Agios Provides Update on Phase 2 Trial of Tebapivat in Sickle Cell Disease

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- Hemoglobin response rates for tebapivat, a PK activator, were consistent with the class of medicine, but Phase 2 results did not establish a differentiated profile to support continued development in sickle cell disease
- Safety and tolerability of tebapivat were consistent with prior sickle cell disease trials
- sNDA for mitapivat, company's foundational PK activator, is under FDA Priority Review in sickle cell disease; PDUFA goal date is November 1, 2026

CAMBRIDGE, Mass., July 21, 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 topline results from the Phase 2 trial of tebapivat, an oral pyruvate kinase (PK) activator, in patients aged 16 years or older with sickle cell disease.

The Phase 2 trial was designed to characterize the dose-response relationship of tebapivat in sickle cell disease and evaluate whether its profile was meaningfully differentiated relative to other PK activators. The trial included a 12-week, double-blind, placebo-controlled period in which 59 participants were randomized 2:2:2:1 to receive one of three once-daily (QD) tebapivat doses (2.5 mg, 5.0 mg, or 7.5 mg) or placebo.

Over the 12-week treatment period, improvements in hemoglobin levels and hemolysis were observed across all tebapivat dose levels, consistent with the established PK activation mechanism. The trial's primary endpoint of hemoglobin response (≥1.0 g/dL increase in average hemoglobin concentration from Weeks 10 through 12 compared with baseline) was achieved by 43.8% (n=7/16), 47.1% (n=8/17), and 29.4% (n=5/17) in the 2.5 mg QD, 5.0 mg QD, and 7.5 mg QD tebapivat arms, respectively, and 33.3% (n=3/9) in the placebo arm. The safety and tolerability of tebapivat in the Phase 2 trial were consistent with that observed in prior sickle cell disease trials. The trial did not demonstrate the level of differentiation required to support continued development; therefore, Agios has decided not to advance tebapivat in sickle cell disease.

"These Phase 2 data further reinforce PK activation as a clinically validated mechanism in sickle cell disease, with tebapivat demonstrating hematologic activity consistent with this class of medicine. However, the results did not establish the level of differentiation we believe is necessary to support continued development," said Sarah Gheuens, M.D., Ph.D., Chief Medical Officer and Head of R&D, Agios. "We remain focused on mitapivat, our foundational PK activator, which is under FDA Priority Review in sickle cell disease with an expected U.S. approval later this year. We look forward to bringing this first-in-class medicine to the sickle cell community and building on the extensive clinical experience generated to date as we work to address the significant unmet need in this debilitating disease."

Earlier this month, Agios announced that the U.S. Food and Drug Administration (FDA) accepted its supplemental New Drug Application (sNDA) for the accelerated approval of mitapivat, an oral PK activator, in sickle cell disease with a Priority Review. The Prescription Drug User Fee Act (PDUFA) goal date for this sNDA is November 1, 2026.

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.

Available Information about Agios
To achieve broad dissemination, Agios may disclose information to the public through a variety of disclosure channels including press releases, SEC filings, and public conference calls and webcasts. Some of the information distributed through these disclosure channels may be considered material information. Investors and others should note that Agios plans to use its website (www.agios.com) as a distribution channel to announce and give notice of Agios' upcoming events and presentations (including, but not limited to, presentations at medical or healthcare conferences). Such information, which may be deemed material, will be available on the Investors section of the company's website under the "Events & Presentations" tab. In addition, you may sign up to automatically receive email alerts about Agios' upcoming events and presentations ("Calendar Alerts") by visiting the "Email Alerts" option under the "IR Resources" tab of the Investors section of the company's website and submitting your email address.

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 tebapivat and mitapivat; Agios' expectations for the review of its sNDA for mitapivat by the FDA; 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 or cevidoplenib, 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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