Agios Announces FDA Orphan Drug Designation Granted to Tebapivat (AG-946) for Treatment of Myelodysplastic Syndromes (MDS)

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CAMBRIDGE, Mass., Sept. 11, 2024 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a leader in cellular metabolism and PK activation pioneering therapies for rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to the company's novel pyruvate kinase (PK) activator tebapivat (AG-946) for the treatment of myelodysplastic syndromes (MDS).

"Receiving orphan drug designation for tebapivat in MDS underscores the importance of bringing new oral treatment options to patients suffering from this rare disease," said Sarah Gheuens, M.D., Ph.D., chief medical officer and head R&D at Agios. "We aim to deliver the first oral therapy that addresses anemia due to ineffective erythropoiesis in lower-risk MDS, which affects approximately 75,000-80,000 patients in the U.S. and EU5 and accounts for approximately 70% of MDS cases."

Agios completed a Phase 2a study of tebapivat in lower-risk MDS late last year and is currently initiating a Phase 2b study of tebapivat in lower-risk MDS.

The FDA's Office of Orphan Drug Products grants orphan drug designation to support the development of medicines for rare disorders that affect fewer than 200,000 people in the U.S. Under the Orphan Drug Act, orphan drug designation qualifies a company for incentives, including tax credits, exemptions from certain FDA fees for clinical trials, and the potential for seven years of market exclusivity following drug approval.

Mitapivat, the company's lead PK activator, was previously granted FDA orphan drug designation for the treatment of PK deficiency, thalassemia, and sickle cell disease.

Tebapivat is not approved for use by any regulatory authority.

About Agios
Agios is the pioneering leader in PK activation and is dedicated to developing and delivering transformative therapies for patients living with rare diseases. In the U.S., Agios markets a first-in-class pyruvate kinase (PK) activator for adults with PK deficiency, the first disease-modifying therapy for this rare, lifelong, debilitating hemolytic anemia. Building on the company's deep scientific expertise in classical hematology and leadership in the field of cellular metabolism and rare hematologic diseases, Agios is advancing a robust clinical pipeline of investigational medicines with programs in alpha- and beta-thalassemia, sickle cell disease, pediatric PK deficiency, MDS-associated anemia and phenylketonuria (PKU). In addition to its clinical pipeline, Agios is advancing a preclinical TMPRSS6 siRNA as a potential treatment for polycythemia vera. For more information, please visit the company's website at www.agios.com.

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), tebapivat (AG-946), TMPRSS6 siRNA and AG-181, Agios' PAH stabilizer; Agios' plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®, tebapivat and AG-181; Agios' use of proceeds from the transaction with Royalty Pharma; potential U.S. net sales of vorasidenib and potential future royalty payments; Agios' strategic vision and goals, including its key milestones for 2024; 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 milestone or royalty payments related to the sale of its oncology business or its in-licensing of TMPRSS6 siRNA, 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.

Contacts:

Investor Contact
Chris Taylor, VP, Investor Relations and Corporate Communications

Agios Pharmaceuticals
IR@agios.com

Media Contact
Dan Budwick
1AB
dan@1abmedia.com

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