Agios Announces FDA Acceptance and Priority Review of New Drug Application for Mitapivat for Treatment of Adults with Pyruvate Kinase Deficiency

August 17, 2021

-- PDUFA Date Set for February 17, 2022 --

CAMBRIDGE, Mass., Aug. 17, 2021 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism developing and delivering innovative treatments for genetically defined diseases, today announced that the U.S. Food and Drug Administration (FDA) has accepted the company's New Drug Application (NDA) for mitapivat for the treatment of adults with pyruvate kinase (PK) deficiency. The NDA was granted a Priority Review designation and has been given a Prescription Drug User Fee Act (PDUFA) action date of February 17, 2022, accelerating the review time from 10 months to six months from the day of filing acceptance. The FDA's Priority Review designation is given to investigational medicines that treat a serious condition and offer significant improvements in safety or effectiveness.

"The acceptance of our NDA for mitapivat with priority review represents an important milestone on the path to expeditiously deliver the first potentially disease-modifying therapy for people with PK deficiency, a chronic, lifelong hemolytic anemia characterized by serious complications affecting multiple organs," said Sarah Gheuens, M.D., Ph.D., senior vice president of clinical development and incoming chief medical officer at Agios. "We look forward to working with the FDA during the review process and will continue to execute on our global strategy to ensure we are well positioned to rapidly deliver mitapivat to patients and healthcare providers upon approval."

Agios also submitted a marketing authorization application (MAA) to the European Medicines Agency (EMA) in June 2021 for mitapivat as a potential treatment for adults with PK deficiency. As announced on the company's second quarter 2021 earnings call, the MAA passed validation which triggered the start of the MAA review procedure.

The NDA and MAA submissions are based on results from two pivotal studies, ACTIVATE and ACTIVATE-T, conducted in not regularly transfused and regularly transfused adults with PK deficiency, respectively. A full analysis of these data – including patient-reported outcomes (PRO) – was recently presented at the European Hematology Association (EHA) Virtual Congress. An extension study for adults with PK deficiency previously enrolled in ACTIVATE or ACTIVATE-T is ongoing and designed to evaluate the long-term safety, tolerability and efficacy of treatment with mitapivat.

Mitapivat is not currently approved for use in any country.

About PK Deficiency
Pyruvate kinase (PK) deficiency is a rare, inherited disease that presents as chronic hemolytic anemia, which is the accelerated destruction of red blood cells. The inherited mutations in PKR genes cause a deficit in energy within the red blood cell, as evidenced by lower PK enzyme activity, a decline in adenosine triphosphate (ATP) levels and a build-up of upstream metabolites, including 2,3-DPG (2,3-diphosphoglycerate).

PK deficiency is associated with serious complications, including gallstones, pulmonary hypertension, extramedullary hematopoiesis, osteoporosis and iron overload and its sequelae, which can occur regardless of the degree of anemia or transfusion burden. PK deficiency can also cause quality of life problems, including challenges with work and school activities, social life and emotional health. Current management strategies for PK deficiency, including red blood cell transfusions and splenectomy, are associated with both short- and long-term risks. There are no currently approved therapies for PK deficiency. For more information, please visit www.knowpkdeficiency.com.

Agios, in partnership with PerkinElmer Genomics, launched the Anemia ID program to offer no-cost genetic testing to eligible patients in the U.S with suspected hereditary anemias, including PK deficiency. The program was created in response to feedback from patients, advocates and physicians about the need for improved diagnosis to inform disease management decisions. To learn more, please visit www.AnemiaID.com.

Agios also launched the myAgios® patient support services program for people living with pyruvate kinase (PK) deficiency and their caregivers. After enrolling in the program, patients and caregivers are connected with a dedicated Patient Support Manager (PSM) with a clinical background to provide tailored support, educational resources and opportunities to connect with other patients and caregivers in the community. To learn more or enroll, please visit www.myagios.com.

About Agios
Agios is focused on discovering and developing novel investigational medicines to treat genetically defined diseases through scientific leadership in the field of cellular metabolism. The company's most advanced drug candidate is a first-in-class pyruvate kinase R (PKR) activator, mitapivat, that is currently being evaluated for the treatment of three distinct hemolytic anemias. In addition to its active late-stage clinical pipeline, Agios has multiple novel, investigational therapies in clinical and preclinical development. 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 Agios' expectations for the FDA's review of its NDA for mitapivat. The words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" 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, the FDA's acceptance of Agios's NDA for mitapivat does not represent evaluation of the efficacy and safety of mitapivat, and is not a guarantee of approval. 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: risks associated with the regulatory review process generally; the risk that the FDA may determine that the data included in the NDA are insufficient for approval and that the Company must conduct additional clinical trials, or nonclinical or other studies, before mitapivat can be approved; the risk that the results of previously conducted studies involving mitapivat will not be repeated or observed in ongoing or future studies or following commercial launch, if mitapivat is approved; and risks associated with the Company's dependence on third parties with respect to regulatory

matters for mitapivat. 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, or SEC. While the list of factors presented here is considered representative, this list should not be considered to be a complete statement of all potential risks and uncertainties. Any forward-looking statements contained in this communication are made only as of the date hereof, and we undertake no obligation to update forward-looking statements to reflect developments or information obtained after the date hereof and disclaim any obligation to do so other than as may be required by law.

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