Lead Author: Erin Zagadailov, Pharm.D., Agios Pharmaceuticals, Cambridge, MA, United States

Collaborator-led Abstracts:

Oral Presentations

Title: Evidence of Noninferiority of Mitapivat Versus Splenectomy in Murine Hereditary Spherocytosis
Session Date and Time: Saturday, June 11 at 16:30 – 17:45 CEST (Hall A2-A3)
Abstract: S273
Presenter: Alessandro Mattè, Ph.D., University of Verona and AOUI Verona, Verona, Italy

Poster Presentations

Title: Pharmacodynamic Effects of AG-946, a Highly Potent Novel Activator of Pyruvate Kinase, In *Ex Vivo* Treatment of Red Blood Cells From Sickle Cell Disease Patients
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1492
Lead Author: Minke A.E. Rab, M.D., Ph.D., Central Diagnostic Laboratory – Research, University Medical Center Utrecht, Utrecht University, Utrecht, The Netherlands

Title: Follow-up Results of a Phase 2 Study Assessing the Safety and Efficacy of Mitapivat Treatment, an Oral Pyruvate Kinase Activator, for up to 60 Weeks in Subjects With Sickle Cell Disease
Session date and time: Friday, June 10 at 16:30 - 17:45 CEST
Abstract: P1501
Presenting Author: Myrthe J. van Dijk, Ph.D., Department of Central Diagnostic Laboratory - Research, University Medical Center Utrecht, Utrecht University, Utrecht, Netherlands

Publication Only

Title: Adapting Patient Educational Approaches in Pyruvate Kinase Deficiency: Europe and North America Findings From the AAC Patient and Caregiver Survey
Abstract: PB2345
Presenting Author: Wilma Barcellini, M.D., Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, Milan, Italy

Conference Call Information
Agios will host a virtual investor event on June 13, 2022, at 8 a.m. ET to review select data from the EHA presentations. 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 a biopharmaceutical company that is fueled by connections. The Agios team cultivates strong bonds with patient communities, healthcare professionals, partners and colleagues to discover, develop and deliver therapies for genetically defined 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 leadership in the field of cellular metabolism, Agios is advancing a robust clinical pipeline of investigational

medicines with active and planned programs in alpha- and beta-thalassemia, sickle cell disease, pediatric PK deficiency and MDS-associated anemia. In addition to its clinical pipeline, Agios has multiple investigational therapies in preclinical development and an industry-leading research team with unmatched expertise in cellular metabolism and genetics. 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 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.

Contacts

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

Media:
Jessica Rennekamp, 857-209-3286
Director, Corporate Communications
Jessica.Rennekamp@agios.com

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