Agios Pharmaceuticals to Present Broad Set of Clinical and Translational Data in Rare Blood Disorders at 65th ASH Annual Meeting and Exposition

November 2, 2023

– Both Mitapivat Dose Arms Achieved Statistically Significant Hemoglobin Response in Phase 2 Portion of RISE UP Pivotal Study in Sickle Cell Disease, Compared to the Placebo Arm; Data Support Advancement to Phase 3 Portion of RISE UP –

– Agios to Highlight Additional Data on the Burden of Disease in Pyruvate Kinase (PK) Deficiency and Alpha- and Beta-Thalassemia –

– New Preclinical Data for Novel PK Activator AG-946 Suggest Improvements in Ineffective Erythropoiesis in Model of Myelodysplastic Syndromes (MDS) –

– Agios to Host Live and Webcast Investor Event on Dec. 11, 2023, at 7:00 a.m. Pacific Time –

CAMBRIDGE, Mass., Nov. 02, 2023 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism pioneering therapies for rare diseases, today announced that a broad set of clinical and translational data from its programs will be presented at the 65th American Society of Hematology (ASH) Annual Meeting & Exposition, to be held Dec. 9-12, 2023, in San Diego. The presentations will focus on rare blood disorders, including PK deficiency, thalassemia, sickle cell disease and anemia associated with lower-risk myelodysplastic syndromes (LR-MDS).

In total, 23 abstracts led by Agios and external collaborators will be presented or published. The accepted abstracts are listed below and are available online on the ASH conference website at https://www.hematology.org/meetings/annual-meeting/abstracts.

PK Deficiency

Data to be presented highlight that long-term treatment with PYRUKYND® (mitapivat) in adults with PK deficiency is associated with sustained clinical benefits, including improvements in hemoglobin, iron overload, and decreased burden of disease on work and school activities.

Poster Presentations:

Title: Mitapivat Treatment Reduces Levels of Interference in Work/School Activity for Adult Patients with Pyruvate Kinase Deficiency
Poster Session: 904. Outcomes Research – Non-Malignant Conditions: Poster I
Session Date and Time: Saturday, Dec. 9, 2023, 5:30-7:30 p.m. PT
Abstract: 2365
Lead Author: Jennifer A. Rothman, MD, Duke University Medical Center, Durham, NC

Title: Understanding the Physical and Psychosocial Impacts of Pyruvate Kinase Deficiency: Patient-Led Development of the Pyruvate Kinase Deficiency Life Phase Model
Poster Session: 901. Health Services and Quality Improvement - Non-Malignant Conditions: Poster II
Session Date and Time: Sunday, Dec. 10, 2023, 6:00-8:00 p.m. PT
Abstract: 3691
Lead Author: Rachael F. Grace, MD, MMSc, Dana-Farber/Boston Children's Cancer and Blood Disorder Center, Harvard Medical School, Boston, MA

Publication Only:

Title: Improvements in Markers of Hemolysis and Liver Iron Concentration in Mitapivat-Treated Adult Patients with a Delayed Hemoglobin Response
Abstract: 5266
Lead Author: Eduard J. van Beers, MD, PhD; Center for Benign Haematology, Thrombosis and Haemostasis, Van Creveldkliniek, University Medical Center Utrecht, Utrecht University, Utrecht, The Netherlands

Title: Regional Genetic Heterogeneity Among Patients with Pyruvate Kinase Deficiency
Abstract: 5203
Lead Author: Paola Bianchi, BSc, PhD; Hematology Unit, Pathophysiology of Anemia Unit, Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, Milan, Italy

Thalassemia

Data for PYRUKYND® in adults with beta-thalassemia show evidence of reduced oxidative stress in red blood cells, as well as enhanced PK activity and metabolic reprogramming. Additionally, qualitative data demonstrate the equally common burden of disease across alpha- and beta-thalassemia patients, regardless of transfusion dependency.

Poster Presentations:

Title: Mitapivat Treatment Increases β-thalassemic Erythroblasts Energy Production and Responsiveness to Oxidative Stress
Poster Session: 112. Thalassemia and Globin Gene Regulation: Poster III
Session Date and Time: Monday, Dec. 11, 2023, 6:00-8:00 p.m. PT
Abstract: 3850
Lead Author: Alessandro Matte, PhD; Department of Medicine, University of Verona and AOUI Verona, Verona, Italy

Publication Only:

Title: Association of Hemoglobin Levels With Healthcare Resource Utilization and Costs in Non–Transfusion-Dependent α- and β-Thalassemia: A Retrospective Observational Study Using Real-World Data
Abstract: 5244
Lead Author: Arielle L. Langer, MD; MPH, Division of Hematology, Brigham & Women's Hospital, Harvard Medical School, Boston MA

Title: Burden of Illness of Alpha- and Beta-Thalassemia: A Qualitative Study
Abstract: 7329
Lead Author: Sujit Sheth, MD; Joan and Sanford I Weill Medical College of Cornell University, New York, NY

Title: Investigating Health Literacy in Thalassemia: Founding a Patient-Led Research Approach
Abstract: 5251
Lead Author: Sujit Sheth, MD; Joan and Sanford I Weill Medical College of Cornell University, New York, NY

Sickle Cell Disease
Results from the Phase 2 portion of Agios' RISE UP study will highlight improvements in hemoglobin response rates, markers of hemolysis and erythropoiesis and reductions in annualized rates of pain crises for mitapivat compared to placebo, supporting advancement into the Phase 3 portion of RISE UP. Agios will showcase its patient-centric approach to sickle cell disease clinic trials, including the incorporation of patient insights and decision making into the clinical trial design and campaign.

Oral Presentations:

Title: A Phase 2/3, Double-Blind, Randomized, Placebo-Controlled, Multicenter Study of Mitapivat in Patients With Sickle Cell Disease: RISE UP Phase 2 Results
Presentation Time: Saturday, Dec. 9, 2023, at 4:00 p.m. PT
Oral Abstract Session: 114. Sickle Cell Disease, Sickle Cell Trait and Other Hemoglobinopathies, Excluding Thalassemias: Clinical and Epidemiological: Building on Momentum in Disease-Modifying Therapeutics for Sickle Cell Disease
Abstract: 271
Presenter: Modupe Idowu, MD; McGovern Medical School, UT Health, Houston, TX

Title: Long-term safety and Efficacy of Mitapivat, an Oral Pyruvate Kinase Activator, in Adults with Sickle Cell Disease: Extension of a Phase 1 Dose Escalation Study
Presentation Time: Saturday, Dec. 9, 2023, at 4:30 p.m. PT
Oral Abstract Session: 114. Sickle Cell Disease, Sickle Cell Trait and Other Hemoglobinopathies, Excluding Thalassemias: Clinical and Epidemiological: Building on Momentum in Disease-Modifying Therapeutics for Sickle Cell Disease
Abstract: 273
Presenter: Swee Lay Thein, MBBS, DSc, FRCP, FRCPath, MRCP, MRCPath; Sickle Cell Branch, National Heart, Lung & Blood Institute, NIH, Bethesda, MD

Title: Pyruvate Kinase Thermostability Is Associated with Red Blood Cell Adhesion, Deformability and Oxygen Affinity in Patients with Sickle Cell Disease
Presentation Time: Sunday, Dec. 10, 2023, at 5:00 p.m. PT
Oral Abstract Session: 113. Sickle Cell Disease, Sickle Cell Trait and Other Hemoglobinopathies, Excluding Thalassemias: Basic and Translational: Pathophysiology of Sickle Hemoglobinopathies: From Mice to Humans
Abstract: 561
Lead Author: Marissa J.M. Traets, PhD Candidate; Department of Central Diagnostic Laboratory - Research, University Medical Center Utrecht, Utrecht University, Utrecht, The Netherlands

Poster Presentations:

Title: A Patient-Centric Approach to Sickle Cell Disease Clinical Trials: Integrating Patient Perspectives in the RISE UP Phase 2/3 Trial of Mitapivat for Informed Protocol Design and Associated Patient Community Benefit
Poster Session: 904. Outcomes Research – Non-Malignant Conditions: Poster I
Session Date and Time: Saturday, Dec. 9, 2023, 5:30-7:30 p.m. PT
Abstract: 2376
Lead Author: Charles Jonassaint, PhD, MHS; School of Medicine, University of Pittsburgh, Pittsburgh, PA

Title: One-Year Safety and Efficacy of Mitapivat in Sickle Cell Disease: Follow-Up Results of a Phase 2, Open-Label Study
Poster Session: 114. Sickle cell Disease, Sickle Cell Trait and Other Hemoglobinopathies, Excluding Thalassemias: Clinical and Epidemiological: Poster II
Session Date and Time: Sunday, Dec. 10, 2023, 6:00-8:00 p.m. PT
Abstract: 2515
Lead Author: Myrthe J. van Dijk, PhD; Division Laboratories, Pharmacy and Biomedical Genetics, CDL en Van Creveldkliniek, University Medical Center Utrecht, Utrecht University, Utrecht, Netherlands

Title: Functional and Multi-omics Signatures of Mitapivat Efficacy Upon Activation of Pyruvate Kinase in Red Blood Cells from Patients with Sickle Cell Disease
Poster Session: 113. Sickle Cell Disease, Sickle Cell Trait and Other Hemoglobinopathies, Excluding Thalassemias: Basic and Translational: Poster II
Session Date and Time: Sunday, Dec. 10, 2023, 6:00-8:00 p.m. PT
Abstract: 2485
Lead Author: Angelo D'Alessandro, PhD; Biochemistry and Molecular Genetics, University of Colorado Anschutz Medical Campus, Aurora, CO

Title: Longitudinal Characterization of Hemodynamic Changes with Multimodal Optical Techniques in Patients with Sickle Cell Disease Treated with

Mitapivat
Poster Session: 113. Sickle Cell Disease, Sickle Cell Trait and Other Hemoglobinopathies, Excluding Thalassemias: Basic and Translational: Poster II
Session Date and Time: Sunday, Dec. 10, 2023, 6:00-8:00 p.m. PT
Abstract: 2492
Lead Author: Timothy Quang, PhD; Section on Biomedical Optics, National Institute of Child Health and Human Development, NIH, Bethesda, MD

Publication Only:

Title: Outpatient Costs of Patients with Sickle Cell Disease With or Without Hydroxyurea at an Institution in Rio de Janeiro, Brazil
Abstract: 7325
Lead Author: Tarun Aurora, MD; Department of Global Pediatric Medicine, St Jude Children's Research Hospital, Memphis, TN

AG-946
New data from preclinical studies of Agios' AG-946 continue to support its novel mechanism of action and PK activation as a promising potential treatment option for anemia associated with lower-risk myelodysplastic syndromes.

Poster Presentations:

Title: AG-946, An Activator of Pyruvate Kinase, Improves Ineffective Erythropoiesis in the Bone Marrow of Mouse Models of Myelodysplastic Syndromes
Poster Session: 636. Myelodysplastic Syndromes – Basic and Translational: Poster I
Session Date and Time: Saturday, Dec. 9, 2023, 5:30-7:30 p.m. PT
Abstract: 1854
Lead Author: Megan Wind-Rotolo, PhD, Agios Pharmaceuticals, Cambridge, MA

Title: The Pyruvate Kinase (PK) Activator AG-946 Improves PK Properties and Red Blood Cell (RBC) Characteristics upon Ex Vivo Treatment of RBCs from Patients with Myelodysplastic Syndromes
Poster Session: 636. Myelodysplastic Syndromes—Basic and Translational: Poster II
Session Date and Time: Sunday, Dec. 10, 2023, 6:00-8:00 p.m. PT
Abstract: 3222
Lead Author: Jonathan R.A. de Wilde, PhD Candidate; Red Blood Cell Research Group, Central Diagnostic Laboratory-Research, University Medical Center Utrecht, Utrecht University, Utrecht, the Netherlands

Publication Only:

Title: Biochemical and Metabolomic Analysis of Glycolytic Activity in Red Blood Cells (RBC) from Low-risk Myelodysplastic Syndromes (LR-MDS) Patients and In-vitro Effect of the Pyruvate Kinase (PK) Activator AG-946
Abstract: 6456
Lead Author: Bruno Fattizzo, MD; Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, University of Milan, Milan, Italy

Patient Advocacy
Agios is fueled by connections, with patient and KOL engagement at the core of our mission. Information presented provides an overview of the formation and vision of the Red Cell Revolution – a multi-stakeholder, patient-advocacy data collection approach to understanding the unmet needs of the patients, caregivers and healthcare professionals for PK deficiency, sickle cell disease and thalassemia.

Poster Presentation:

Title: Cross-community Collaboration and Data Collection to Optimize Patient Care in Hemolytic Anemias
Poster Session: 901. Health Services and Quality Improvement - Non-Malignant Conditions: Poster II
Session Date and Time: Sunday, Dec. 10, 2023, 6:00-8:00 p.m. PT
Abstract: 3692
Lead Author: Biree Andemariam, MD; New England Sickle Cell Institute, University of Connecticut Health, Farmington, CT

Publication Only:

Title: Setting Industry Standards for Patient Engagement, Partnership, Allyship and Care: The Patient Vision Project
Abstract: 7233
Lead Author: Biree Andemariam, MD; New England Sickle Cell Institute, University of Connecticut Health, Farmington, CT

Other
Agios' collaborators present new data evaluating mitapivat as a potential treatment for other rare blood diseases.

Poster Presentation:

Title: Ex Vivo Treatment by Mitapivat, an Allosteric Pyruvate Kinase Activator, Reduced Oxidative Stress and Promoted Terminal Erythropoiesis in a Severe Hemolytic Anemia Patients Due to Krüppel-like Factor 1 Mutations
Poster Session: 101. Red Cells and Erythropoiesis, Excluding Iron: Poster I
Session Date and Time: Saturday, Dec. 9, 2023, 5:30-7:30 p.m. PT
Abstract: 1071
Lead Author: Thidarat Suksangpleng, PhD.; Siriraj-Thalassemia Center, Faculty of Medicine Siriraj Hospital, Mahidol University, Bangkok, Thailand

Title: Safety and Efficacy of Mitapivat Sulfate in Adult Patients with Erythrocyte Membranopathies (SATISFY)
Poster Session: 101. Red Cells and Erythropoiesis, Excluding Iron: Poster I
Session Date and Time: Saturday, Dec. 9, 2023, 5:30-7:30 p.m. PT
Abstract: 1085

Lead Author: Andreas Glenthøj, MD, PhD; Department of Haematology, Copenhagen University Hospital - Rigshospitalet, Copenhagen, Denmark

Conference Call Information
Agios will host a live investor event on Dec. 11, 2023, at 7:00 a.m. PT in San Diego to review the key clinical oral and poster presentations from this year's ASH 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 and MDS-associated anemia. In addition to its clinical pipeline, Agios is advancing a preclinical TMPRSS6 siRNA as a potential treatment for polycythemia vera, and a preclinical PAH stabilizer as a potential treatment for phenylketonuria (PKU). 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) and AG-946; 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 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 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; 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; 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 proceeds from the transaction with Servier; competitive factors; 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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