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

May 11, 2023

– Agios to Present New Analyses from ACTIVATE, ACTIVATE-T and Long-Term Extension Studies in Adults with PK Deficiency Reinforcing Clinical Benefit of PYRUKYND® (mitapivat) on Hemoglobin, Hemolysis, Iron Overload and Patient-Reported Outcomes –

– Agios to Present Updated Real-World Data on the Burden of Disease in Pyruvate Kinase (PK) Deficiency and Alpha- and Beta-Thalassemia –

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

CAMBRIDGE, Mass., May 11, 2023 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a leader in the field of cellular metabolism pioneering therapies for rare diseases, today announced that the company and its collaborators will present a broad range of clinical and translational data at the European Hematology Association (EHA) Hybrid Congress, hosted virtually and in person in Frankfurt, Germany, June 8-11, 2023, and continuing virtually on the Congress platform June 14-15, 2023.

The accepted abstracts are listed below and are available online on the EHA meeting library website. All posters will be presented during the poster session on Friday, June 9 at 16:30-17:45 CEST. All presentations can be accessed on demand by registered meeting attendees on the EHA Virtual Congress platform until Aug. 15, 2023.

Agios-led Abstracts:

Poster Presentations

Title: Healthcare Resource Use, Economic Burden and In-Patient Mortality in Patients with Alpha- and Beta-Thalassemia Compared to Matched Controls in the Real-World Setting
Abstract: P1463
Presenting Author: Louise Lombard, M Nutr, Agios Pharmaceuticals, Cambridge, MA, United States

Title: Clinically Relevant Hemoglobin Response in Adults with Pyruvate Kinase Deficiency Treated with Mitapivat – A Sub-Analysis of the ACTIVATE Trial
Abstract: P1473
Presenting Author: Hanny Al-Samkari, M.D., Division of Hematology, Massachusetts General Hospital, Harvard Medical School, Boston, MA, United States

Title: The Clinical Characteristics and Overall Survival of Pyruvate Kinase Deficiency Patients in the UK: A Real-World Study
Abstract: P1476
Presenting Author: Sarah Higa, PharmD, M.S., Agios Pharmaceuticals, Cambridge, MA, United States

Title: Mitapivat Efficacy in Adults with Pyruvate Kinase Deficiency and Baseline Hemoglobin Levels >10 g/dL
Abstract: P1477
Presenting Author: Rachael F. Grace, M.D., Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Harvard Medical School, Boston, MA, United States

Title: Comorbidities and Complications in Adults with Pyruvate Kinase Deficiency According to Hemoglobin Strata – A Descriptive Analysis from the Peak Registry
Abstract: P1479
Presenting Author: Dagmar Pospíšilová, M.D., Ph.D., Department of Pediatrics, Palacky University and University Hospital, Olomouc, Czech Republic

Title: *PKM* And *PKR* Expression During Hematopoiesis and Erythropoiesis
Abstract: P1485
Presenting Author: Erin Tsai, M.S., Agios Pharmaceuticals, Cambridge, MA, United States

Title: Mitapivat Improves Iron Overload in Patients with Pyruvate Kinase Deficiency Who Are Regularly Transfused
Abstract: P1497
Presenting Author: Eduard J. van Beers, M.D., Center for Benign Haematology, Thrombosis and Haemostasis, Van Creveldkliniek, University Medical Center Utrecht, Utrecht University, Utrecht, The Netherlands

Publication Only

Title: Estimating Utility Values for Health States in Pyruvate Kinase Deficiency
Abstract: PB2547
Lead Author: Sarah Higa, PharmD, M.S., Agios Pharmaceuticals, Cambridge, MA, United States

Collaborator-led Abstracts:

Poster Presentations

Title: AG946, A Pyruvate Kinase (PK) Activator Improves PK Properties and Red Blood Cell (RBC) Metabolism Upon Ex Vivo Treatment of RBCs from Patients with Myelodysplastic Syndromes
Abstract: P717
Presenting Author: Jonathan de Wilde, MSc, University Medical Center Utrecht, Utrecht University, Utrecht, Netherlands

Title: One-Year Follow-Up of a Phase 2 Study of Mitapivat, an Oral Pyruvate Kinase Activator, for the Treatment of Sickle Cell Disease
Abstract: P1424
Presenting Author: Myrthe J. van Dijk, Ph.D., Division Laboratories, Pharmacy and Biomedical Genetics, CDL en Van Creveldkliniek , University Medical Center Utrecht, Utrecht University, Utrecht, Netherlands

Title: Rare Anaemia Disorders European Epidemiological Platform (RADeep): Distribution of Patients Affected by RADs in Europe
Abstract: P1427
Presenting Author: Maria Del Mar Mañú Pereira, Ph.D., Vall d'Hebron University Hospital, Group of Translational Research in Cancer and Blood disorders in Children, Barcelona, Spain

Title: Mitapivat Ameliorates In Vitro Human β Thalassemic Erythroid Maturation Index and Modulates the Expression of Peroxiredoxin-2
Abstract: P1458
Presenting Author: Lucia De Franceschi, M.D., Department of Medicine, University of Verona, and Azienda Ospedaliera Universitaria Verona, Policlinico GB Rossi, Verona, Italy

Publication Only

Title: Glycolytic Activity and Effect of Ex-Vivo Treatment with the Pyruvate Kinase (PK) Activator AG-946 in Red Blood Cells (RBC) From Low-Risk Myelodysplastic Syndromes (LR-MDS) Patients: A Proof-Of-Concept Study
Abstract: PB1990
Lead Author: Bruno Fattizzo, Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, University of Milan, Milan, Italy

Title: A Pilot Study of the International Hemoglobinopathy Research Network (INHERENT)
Abstract: PB2515
Lead Author: Petros Kountouris, Ph.D., The Cyprus Institute of Neurology and Genetics, Nicosia, Cyprus

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 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 leadership in the field of cellular metabolism, Agios is advancing a robust clinical pipeline of investigational medicines with programs in alpha- and beta-thalassemia, sickle cell disease, pediatric PK deficiency and MDS-associated anemia. In addition to its clinical pipeline, Agios has a PAH stabilizer in preclinical development as a potential treatment for phenylketonuria (PKU) and deep scientific expertise in classical hematology. For more information, please visit the company's website at .

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 and AG-946; Agios' plans regarding future data presentations; and the potential benefit of its 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 the COVID-19 pandemic 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 maintain key collaborations; the failure of Agios to receive milestone or royalty payments related to the sale of its oncology business, the uncertainty of the timing of any receipt of any such payments, and the uncertainty of the results and effectiveness of the use of proceeds from the transaction with Servier; 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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