Agios' PYRUKYND® (mitapivat) Approved for Adults with Thalassemia in Saudi Arabia

August 4, 2025

- First regulatory approval for PYRUKYND in thalassemia, evaluated under SFDA's Breakthrough Medicines Program
- Agios partnered with NewBridge Pharmaceuticals, a regional specialty company focused on the Middle East and North Africa, in 2024 to manage PYRUKYND commercialization in the Gulf Region

CAMBRIDGE, Mass., Aug. 04, 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 the Saudi Food and Drug Authority (SFDA) has approved PYRUKYND® (mitapivat) for the treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia.

"The SFDA's decision marks a significant milestone, as Saudi Arabia becomes the first country to approve PYRUKYND for the treatment of adult patients with thalassemia, offering new hope to a community that has long faced debilitating, often life-threatening symptoms with limited or no therapeutic options," said Brian Goff, Chief Executive Officer, Agios. "Given the substantial burden and high estimated prevalence of thalassemia in Saudi Arabia, we are proud to partner with NewBridge Pharmaceuticals – a company specializing in delivering innovative treatments across the Middle East and North Africa – to help ensure PYRUKYND is accessible to these patients. We look forward to bringing PYRUKYND to more thalassemia patients globally and are actively preparing for potential launches in the U.S., United Arab Emirates, and Europe."

The SFDA approval of PYRUKYND in thalassemia is based on the results from the global, randomized, double-blind, placebo-controlled ENERGIZE and ENERGIZE-T Phase 3 trials in adults with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia, respectively. The New Drug Application (NDA) for PYRUKYND was accepted under the SFDA's Breakthrough Medicines Program, a program that aims to facilitate and accelerate the development and review of new medicines that address unmet medical need in the treatment of serious or life-threatening conditions.

In 2024, Agios entered into a distribution agreement with NewBridge Pharmaceuticals to advance regulatory filings and commercialization of PYRUKYND in the Gulf Cooperation Council (GCC) region, which includes Saudi Arabia, United Arab Emirates, Kuwait, Qatar, Oman, and Bahrain. NewBridge Pharmaceuticals was founded in 2010 to be a first-in-class commercialization platform for innovative therapeutics developed by global pharmaceutical companies, with a mission to bridge the access gap in the Middle East and North Africa.

"Thalassemia is a rare, lifelong genetic disorder that causes chronic anemia and can result in severe complications, including organ damage and cardiac disease," said Ali Taher, M.D., Ph.D., Professor of Medicine, Hematology & Oncology, and Director of Naef K. Basile Cancer Institute, American University of Beirut Medical Center in Beirut, Lebanon. "Until now, treatment options have been limited and often come with serious risks. The findings from the ENERGIZE and ENERGIZE-T trials support PYRUKYND as a disease-modifying, oral therapy for thalassemia – offering a much-needed new option to address the critical needs of this patient population."

"Historically, treatment options have been limited and are often specific to certain thalassemia subtypes or transfusion needs, leaving too many patients underserved," said Khaled Musallam, M.D., Ph.D., Burjeel Medical City, Abu Dhabi, United Arab Emirates. "With this approval, PYRUKYND is now indicated in Saudi Arabia for all adult patients with alpha- or beta-thalassemia, regardless of transfusion dependency status. This broad indication represents a truly meaningful advancement for the entire thalassemia community."

Regulatory applications for PYRUKYND in adult patients with thalassemia are under review by health authorities in the U.S., United Arab Emirates, and European Union. The Prescription Drug User Fee Act (PDUFA) goal date assigned by the U.S. Food and Drug Administration is September 7, 2025.

PYRUKYND is also approved for the treatment of hemolytic anemia in adults with pyruvate kinase (PK) deficiency in the U.S., and for the treatment of PK deficiency in adult patients in the European Union and Great Britain.

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' expectations for the FDA's review of its sNDA for PYRUKYND® in alpha-and-beta thalassemia and for the review of PYRUKYND by regulatory agencies in other countries; Agios' commercial expectations for PYRUKYND in Saudi Arabia and elsewhere; 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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