Agios to Present New Mitapivat Data in Rare Blood Disorders at 67th ASH Annual Meeting and Exposition

November 3, 2025

CAMBRIDGE, Mass., Nov. 03, 2025 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a commercial-stage biopharmaceutical company focused on delivering innovative medicines for patients with rare diseases, today announced that new data on mitapivat, an oral pyruvate kinase (PK) activator, will be featured in oral and poster presentations during the 67th American Society of Hematology (ASH) Annual Meeting and Exposition (ASH 2025) in Orlando, Florida, December 6-9, 2025.

“This year’s presentations at ASH highlight the growing momentum of our PK activation franchise, featuring new clinical and preclinical data that reinforce the therapeutic potential of mitapivat for patients with thalassemia, sickle cell disease, and PK deficiency – debilitating and life-threatening rare blood disorders with few or no treatment options,” said Sarah Gheuens, M.D., Ph.D., Chief Medical Officer and Head of R&D, Agios. “Building on these findings, we are also sharing research led by two of our advisory councils, each comprised of patients, caregivers, advocates, and physicians, that deepens our understanding of these rare diseases and helps guide the development of critical studies and resources tailored to patient needs. ASH provides a vital platform to showcase this important body of data and strengthen our connections with the hematology and rare disease communities.”

Select presentations and publications at ASH 2025 will include:

- Two poster presentations on results from the ENERGIZE-T Phase 3 trial of mitapivat in adults with transfusion-dependent alpha- or beta-thalassemia.
    - The first is a subgroup analysis of patients with alpha-thalassemia, showing that 77.8% (7 of 9) of individuals in the mitapivat arm achieved the primary endpoint of transfusion reduction response, compared to 0% (0 of 3) in the placebo arm. Additionally, reductions in transfusion burden were observed in the mitapivat arm versus none in the placebo arm for all key secondary endpoints.
    - The second highlights long-term results from the 17 patients who achieved transfusion independence with mitapivat during the double-blind phase of ENERGIZE-T, showing that the mean duration of their longest transfusion-free period was 30.5 weeks, with a maximum of 84.3 weeks, across the double-blind and ongoing open-label extension periods.
- An oral presentation with preclinical data from an investigator-led study demonstrating that mitapivat protects against cardiomyopathy (heart muscle disease) in a mouse model of beta-thalassemia, with this mechanism potentially linked to its activation of the PKM2 isoform (or variant) of the PK enzyme in the heart. Cardiomyopathy is a leading cause of morbidity and mortality in patients with hemolytic anemias.
- A poster presentation with positive findings from the ACTIVATE-Kids Phase 3 trial of mitapivat in children aged 1 to <18 years with PK deficiency who are not regularly transfused. The trial met its primary endpoint, with the mitapivat arm showing a higher hemoglobin response rate compared to the placebo arm. Additionally, the mitapivat arm showed improvements in changes from baseline for hemoglobin concentration and markers of hemolysis (indirect bilirubin and lactate dehydrogenase) compared to the placebo arm. The safety results were consistent with the safety profile for mitapivat previously observed for adult patients with PK deficiency who are not regularly transfused.
- Research from two Agios-supported advisory councils, each comprised of patients, caregivers, advocates, and physicians, that builds on clinical and preclinical findings to help advance the scientific understanding of rare blood disorders.
    - The first is a poster from the Thalassemia Advocacy Advisory Council, which showcases a global patient survey that identified key knowledge gaps about thalassemia, including awareness of complication risks at certain hemoglobin levels and the importance of regular monitoring in non-transfusion-dependent patients.
    - The second is a publication-only study from the Red Cell Revolution, which highlights interim results of a qualitative survey assessing the physical, mental, and emotional impact of fatigue across patients with thalassemia, sickle cell disease, and PK deficiency, with cognitive impairment reported as the most bothersome manifestation of fatigue.

In total, 10 presentations and publications led by Agios and external collaborators will be shared at ASH 2025.

ASH 2025 Accepted Abstracts

Title	Number	Date/Time	Presenter	Acceptance
Thalassemia				
Efficacy of Mitapivat in Patients with Transfusion-Dependent Alpha-Thalassemia: Subgroup Analysis from the ENERGIZE-T Trial	4699	Monday, December 8, 2025, 6:00 – 8:00 p.m. EST	Ashutosh Lal, M.D., MBBS, University of California San Francisco Benioff Children's Hospital Oakland	Poster

Long-Term Transfusion-Free Duration and Impact on Transfusion-Related Burdens: Results from the Ongoing ENERGIZE-T Open-Label Extension Study of Mitapivat in Transfusion-Dependent Alpha- or Beta-Thalassemia	4697	Monday, December 8, 2025, 6:00 – 8:00 p.m. EST	Sujit Sheth, M.D., Weill Cornell Medicine	Poster
Ex Vivo Treatment by Mitapivat, an Allosteric Pyruvate Kinase Activator, Reduced Oxidative Stress to Support Terminal Erythropoiesis of Non-Transfusion Dependent Thalassemia Patients Due to β-Thalassemia/Hb E Disease	2916	Sunday, December 7, 2025, 6:00 – 8:00 p.m. EST	Thidarat Suksangpleng, Ph.D., Siriraj Hospital, Siriraj-Thalassemia Center, Mahidol University, Bangkok, Thailand	Poster
Long-Term Mitapivat Treatment Improves Inflammatory Pro-Fibrotic Cardiomyopathy in a Murine Model of β-Thalassemia	727	Monday, December 8, 2025, 10:30 - 10:45 a.m. EST	Enrica Federti, Ph.D., University of Verona, Italy	Oral
Sickle Cell Disease				
Mitapivat Improves RBC Integrity by Reducing Membrane Ubiquitination Accumulation	1146	Saturday, December 6, 2025, 5:30 - 7:30 p.m. EST	Kang Le, Ph.D., National Heart, Lung, and Blood Institute, National Institutes of Health	Poster
Pyruvate Kinase Deficiency				
Efficacy and Safety of Mitapivat in Pediatric Patients with Pyruvate Kinase Deficiency Who Are Not Regularly Transfused: Results from the Phase 3, Global, Randomized, Double-Blind, Placebo-Controlled ACTIVATE-Kids Trial	4654	Monday, December 8, 2025, 6:00 - 8:00 p.m. EST	Satheesh Chonat, M.D., Emory University School of Medicine and Aflac Cancer and Blood Disorders Center, Children's Healthcare of Atlanta	Poster
Disease Monitoring and Management Among Pediatric Patients with Pyruvate Kinase Deficiency: Real-World Practices from Pyruvate Kinase Deficiency Registries Prior to 2024 International Expert Guidelines	4454	Sunday, December 7, 2025, 6:00 - 8:00 p.m. EST	Sule Unal, M.D., Hacettepe University, Ankara, Turkey	Poster
Other				
Understanding Health Literacy Among Patients with Thalassemia: Results from a Global Patient Survey by the Thalassemia Advocacy Advisory Council	6421	Monday, December 8, 2025, 6:00 - 8:00 p.m. EST	Sujit Sheth, M.D., Weill Cornell Medical College	Poster
Qualitative Interviews Exploring the Patient Experience of Fatigue in Individuals with Sickle Cell Disease (SCD), Thalassemia, and Pyruvate Kinase (PK) Deficiency	7971	N/A	Biree Andemariam, M.D., University of Connecticut Health	Publication
Activation of Pyruvate Kinases by Mitapivat Potentially Rescues Ineffective Erythropoiesis in Models of Diamond Blackfan Anemia	1121	Saturday, December 6, 2025, 5:30 - 7:30 p.m. EST	Jonathan de Wilde, M.D., Feinstein Institutes for Medical Research, Northwell Health	Poster

Please refer to the ASH 2025 website for full session details and data presentation listings, and visit the Agios booth (#1661) onsite.

About PYRUKYND® (mitapivat)
U.S. INDICATION
PYRUKYND is a pyruvate kinase activator indicated for the treatment of hemolytic anemia in adults with pyruvate kinase (PK) deficiency.

U.S. IMPORTANT SAFETY INFORMATION
Acute Hemolysis: Acute hemolysis with subsequent anemia has been observed following abrupt interruption or discontinuation of PYRUKYND in a dose-ranging study. Avoid abruptly discontinuing PYRUKYND. Gradually taper the dose of PYRUKYND to discontinue treatment if possible. When discontinuing treatment, monitor patients for signs of acute hemolysis and anemia including jaundice, scleral icterus, dark urine, dizziness, confusion, fatigue, or shortness of breath.

Hepatocellular Injury in Another Condition: In patients with another condition treated with PYRUKYND at a higher dose than that recommended for patients with PK deficiency, liver injury has been observed. These events were characterized by a time to onset within the first 6 months of treatment with peak elevations of alanine aminotransferase of >5× upper limit of normal (ULN) with or without jaundice. All patients discontinued treatment with PYRUKYND, and these events improved upon treatment discontinuation.

Obtain liver tests prior to the initiation of PYRUKYND and monthly thereafter for the first 6 months and as clinically indicated. Interrupt PYRUKYND if clinically significant increases in liver tests are observed or alanine aminotransferase is >5x ULN. Discontinue PYRUKYND if hepatic injury due to PYRUKYND is suspected.

Adverse Reactions: The most common adverse reactions including laboratory abnormalities (≥10%) in patients with PK deficiency were estrone decreased (males), increased urate, back pain, estradiol decreased (males), and arthralgia.

Drug Interactions:

- Strong CYP3A Inhibitors and Inducers: Avoid concomitant use.
- Moderate CYP3A Inhibitors: Do not titrate PYRUKYND beyond 20 mg twice daily.
- Moderate CYP3A Inducers: Consider alternatives that are not moderate inducers. If there are no alternatives, adjust PYRUKYND dosage.
- Sensitive CYP3A, CYP2B6, CYP2C Substrates Including Hormonal Contraceptives: Avoid concomitant use with substrates

that have narrow therapeutic index.
- UGT1A1 Substrates: Avoid concomitant use with substrates that have narrow therapeutic index.
- P-gp Substrates: Avoid concomitant use with substrates that have narrow therapeutic index.

Hepatic Impairment: Avoid use of PYRUKYND in patients with moderate and severe hepatic impairment.

Please see full Prescribing Information for PYRUKYND.

About Agios: Fueled by Connections to Transform Rare Diseases™
At Agios, our vision is to redefine the future of rare disease treatment. Fueled by connections, we build trusted partnerships with communities – collaborating to develop and deliver innovative medicines that have the potential to transform lives. With a foundation in hematology, we combine biological expertise with real-world insights to advance a growing pipeline of rare disease medicines that reflect the priorities of the people we serve. Agios is a commercial-stage biopharmaceutical company headquartered in Cambridge, Massachusetts. To learn more, visit www.agios.com and follow us on LinkedIn and X.

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); Agios' plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®; Agios' plans regarding future data presentations; and the potential benefits of Agios' 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 pandemics 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 establish and maintain key collaborations; uncertainty regarding any royalty payments related to the sale of its oncology business or any milestone or royalty payments related to its in-licensing of AG-236, and the uncertainty of the timing of any such payments; uncertainty of the results and effectiveness of the use of Agios' cash and cash equivalents; 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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