Agios' PYRUKYND® (mitapivat) Approved for Adults with Thalassemia in the United Arab Emirates

March 2, 2026

- PYRUKYND is the only medicine approved in the UAE for adults with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia
- NewBridge Pharmaceuticals, a regional specialty company, will continue to manage commercialization of PYRUKYND in the Gulf region

CAMBRIDGE, Mass., March 02, 2026 (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 Emirates Drug Establishment (EDE) of the United Arab Emirates (UAE) has approved PYRUKYND® (mitapivat), an oral pyruvate kinase (PK) activator, for the treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia. With this approval, PYRUKYND becomes the only medicine approved in the UAE for this patient population.

"For too long, thalassemia patients have endured debilitating and often life-threatening symptoms in the absence of significant therapeutic innovation," said Khaled Musallam, M.D., Ph.D., Burjeel Medical City, Abu Dhabi, UAE, and an investigator in the PYRUKYND thalassemia Phase 3 clinical program. "The robust Phase 3 results demonstrate that PYRUKYND can meet this urgent need by addressing the complex challenges of thalassemia. Given the high prevalence of the disease in the Gulf region, today's approval marks a critical milestone, offering thalassemia patients in the UAE – regardless of genotype or transfusion burden – a new treatment option to help manage their disease."

The EDE approval of PYRUKYND in thalassemia is based on 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.

"Agios is steadfast in our dedication to advancing innovative medicines for patients with rare blood disorders," said Brian Goff, Chief Executive Officer, Agios. "Today's milestone underscores this commitment while highlighting the transformative potential of PYRUKYND as a disease-modifying oral therapy for thalassemia. Together with our partner NewBridge Pharmaceuticals, we look forward to bringing this novel treatment option to patients in the UAE."

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), which includes Saudi Arabia, the UAE, Kuwait, Qatar, Oman, and Bahrain. NewBridge Pharmaceuticals, a first-in-class commercialization platform for innovative therapeutics in the Middle East and North Africa, is supporting the commercial launch of PYRUKYND for thalassemia in Saudi Arabia following its approval by the Saudi Food and Drug Authority (SFDA) in August 2025.

"This EDE decision represents a pivotal moment for thalassemia patients and their families in the UAE," said Androulla Eleftheriou, Ph.D., Executive Director, Thalassaemia International Federation. "For many years, this patient community has had limited treatment options to manage their disease. The approval of PYRUKYND marks an important turning point, expanding the treatment landscape and helping to address the daily needs of those living with thalassemia across the country."

PYRUKYND is also approved for adults with PK deficiency in the U.S. and Europe, and a marketing application for PYRUKYND in thalassemia is currently under review by the European Commission. In the U.S., mitapivat is approved for adults with thalassemia under the brand name AQVESME™ (mitapivat).

About Thalassemia
Thalassemia is a rare, inherited blood disease that affects the production of hemoglobin, the protein in red blood cells responsible for carrying oxygen throughout the body. The disease is categorized into two main types: alpha-thalassemia and beta-thalassemia, depending on which globin chain of the hemoglobin is affected. By disrupting hemoglobin production, thalassemia reduces the number of circulating red blood cells and shortens their lifespan, which leads to anemia, fatigue, and serious complications.

Some individuals with thalassemia require regular transfusions (classified as transfusion-dependent thalassemia), while others only need them intermittently (classified as non-transfusion-dependent thalassemia). All patients with thalassemia experience a significant disease burden, including comorbidities, reduced quality of life, and shortened life expectancy.

In the Gulf Cooperation Council (GCC), which includes Saudi Arabia, the United Arab Emirates, Kuwait, Qatar, Oman, and Bahrain, the prevalence of thalassemia is approximately 70,000 individuals.

About ENERGIZE and ENERGIZE-T
ENERGIZE (NCT04770753) and ENERGIZE-T (NCT04770779) are global, double-blind, placebo-controlled Phase 3 trials evaluating the efficacy and safety of mitapivat in adults with alpha- or beta-thalassemia.

The ENERGIZE trial randomized 194 non-transfusion-dependent alpha- or beta-thalassemia patients 2:1 to receive either mitapivat 100 mg twice daily or placebo. The primary endpoint was hemoglobin response, defined as an increase of ≥1.0 g/dL in average hemoglobin concentration from Week 12 through Week 24 compared with baseline. Key secondary endpoints included changes from baseline in average fatigue scores and in average hemoglobin concentration from Week 12 to Week 24. The trial also assessed safety and tolerability.

The ENERGIZE-T trial randomized 258 transfusion-dependent alpha- or beta-thalassemia patients 2:1 to receive either mitapivat 100 mg twice daily

or placebo. The primary endpoint was transfusion reduction response, defined as a ≥50% reduction in transfused red blood cell (RBC) units with a reduction of ≥2 units of RBCs transfused in any consecutive 12-week period through Week 48 compared with baseline. Several transfusion reduction measures were included as key secondary endpoints, and achievement of transfusion independence was a secondary endpoint. The trial also assessed safety and tolerability.

For each trial, patients who completed the double-blind phase had the option to transition into a corresponding open-label extension phase, during which all patients receive mitapivat.

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 mitapivat 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 >5x upper limit of normal (ULN) with or without jaundice. All patients discontinued treatment with mitapivat, 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 AQVESME™ (mitapivat)
U.S. INDICATION
AQVESME is indicated for the treatment of anemia in adults with alpha- or beta-thalassemia.

U.S. IMPORTANT SAFETY INFORMATION
BOXED WARNING: HEPATOCELLULAR INJURY

AQVESME can cause serious hepatocellular injury. Measure liver laboratory tests (ALT, AST, alkaline phosphatase and total bilirubin with fractionation) at baseline and every 4 weeks for 24 weeks and then as clinically indicated. Avoid use of AQVESME in patients with cirrhosis. Discontinue AQVESME if hepatic injury is suspected.
Because of the risk of hepatocellular injury, AQVESME is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called the AQVESME REMS.

WARNINGS AND PRECAUTIONS

Hepatocellular Injury
AQVESME can cause hepatocellular injury. Avoid use of AQVESME in patients with cirrhosis. In patients with thalassemia treated with AQVESME, liver injury with and without jaundice has been observed within the first 6 months of exposure. Obtain liver tests (including ALT, AST, alkaline phosphatase, total bilirubin with fractionation) prior to the initiation of AQVESME, then every 4 weeks for the first 24 weeks, and as clinically indicated thereafter. Interrupt AQVESME if clinically significant increases in liver tests are observed or alanine aminotransferase is >5 times the upper limit of normal (ULN). Complete a comprehensive evaluation to rule out other causes of liver injury when drug-induced liver injury is suspected. Discontinue AQVESME if hepatocellular injury due to AQVESME is suspected.

Symptoms and signs of early liver injury may mimic those of thalassemia. Advise patients to report new or worsening symptoms of loss of appetite, nausea, right-upper-quadrant abdominal pain, vomiting, scleral icterus, jaundice, or dark urine while on AQVESME treatment.

During the double-blind period, 2 of 301 patients (0.66%) with thalassemia treated with AQVESME experienced adverse reactions suggestive of

hepatocellular injury. Three additional patients experienced adverse reactions suggestive of hepatocellular injury during the open-label extension periods after switching from placebo to AQVESME. Of these 5 patients, 2 had serious liver injury requiring hospitalization, including 1 patient who developed jaundice (peak bilirubin 32 mg/dL). Another patient developed jaundice (peak bilirubin 4 mg/dL) without requiring hospitalization. These reactions were characterized by a time to onset within the first 6 months of treatment with peak elevations of alanine aminotransferase of >5×ULN with or without jaundice. All patients discontinued treatment with AQVESME, and these reactions improved upon treatment discontinuation.

AQVESME REMS
AQVESME is available only through a restricted program under a REMS called the AQVESME REMS because of the risk of hepatocellular injury.

ADVERSE REACTIONS
The most common adverse reactions among patients taking AQVESME were headache and insomnia.

DRUG INTERACTIONS

- Strong CYP3A Inhibitors and Inducers: Avoid concomitant use.
- Moderate CYP3A Inhibitors: Avoid concomitant use.
- Moderate CYP3A Inducers: Consider alternatives that are not moderate inducers. If there are no alternatives, see full Prescribing Information for recommended dosage for drug interactions with moderate CYP3A inducers.
- Sensitive CYP3A Substrates, including hormonal contraceptives: Avoid concomitant use with substrates that have narrow therapeutic index.
- CYP2B6, CYP2C, and UGT1A1 Substrates: Monitor patients for efficacy of the substrates with narrow therapeutic index.
- P-gp Substrates: Monitor patients for adverse reactions of the substrates with narrow therapeutic index.

HEPATIC IMPAIRMENT
Avoid use of AQVESME in patients with cirrhosis (Child-Pugh Class A, B, or C).

Please see full Prescribing Information for AQVESME, including Boxed Warning.

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 review of mitapivat by various regulatory agencies; Agios' commercial expectations for PYRUKYND in the UAE 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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