Agios Pharmaceuticals to Present Broad Set of Clinical and Translational Data in Chronic Hemolytic Anemias at 64th ASH Annual Meeting and Exposition

November 3, 2022

– Long-term Data for PYRUKYND® (mitapivat) in Adults with Pyruvate Kinase (PK) Deficiency and Adults with Thalassemia Demonstrate Sustained Improvement in Hemoglobin, Markers of Hemolysis and Other Disease Impacts –

– New Data from Single Ascending Dose (SAD) and Multiple Ascending Dose (MAD) Cohorts of Phase 1 Study of AG-946 in Healthy Volunteers Support Profile as Potent PK Activator and Initiation of Phase 2 Study in Myelodysplastic Syndromes (MDS)-associated Anemia –

– Agios to Host Live and Webcast Investor Event on Dec. 12, 2022, at 7 a.m. CT –

CAMBRIDGE, Mass., Nov. 03, 2022 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism to treat rare and genetically defined diseases, today announced that a broad set of clinical and translational data from its programs in hemolytic anemias, including PK deficiency, thalassemia and sickle cell disease, will be presented at the 64th American Society of Hematology (ASH) Annual Meeting & Exposition, to be held Dec. 10-13, 2022, in New Orleans.

In total, 22 abstracts led by Agios and external collaborators will be presented. 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® in adults with PK deficiency is associated with sustained clinical benefits, including improvements in hemoglobin, iron overload, transfusion burden and patient-reported outcomes. In addition, Agios is presenting data supporting its pediatric PK deficiency program, including characterization of disease complications and co-morbidities in pediatric patients.

Oral Presentation:

Title: Long-term Improvements in Patient-reported Outcomes in Patients with Pyruvate Kinase Deficiency Treated with Mitapivat
Presentation Time: Sunday, Dec. 11, 2022, at 9:45 a.m. CT
Oral Abstract Session: 904. Outcomes Research—Non-malignant Conditions: Classical Hematology: From Horses to Zebras
Abstract: 506
Presenter: Kevin H. M. Kuo, M.D., Division of Hematology, University of Toronto, Toronto, Canada

Poster Presentations:

Title: Next Generation Sequencing for the Diagnosis of Hereditary Hemolytic Anemias Including Pyruvate Kinase Deficiency: Report from a No-cost Diagnostic Program
Poster Session: 101. Red Cells and Erythropoiesis, Excluding Iron: Poster I
Session Date and Time: Saturday, Dec. 10, 2022, 5:30-7:30 p.m. CT
Abstract: 1010
Lead Author: Jorune Balciuniene, PerkinElmer Genomics

Title: Mitapivat Improves Iron Overload in Patients with Pyruvate Kinase Deficiency
Poster Session: 102. Iron Homeostasis and Biology: Poster I
Session Date and Time: Saturday, Dec. 10, 2022, 5:30-7:30 p.m. CT
Abstract: 1021
Lead Author: Kevin H. M. Kuo, M.D., Division of Hematology, University of Toronto, Toronto, Canada

Title: Long-term Hemoglobin Response and Reduction in Transfusion Burden Are Maintained in Patients with Pyruvate Kinase Deficiency Treated with Mitapivat
Poster Session: 101. Red Cells and Erythropoiesis, Excluding Iron: Poster II
Session Date and Time: Sunday, Dec. 11, 2022, 6-8 p.m. CT
Abstract: 2328
Lead Author: Rachael F. Grace, M.D., MMSc, Dana-Farber/Boston Children's Cancer and Blood Disorders Center

Title: Comorbidities and Complications in Pediatric Patients with Pyruvate Kinase Deficiency Enrolled in the Peak Registry / Peak Pediatric Comorbidities
Poster Session: 101. Red Cells and Erythropoiesis, Excluding Iron: Poster II
Session Date and Time: Sunday, Dec. 11, 2022, 6-8 p.m. CT
Abstract: 2329
Lead Author: Rachael F. Grace, M.D., MMSc, Dana-Farber/Boston Children's Cancer and Blood Disorders Center

Title: ACTIVATE-KidsT: Mitapivat in Children with Pyruvate Kinase Deficiency Who Are Regularly Transfused
Poster Session: 101. Red Cells and Erythropoiesis, Excluding Iron: Poster II
Session Date and Time: Sunday, Dec. 11, 2022, 6-8 p.m. CT
Abstract: 2330
Lead Author: Rachael F. Grace, M.D., MMSc, Dana-Farber/Boston Children's Cancer and Blood Disorders Center

Title: Age of Onset of Complications in Patients with Pyruvate Kinase Deficiency: Analysis from the Peak Registry
Poster Session: 101. Red Cells and Erythropoiesis, Excluding Iron: Poster II
Session Date and Time: Sunday, Dec. 11, 2022, 6-8 p.m. CT
Abstract: 2332
Lead Author: Andreas Glenthøj, M.D., Ph.D., Department of Haematology, Copenhagen University Hospital - Rigshospitalet, Copenhagen, Denmark

Title: ACTIVATE-Kids: Mitapivat in Children with Pyruvate Kinase Deficiency Who Are Not Regularly Transfused
Poster Session: 101. Red Cells and Erythropoiesis, Excluding Iron: Poster II
Session Date and Time: Sunday, Dec. 11, 2022, 6-8 p.m. CT
Abstract: 2335
Lead Author: Rachael F. Grace, M.D., MMSc, Dana-Farber/Boston Children's Cancer and Blood Disorders Center

Title: Mitapivat Improves Markers of Hemolysis and Erythropoiesis in Patients with Pyruvate Kinase Deficiency Irrespective of Hemoglobin Response
Poster Session: 101. Red Cells and Erythropoiesis, Excluding Iron: Poster III
Session Date and Time: Monday, Dec. 12, 2022, 6-8 p.m. CT
Abstract: 3644
Lead Author: Hanny Al-Samkari, M.D., Division of Hematology, Massachusetts General Hospital, Harvard Medical School, Boston, MA, United States

Publication Only:

Title: The Launch of Two Sub-studies of the Peak Registry, A Global, Longitudinal Study of Pyruvate Kinase Deficiency
Abstract: 4960
Lead Author: Carl Lander, Thrive with Pyruvate Kinase Deficiency Patient Organization

Thalassemia

Long-term Phase 2 data for PYRUKYND® in adults with alpha- or beta-thalassemia treated for up to 72 weeks show sustained hemoglobin response and improvements in hemolysis and ineffective erythropoiesis. Additional data underscore the significant disease burden across both alpha- and beta-thalassemia.

Poster Presentations:

Title: Mitapivat Improves Markers of Erythropoietic Activity in Long-term Study of Adults with Alpha- or Beta-non-transfusion-dependent Thalassemia
Poster Session: 112. Thalassemia and Globin Gene Regulation: Poster I
Session Date and Time: Saturday, Dec. 10, 2022, 5:30-7:30 p.m. CT
Abstract: 1030
Lead Author: Kevin H. M. Kuo, M.D., Division of Hematology, University of Toronto, Toronto, Canada

Title: Characterizing the Clinical, Health-related Quality of Life and Economic Burden of Alpha-thalassemia: A Systematic Literature Review and Evidence Gaps Assessment
Poster Session: 112. Thalassemia and Globin Gene Regulation: Poster I
Session Date and Time: Saturday, Dec. 10, 2022, 5:30-7:30 p.m. CT
Abstract: 1036
Lead Author: Khaled M. Musallam, M.D., Ph.D., Thalassemia Center, Burjeel Medical City, Abu Dhabi, United Arab Emirates

Title: Clinical Burden of Alpha- and Beta-thalassemia Compared to Matched Controls in the Real-world Setting
Poster Session: 112. Thalassemia and Globin Gene Regulation: Poster II
Session Date and Time: Sunday, Dec. 11, 2022, 6-8 p.m. CT
Abstract: 2351
Lead Author: Arielle L. Langer, M.D., MPH, Division of Hematology, Brigham & Women’s Hospital

Sickle Cell Disease

Data to be presented continue to highlight that PK activation may be a promising therapeutic approach for patients with sickle cell disease.

Oral Presentations:

Title: Effects of Pyruvate Kinase Activators on Red Blood Cell Rheology, Sickling and Senescence in Sickle Cell Disease
Presentation Time: Saturday, Dec. 10, 2022, at 9:45 a.m. CT
Oral Abstract Session: 114. Hemoglobinopathies, Excluding Thalassemia: Clinical and Epidemiological: Novel Therapies
Abstract: 8
Presenter: Philippe Joly, Ph.D., Laboratory of Biochemistry and Molecular Biology, UF Biochemistry of Red Blood Cell Diseases, Est Center of Biology and Pathology, Hospices Civils de Lyon, Lyon France

Title: Untargeted Metabolomics in Dried Blood Spots of Patients with Sickle Cell Disease Treated with the Pyruvate Kinase Activator Mitapivat
Presentation Time: Saturday, Dec. 10, 2022, at 10 a.m. CT
Oral Abstract Session: 114. Hemoglobinopathies, Excluding Thalassemia: Clinical and Epidemiological: Novel Therapies
Abstract: 9
Presenter: Myrthe J. van Dijk, Van Creveldkliniek, University Medical Center Utrecht, Utrecht University, Utrecht, The Netherlands

Title: AG-946 Normalizes Glycolysis and Improves Red Cell Indices in a Humanized Sickle Cell Mouse Model
Presentation Time: Sunday, Dec. 11, 2022, at 9:30 a.m. CT
Oral Abstract Session: 113. Hemoglobinopathies, Excluding Thalassemia: Basic and Translational: Targeting the Red Blood Cell: Novel Therapeutic Approaches in Sickle Cell Disease
Abstract: 391
Lead Author: Rohitash Jamwal, Ph.D., Agios Pharmaceuticals

Title: PKLR Variants Associated with Acute Pain in Sickle Cell Disease Influence ATP Concentrations in Red Blood Cells
Presentation Time: Sunday, Dec. 11, 2022, at 10 a.m. CT
Oral Abstract Session: 113. Hemoglobinopathies, Excluding Thalassemia: Basic and Translational: Targeting the Red Blood Cell: Novel Therapeutic Approaches in Sickle Cell Disease
Abstract: 393
Presenter: Xunde Wang, Ph.D., Sickle Cell Branch, National Heart Lung and Blood Institute, National Institutes of Health

Poster Presentations:

Title: The Pyruvate Kinase Activator Mitapivat Improves Red Blood Cell Deformability and Sickling Kinetics in Adult Patients with Sickle Cell Disease
Poster Session: 113. Hemoglobinopathies, Excluding Thalassemia: Basic and Translational: Poster I
Session Date and Time: Saturday, Dec. 10, 2022, 5:30-7:30 p.m. CT
Abstract: 1044
Lead Author: Maureen Lundt, NHLBI / NIH, Bethesda, Maryland, United States

Title: Feasibility of Near-infrared Spectroscopy for Monitoring Hemodynamic Changes in Sickle Cell Disease Patients Treated with Mitapivat
Poster Session: 113. Hemoglobinopathies, Excluding Thalassemia: Basic and Translational: Poster I
Session Date and Time: Saturday, Dec. 10, 2022, 5:30-7:30 p.m. CT
Abstract: 1053
Lead Author: Timothy Quang, National Institute of Child Health and Human Development

Title: Activating Pyruvate Kinase Improves Red Blood Cell Integrity by Reducing Band3 Tyrosine Phosphorylation
Poster Session: 113. Hemoglobinopathies, Excluding Thalassemia: Basic and Translational: Poster II
Session Date and Time: Sunday, Dec. 11, 2022, 6-8 p.m. CT
Abstract: 2367
Lead Author: Kang Le, Ph.D., National Institutes of Health

AG-946
New clinical data from the Phase 1 healthy volunteers study of Agios' novel PK activator, AG-946, support the initiation of a Phase 2a/2b study in lower-risk myelodysplastic syndromes.

Poster Presentations:

Title: A Phase 2a/2b Multicenter Study of AG-946 in Patients with Anemia Due to Lower-risk Myelodysplastic Syndromes
Poster Session: 637. Myelodysplastic Syndromes – Clinical and Epidemiological: Poster I
Session Date and Time: Saturday, Dec. 10, 2022, 5:30-7:30 p.m. CT
Abstract: 1773
Lead Author: Hanny Al-Samkari, M.D., Division of Hematology, Massachusetts General Hospital, Harvard Medical School, Boston, MA, United States

Title: Results from the Single and Multiple Ascending Dose Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of AG-946 in Healthy Volunteers
Poster Session: 114. Hemoglobinopathies, Excluding Thalassemia: Clinical and Epidemiological: Poster II
Session Date and Time: Sunday, Dec. 11, 2022, 6:00-8:00 p.m. CT
Abstract: 2383
Lead Author: Xiaoshu Dai, Ph.D., Agios Pharmaceuticals

Conference Call Information
Agios will host a live investor event on Dec. 12, 2022, at 7:00 a.m. CT in New Orleans 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 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 and 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 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 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 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; 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 ;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.

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