Agios Receives Positive Opinion on Orphan Drug Designation from the European Medicines Agency for Mitapivat in Pyruvate Kinase Deficiency

March 30, 2020

CAMBRIDGE, Mass., March 30, 2020 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, today announced that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products issued a positive opinion on the company's application for orphan drug designation for its investigational medicine mitapivat as a potential treatment for pyruvate kinase (PK) deficiency, a rare, debilitating, hemolytic anemia. Mitapivat was previously granted orphan drug designation by the United States Food and Drug Administration. Mitapivat is an investigational, first-in-class, oral, small molecule allosteric activator of wild-type and a variety of mutated pyruvate kinase-R (PKR) enzymes that directly targets the underlying metabolic defect in PK deficiency.

"We are pleased to receive a positive opinion from EMA for orphan drug designation for mitapivat as a recognition of the need for innovative treatments for patients with PK deficiency, a serious lifelong disease characterized by hemolytic anemia that can lead to severe symptoms including iron overload, splenomegaly and heart failure," said Chris Bowden, M.D., chief medical officer at Agios. "Both of our pivotal trials are fully enrolled, and we look forward to the completion of these trials intended to support global regulatory filings. Mitapivat is the first and only PKR activator to receive this important designation in Europe and the first potential disease-modifying treatment for PK deficiency patients."

Orphan drug designation in the European Union (EU) is granted by the European Commission based on a positive opinion issued by the EMA Committee for Orphan Medicinal Products. To qualify, an investigational medicine must be intended to treat a seriously debilitating or life-threatening condition that affects fewer than five in 10,000 people in the EU, and there must be sufficient non-clinical or clinical data to suggest the investigational medicine may produce clinically relevant outcomes. EMA orphan drug designation provides companies with certain benefits and incentives, including clinical protocol assistance, differentiated evaluation procedures for Health Technology Assessments in certain countries, access to a centralized marketing authorization procedure valid in all EU member states, reduced regulatory fees and 10 years of market exclusivity.

Mitapivat Clinical Development
Agios has two ongoing global, pivotal trials in adults with PK deficiency that are fully enrolled. Learn more at activatetrials.com.

- ACTIVATE: A placebo-controlled trial with a 1:1 randomization evaluating patients who do not receive regular transfusions. The primary endpoint of the trial is the proportion of patients who achieve a sustained hemoglobin increase of ≥1.5 g/dL.
- ACTIVATE-T: A single arm trial of regularly transfused patients with a primary endpoint of reduction in transfusion burden over six months compared to individual historical transfusion burden over prior 12 months.

In addition, Agios is conducting a Phase 2 study evaluating the efficacy, safety, pharmacokinetics and pharmacodynamics of treatment with mitapivat in adults with non-transfusion-dependent β- and α-thalassemia (NTDT). The trial is fully enrolled, and the primary endpoint is hemoglobin response. Preliminary Phase 2 data establishing proof-of-concept for mitapivat in thalassemia were disclosed at the end of 2019. Mitapivat is also being studied in sickle cell disease under a Cooperative Research and Development Agreement (CRADA) with the U.S. National Institutes of Health.

Mitapivat is not approved for use by any regulatory authority.

About Agios
Agios is focused on discovering and developing novel investigational medicines to treat malignant hematology, solid tumors and rare genetic diseases through scientific leadership in the field of cellular metabolism. In addition to an active research and discovery pipeline across these three therapeutic areas, Agios has two approved oncology precision medicines and multiple first-in-class investigational therapies in clinical and/or 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 mitapivat; Agios' plans regarding future data presentations; and the benefit of Agios' strategic plans and focus. The words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook," "goal", "potential" 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, a positive opinion on Agios' application for orphan drug designation for mitapivat 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: the results of Agios' 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 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 and conduct its current and future 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 maintain key collaborations; and general economic, market and global health 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

Investors:

Holly Manning, 617-844-6630
Director, Investor Relations
Holly.Manning@agios.com

Media:
Jessica Rennekamp, 857-209-3286
Associate Director, Corporate Communications
Jessica.Rennekamp@agios.com

Source: Agios Pharmaceuticals, Inc.