Agios Announces FDA Orphan Drug Designation Granted to Mitapivat for Treatment of Thalassemia

June 8, 2020

CAMBRIDGE, Mass., June 08, 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 U.S. Food and Drug Administration (FDA) has granted orphan drug designation to the company's first-in-class pyruvate kinase-R (PKR) activator mitapivat for the treatment of patients with thalassemia. Mitapivat is an investigational, oral, small molecule allosteric activator of wild-type and a variety of mutated PKR enzymes.

"Receiving orphan drug designation is an important milestone as we continue to advance mitapivat for patients with thalassemia, a serious hemolytic anemia with limited treatment options," said Chris Bowden, M.D., chief medical officer at Agios. "We look forward to presenting updated data from our Phase 2 study of mitapivat in both alpha- and beta-thalassemia patients at the virtual European Hematology Association Annual Congress later this week."

The FDA's Office of Orphan Drug Products grants orphan status to support the development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the U.S. Orphan drug designation provides certain benefits, including market exclusivity upon regulatory approval if received, exemption of FDA application fees and tax credits for qualified clinical trials.

Mitapivat was previously granted orphan drug designation by the FDA and the European Medicines Agency for pyruvate kinase (PK) deficiency, a rare, debilitating, hemolytic anemia.

Mitapivat Clinical Development
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, and updated data from this trial will be presented at the 25th European Hematology Association (EHA) Annual Congress, which is being held virtually on June 11-14, 2020.

In addition, Agios has two ongoing global, pivotal trials in adults with PK deficiency that are fully enrolled.

- 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.

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: 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 current or future approved products, and launching, marketing and selling current or future approved products; 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.