



AgiOS Recognizes 14th Annual Rare Disease Day and Raises Awareness of Unmet Needs in Rare Hemolytic Anemias

February 26, 2021

– In Partnership with the Sickle Cell Community Consortium, Agios Works to Address Educational Resource Gaps Caused by COVID-19 for Students with Sickle Cell Disease –

– Company to Host Facebook Live Discussion with Pyruvate Kinase (PK) Deficiency Patient, Caregiver and Key Opinion Leader Today at 2 p.m. ET –

CAMBRIDGE, Mass., Feb. 26, 2021 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ: AGIO), a leader in the field of cellular metabolism to treat cancer and genetically defined diseases, today announced its support for the 14th annual Rare Disease Day, a global awareness campaign for the more than 300 million people living with a rare disease worldwide.

"Patients are central to everything we do at Agios, and we are proud to join with patients, advocates, caregivers, clinicians and industry colleagues around the world to support Rare Disease Day," said Jackie Fouse, Ph.D., chief executive officer at Agios. "We strive to work closely with patient communities, understanding their perspectives and cultivating true partnerships. In honor of Rare Disease Day, we are collaborating with the PK deficiency, thalassemia and sickle cell disease communities to advance important initiatives designed to raise awareness and address needs faced by people living with these hemolytic anemias."

Throughout the month of February, the Agios team raised funds for the [Sickle Cell Community Consortium \(SCCC\) COVID-19 relief fund](#). Following a series of sickle cell disease community meetings and surveys organized by the SCCC, patients and caregivers identified areas where they most need support as a result of the COVID-19 pandemic. The community identified a critical need for better educational resources for students with sickle cell disease who are learning in remote or hybrid environments. The funds raised by Agios help address this gap by providing tablets, printers, online tools, access to tutors and other resources for these students.

"We are living through the first global pandemic in any of our lifetimes, and every aspect of life has changed, including the way students are expected to learn. In this new environment, many students impacted by sickle cell disease are falling behind from both health concerns and insufficient access to resources and support. Without equalizing resources and support, the education system is no longer grading children's ability to learn, but instead, is judging their access to resources and grading their privilege. In the 'land of plenty,' this is not acceptable," said Lakiea Bailey, Ph.D., executive director of the SCCC. "Our children are intelligent, hardworking, talented and capable of great educational achievement. We are grateful to the Agios team for their support to ensure that children and teens living with sickle cell disease have equal access to the resources they need to achieve their educational goals."

In recognition of Rare Disease Day, Agios is spearheading the following additional initiatives:

- Today at 2 p.m. ET, Agios is hosting a Facebook Live discussion on its ["Know PK Deficiency" Facebook page](#). The event will feature Jenny M. Despotovic, D.O., hematology center director at Texas Children's Hospital and associate professor of hematology/oncology at Baylor College of Medicine, as well as 16-year old PK deficiency patient Jonathan Watson and his mother Alejandra, discussing the impact of PK deficiency on daily life and how patients, caregivers and healthcare professionals can work together in partnership to best manage the disease.
- Agios sponsored [MassBio's 2021 Rare Disease Day](#) virtual event, which will focus on strategies to better address health inequity and racial disparities among the rare disease community, including improving representation in clinical trials. The event takes place today at 10 a.m. ET.
- Agios organized an internal, virtual speaker series throughout February to increase the team's understanding of the experiences of patients living with hemolytic anemias. Speakers included:
 - Ahmar Zaidi, M.D., hematologist at Children's Hospital of Michigan and sickle cell disease advocate, presented on addressing health inequities in the treatment of sickle cell disease.
 - Shamonica Wiggins, sickle cell disease patient and advocate, and her fiancé shared their personal perspectives on managing sickle cell disease and the impact it can have on relationships. Biree Andemariam, M.D., board member and former chief medical officer of the Sickle Cell Disease Association of America (SCDAA) and associate professor of medicine at the University of Connecticut Health, joined them to provide further insights into clinical management of the disease.
 - Sujit Sheth, M.D., pediatric hematologist-oncologist at Weill Cornell Medicine with a specialty in thalassemia, and one of his thalassemia patients shared their perspectives on disease management and the patient/doctor partnership.

Agios is advancing clinical programs in three serious hemolytic anemias: PK deficiency, thalassemia and sickle cell disease. In late 2020, Agios [announced](#) that it intends to move forward with a singular focus on accelerating and expanding its genetically defined disease portfolio, including these clinical programs and a robust pipeline of therapeutic candidates.

About Agios

Agios is focused on discovering and developing novel investigational medicines to treat malignant hematology, solid tumors and genetically defined 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 and Agios' strategic plans and prospects. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," "could," "potential," "possible," "hope" 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 or its collaborators 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 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; 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 maintain key 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. 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.

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