Agios' PYRUKYND® (mitapivat) Receives Positive CHMP Opinion for Adults with Thalassemia

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CAMBRIDGE, Mass., Oct. 17, 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 Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion for the new indication for PYRUKYND® (mitapivat), an oral pyruvate kinase (PK) activator, in adults for the treatment of anemia associated with transfusion-dependent and non-transfusion-dependent alpha- or beta-thalassemia.

The CHMP's opinion is based on the results from the global, randomized, double-blind, placebo-controlled ENERGIZE-T and ENERGIZE Phase 3 trials in adults with transfusion-dependent and non-transfusion-dependent alpha- or beta-thalassemia, respectively. The European Commission will now review the CHMP's opinion, with the final decision expected by early 2026.

"The CHMP's positive opinion marks an important step forward for the thalassemia community in Europe," said Sarah Gheuens, M.D., Ph.D., Chief Medical Officer and Head of R&D, Agios. "Thalassemia is a debilitating disease that places a profound burden on patients and families, with few, if any, therapeutic options currently available to help manage the condition. PYRUKYND represents a promising new medicine to address this urgent need, and we look forward to the potential of bringing it to patients across Europe."

In June 2025, Agios entered into an exclusive agreement with Avanzanite Bioscience B.V. (Avanzanite) for the commercialization and distribution of PYRUKYND across the European Economic Area, the United Kingdom, and Switzerland, and will support the European commercial launch of PYRUKYND in thalassemia, pending approval. Headquartered in Amsterdam, Avanzanite is a commercial-stage specialty pharmaceutical company dedicated to bringing rare disease medicines to patients across Europe.

PYRUKYND has received approval in Saudi Arabia for the treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia. Additional regulatory applications for PYRUKYND in thalassemia are under review by health authorities in the U.S., with a Prescription Drug User Fee Act (PDUFA) goal date of December 7, 2025, as well as in the United Arab Emirates.

PYRUKYND is also approved for the treatment of hemolytic anemia in adults with PK deficiency in the U.S. and for the treatment of PK deficiency in adult patients in Europe.

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.

An estimated 18,000 to 23,000 children and adults are living with thalassemia in the U.S. and five largest European countries (France, Germany, Italy, Spain, and the United Kingdom; EU5).

About ENERGIZE and ENERGIZE-T
ENERGIZE (NCT04770753) and ENERGIZE-T (NCT04770779) are global, randomized, 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 patients 2:1 to receive either mitapivat 100 mg twice daily or placebo. The primary endpoint was the proportion of patients achieving a hemoglobin response, defined as an increase of ≥1.0 g/dL in average hemoglobin concentrations from week 12 through week 24 compared with baseline. Key secondary endpoints included changes from baseline in Functional Assessment of Chronic Illness Therapy-Fatigue (FACIT-Fatigue) scores and in average hemoglobin concentration from week 12 to week 24. The study also assessed safety and tolerability.

The ENERGIZE-T trial randomized 258 transfusion-dependent patients 2:1 to receive either mitapivat 100 mg twice daily or placebo. The primary endpoint was the proportion of patients achieving a transfusion reduction response (TRR), defined as a ≥50% reduction in transfused red blood cell (RBC) units with a reduction of ≥2 units of transfused RBCs in any continuous 12-week period through week 48. Several additional transfusion reduction measures were included as key secondary endpoints, and achievement of transfusion independence was a secondary endpoint. The study also assessed safety and tolerability.

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 European Commission's review of the CHMP opinion, the review of marketing applications for PYRUKYND by regulatory agencies in other countries, including the United States; Agios' commercial expectations for PYRUKYND in Europe 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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