Agios Submits Marketing Authorisation Application to European Medicines Agency for Mitapivat for Treatment of Adults with Pyruvate Kinase Deficiency

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CAMBRIDGE, Mass., June 28, 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 it has submitted a Marketing Authorisation Application (MAA) for mitapivat to the European Medicines Agency (EMA) for the treatment of adults with pyruvate kinase (PK) deficiency in the European Union. This submission follows the company's recent New Drug Application (NDA) submission of mitapivat to the U.S. Food and Drug Administration (FDA) for treatment of adults with PK deficiency in the U.S.

"With both our NDA and MAA filings, we are poised to 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 Chris Bowden, M.D., chief medical officer at Agios. "There are no approved therapies for PK deficiency, and the current management strategies of blood transfusions and splenectomy are associated with both short- and long-term risks, including iron overload, blood clots and increased risk for infections. PK deficiency can lead to chronic fatigue, hemolytic crisis, gallstones, splenomegaly, liver cirrhosis, pulmonary hypertension and osteoporosis, and the burden of disease can take a toll on patients' ability to navigate work and other day-to-day activities, as well as on their mental health. We are looking forward to working with both the FDA and EMA to provide a potential new treatment option for this community that may help address the significant unmet needs of PK deficiency patients."

Like the NDA, the MAA submission is 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 approved for use by any regulatory authority.

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.

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 the potential benefits of Agios' products and product candidates; Agios' plans for future regulatory submissions; 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. 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 the COVID-19 pandemic 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 future approved products, and launching, marketing and selling 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 and 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 collaborations; 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, or SEC, including the risks and uncertainties set forth under the heading Risk Factors in our filings with the 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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