# Agios to Present Broad Set of Clinical and Translational Data in Chronic Hemolytic Anemias at 63rd ASH Annual Meeting and Exposition

November 4, 2021

*– Extension Data for Mitapivat in PK Deficiency and Thalassemia Demonstrate Long-term Safety Profile and Durable Improvement in Hemoglobin and Markers of Hemolysis –*

*– New Data from Two Investigator Sponsored Studies of Mitapivat in Sickle Cell Disease Continue to Support Advancement to Pivotal Development –*

*– First Data from the Phase 1 Healthy Volunteer Study of AG-946 Show Dose-dependent Changes in ATP and 2,3-DPG –*

*– Agios to Host Investor Webcast on Dec. 14, 2021, at 7:30 a.m. ET –*

CAMBRIDGE, Mass., Nov. 04, 2021 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism to treat genetically defined diseases, today announced that a broad set of clinical and translational data from its genetically defined disease programs in chronic hemolytic anemias, including pyruvate kinase (PK) deficiency, thalassemia and sickle cell disease, will be presented at the American Society of Hematology (ASH) Annual Meeting & Exposition, to be held Dec. 11-14, 2021, virtually and in person in Atlanta.

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

**Presentations by Agios**

***Oral Presentations***:

**Title**: Long-term Efficacy and Safety of the Oral Pyruvate Kinase Activator Mitapivat in Adults with Non-transfusion-dependent Alpha- or Beta-Thalassemia
**Presentation Time**: Monday, Dec. 13, 2021, at 11:45 a.m. ET
**Oral Abstract Session**: 112. Thalassemia and Globin Gene Regulation
**Abstract**: 576
**Presenter**: Kevin H. M. Kuo, M.D., Division of Hematology, University of Toronto, Toronto, Canada

**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
**Presentation Time**: Monday, Dec. 13, 2021, at 6:30 p.m. ET
**Oral Abstract Session**: 101. Red Cells and Erythropoiesis, Excluding Iron: Normal and Perturbed Erythropoiesis
**Location:** Georgia World Congress Center, B401-B402
**Abstract**: 848
**Presenter**: Rachael Grace, M.D., MMSc, Dana-Farber/Boston Children's Cancer and Blood Disorders Center

***Poster Presentations***:

**Title**: Bone Mineral Density Remains Stable in Pyruvate Kinase Deficiency Patients Receiving Long-term Treatment with Mitapivat
**Poster Session**: 101. Red Cells and Erythropoiesis, Excluding Iron: Poster I
**Session Date and Time:** Saturday, Dec. 11, 2021, from 5:30-7:30 p.m. ET
**Abstract**: 924
**Lead Author**: Hanny Al-Samkari, M.D., Division of Hematology, Massachusetts General Hospital, Harvard Medical School, Boston, MA, United States

**Title**: Survey of 275 Patients and Caregivers Affected by Pyruvate Kinase Deficiency: Impact of Communication with Hematologists on Mental Health and Quality of Life
**Poster Session**: 904. Outcomes Research—Non-Malignant Conditions: Poster I
**Session Date and Time:** Saturday, Dec. 11, 2021, from 5:30-7:30 p.m. ET
**Abstract**: 152326
**Lead Author**: Rachael Grace, M.D., MMSc, Dana-Farber/Boston Children's Cancer and Blood Disorders Center

**Title**: Mitapivat Improves Ineffective Erythropoiesis and Reduces Iron Overload in Patients with Pyruvate Kinase Deficiency
**Poster Session**: 102. Iron Homeostasis and Biology: Poster II
**Session Date and Time:** Sunday, Dec. 12, 2021, from 6-8 p.m. ET
**Abstract**: 2005
**Lead Author**: Eduard J. van Beers, M.D., Ph.D., Department of Internal Medicine, University Medical Center Utrecht

**Title**: Phase 1 Single and Multiple Ascending Dose Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of AG-946 in Healthy Volunteers
**Poster Session**: 113. Hemoglobinopathies, Excluding Thalassemia: Poster II
**Session Date and Time:** Sunday, Dec. 12, 2021, from 6-8 p.m. ET
**Abstract**: 2043
**Lead Author**: Varsha Iyer, Ph.D., Agios Pharmaceuticals

**Title**: Characterizing Iron Overload by Age in Patients Diagnosed with Pyruvate Kinase Deficiency – A Descriptive Analysis from the PEAK Registry
**Poster Session**: 102. Iron Homeostasis and Biology: Poster III
**Session Date and Time:** Monday, Dec. 13, 2021, from 5:30-7:30 p.m. ET
**Abstract**: 3074
**Lead Author**: Paola Bianchi, BSc, Ph.D., UOC Ematologia, Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, Milan, Italy

**Title**: A Phase 2/3, Randomized, Double-blind, Placebo-controlled Study of Mitapivat in Patients with Sickle Cell Disease
**Poster Session**: 114. Hemoglobinopathies, Excluding Thalassemia: Clinical and Epidemiological: Poster III
**Session Date and Time:** Monday, Dec. 13, 2021, from 6-8 p.m. ET
**Abstract**: 3109
**Lead Author**: Joanna Howard, MB BChir, MRCP, Department of Hematology, Guy's and St. Thomas' NHS Foundation Trust, London, United Kingdom

***Publication Only**:*

**Title**: Validation of the Pyruvate Kinase Deficiency Impact Assessment (PKDIA): A Patient-reported Outcome Measure for Pyruvate Kinase (PK) Deficiency
**Abstract**: 4145
**Lead Author**: Shayna Egan, MPH, Endpoint Outcomes

**Title**: Validation of the Pyruvate Kinase Deficiency Diary (PKDD): A Patient-reported Outcome Measure for Pyruvate Kinase (PK) Deficiency
**Abstract**: 4144
**Lead Author**: Shayna Egan, MPH, Endpoint Outcomes

**Presentations by External Collaborators**

***Oral Presentations**:*

**Title**: Mitapivat (AG-348) Demonstrates Safety, Tolerability, and Improvements in Anemia, Hemolysis, Oxygen Affinity, and Hemoglobin S Polymerization Kinetics in Adults with Sickle Cell Disease: A Phase 1 Dose Escalation Study
**Presentation Time**: Saturday, Dec. 11, 2021, at 10:15 a.m. ET
**Oral Abstract Session**: 114. Hemoglobinopathies, Excluding Thalassemia: Clinical and Epidemiological: New Therapies for Sickle Cell Disease
**Abstract**: 10
**Presenter**: Julia Z. Xu, M.D., Sickle Cell Branch, National Heart, Lung, and Blood Institute, National Institutes of Health

***Poster Presentations:***

**Title**: Ex Vivo Evaluation of Erythrocyte Adhesion and Whole Blood Thrombosis in PKD Subjects
**Poster Session**: 101. Red Cells and Erythropoiesis, Excluding Iron: Poster I
**Session Date and Time**: Saturday, Dec. 11, 2021, from 5:30-7:30 p.m. ET
**Abstract**: 923
**Lead Author**: Patrick C. Hines, M.D., Ph.D., Wayne State University School of Medicine

**Title**: Safety and Efficacy of Mitapivat (AG-348), An Oral Activator of Pyruvate Kinase-R, in Subjects with Sickle Cell Disease: A Phase 2, Open-label Study (ESTIMATE)
**Poster Session**: 102. Iron Homeostasis and Biology: Poster II
**Session Date and Time:** Sunday, Dec. 12, 2021, from 6-8 p.m. ET
**Abstract**: 2005
**Lead Author**: Myrthe J. van Dijk, Van Creveldkliniek, University Medical Center Utrecht, Utrecht University, Utrecht, The Netherlands

**Title**: Pharmacodynamic Effects of AG-946, A Highly Potent Next-generation Activator of Pyruvate Kinase, In Ex Vivo Treatment of Red Blood Cells from Sickle Cell Disease Patients
**Poster Session**: 113. Hemoglobinopathies, Excluding Thalassemia: Basic and Translational: Poster II
**Session Date and Time:** Sunday, Dec. 12, 2021, from 6-8 p.m. ET
**Abstract**: 2029
**Lead Author**: Minke A. E. Rab, University Medical Center Utrecht

**Title**: Mitapivat Improves Transfusion Burden and Reduces Iron Overload in Thalassemic Mice
**Poster Session**: 112. Thalassemia and Globin Gene Regulation: Poster II
**Session Date and Time:** Sunday, Dec. 12, 2021, from 6-8 p.m. ET
**Abstract**: 2016
**Lead Author**: Alessandro Mattè, Ph.D., University of Verona and AOUI Verona, Verona, Italy

**Conference Call Information**
Agios will host a virtual investor event on Dec. 14, 2021, at 7:30 a.m. ET 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 focused on discovering and developing novel investigational medicines to treat genetically defined diseases through scientific leadership in the field of cellular metabolism. The company's most advanced drug candidate is a first-in-class pyruvate kinase R (PKR) activator, mitapivat, that is currently being evaluated for the treatment of three distinct hemolytic anemias. In addition to its active late-stage clinical pipeline, Agios has multiple novel, investigational therapies in clinical and preclinical development. 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," "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. 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 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; the impact of the COVID-19 pandemic on 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 future approved products, and launching, marketing and selling 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, including with respect to the regulatory submissions for mitapivat, 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 and 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 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, or SEC, including the risks and uncertainties set forth under the heading Risk Factors in our filings with the SEC. While the list of factors presented here is considered representative, this list should not be considered to be a complete statement of all potential risks and uncertainties. Any forward-looking statements contained in this communication are made only as of the date hereof, and we undertake no obligation to update forward-looking statements to reflect developments or information obtained after the date hereof and disclaim any obligation to do so other than as may be required by law.

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