Agios Provides Update on Phase 2b Trial of Tebapivat in Lower-Risk Myelodysplastic Syndromes

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- Results did not meet predefined threshold to support further development in LR-MDS
- Tebapivat was well tolerated, with no new safety signals observed

CAMBRIDGE, Mass., May 29, 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 it will not advance tebapivat, a next-generation oral pyruvate kinase (PK) activator, in lower-risk myelodysplastic syndromes (LR-MDS). This decision follows results from its Phase 2b trial that did not meet the company's predefined threshold to support further development in this indication.

The open-label, multicenter, 24-week dose-finding trial evaluated once-daily tebapivat at 10 mg, 15 mg, and 20 mg in 65 patients with LR-MDS and anemia, representing a heavily pretreated, heterogeneous population. The primary endpoint was transfusion independence, defined as eight consecutive weeks without a transfusion during the 24-week treatment period. While tebapivat demonstrated evidence of biological activity, clinical benefit was not observed in a sufficient proportion of patients or subgroup of patients to meet the company's predefined threshold for advancement in LR-MDS. Tebapivat was well tolerated across all dose levels, with no new safety signals identified.

"The results from the Phase 2b trial underscore the biological complexity of lower-risk myelodysplastic syndromes and the challenges of identifying patients most likely to benefit. On behalf of the entire Agios team, I want to extend our sincere gratitude to the patients, caregivers, investigators, and broader community who made this research possible," said Sarah Gheuens, M.D., Ph.D., Chief Medical Officer and Head of R&D, Agios. "PK activation remains a clinically validated mechanism, and we continue to see significant potential for tebapivat as a next-generation medicine in sickle cell disease. We look forward to sharing topline data from this Phase 2 trial in the second half of 2026."

About Tebapivat
Tebapivat is a next-generation oral pyruvate kinase (PK) activator designed to provide optimized clinical benefits for patients with rare hematologic diseases. It is structurally differentiated by its potent dual activation of the PKR and PKM2 isoforms (or variants) of the PK enzyme, which are expressed in red blood cells. Clinical pharmacology data supports once-daily dosing of tebapivat, without the need for a dose taper. Tebapivat is currently being evaluated in a Phase 2 trial for the treatment of sickle cell disease, with topline data anticipated in the second half of 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 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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