Agios Unveils 2023-2026 Value-driving Catalysts Enabling Company's Vision to Transform Patient Outcomes in Rare Diseases

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– Consistent and compelling clinical data across rare hematological diseases support five ongoing pivotal trials in thalassemia, sickle cell disease and pediatric pyruvate kinase (PK) deficiency –

– Driving toward two additional PYRUKYND® indications, with potential FDA approvals in thalassemia in 2025 and sickle cell disease in 2026 –

– Strong cash position expected to enable completion of ongoing programs and pipeline expansion to cash-flow positivity –

CAMBRIDGE, Mass., Jan. 08, 2023 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a leader in the field of cellular metabolism pioneering therapies for rare diseases, today announced its anticipated 2023 milestones and significant value-driving catalysts through 2026 that support the company's mission to transform patient outcomes in rare diseases. Agios will present at the 41st Annual J.P. Morgan Healthcare Conference on Wednesday, January 11, 2023, at 7:30 a.m. PT, and a live webcast will be available at investor.agios.com.

"Agios is poised for significant growth with the potential for approvals in two additional PYRUKYND ® indications by 2026, and is well capitalized to advance its robust existing clinical pipeline and expand its portfolio within our core areas of expertise," said Brian Goff, chief executive officer at Agios. "As the pioneering leader in PK activation with more than seven years of clinical experience with PYRUKYND ®, we have generated an impressive body of consistent and compelling data across rare hematological diseases with shared underlying pathophysiology that further builds confidence in our five ongoing pivotal clinical trials in thalassemia, sickle cell disease and pediatric PK deficiency. Furthermore, we are executing our first rare disease U.S. product launch with PYRUKYND® in adult PK deficiency, providing the first disease-modifying therapy for this patient community that previously had no treatment options and building the capabilities to set us up for success with our expected launches in meaningfully larger patient populations."

Recent Highlights

- *Adult PK Deficiency:* Received marketing authorization for PYRUKYND® in adults with PK deficiency in the EU and Great Britain
- *Thalassemia:* Enrolled approximately half of patients in the Phase 3 ENERGIZE and ENERGIZE-T studies of PYRUKYND® in not regularly transfused and regularly transfused adults with thalassemia, respectively
- *Sickle Cell Disease:* Closed screening in the Phase 2 portion of the RISE UP study of PYRUKYND® in adults with sickle cell disease in December and expect to complete enrollment in January
- *Data Presentations:* Presented broad set of clinical and translational data at the 64th American Society of Hematology (ASH) Annual Meeting & Exposition, including long-term PYRUKYND® data in adults with non-transfusion-dependent thalassemia and in adults with PK deficiency
- *Leadership:* Appointed Tsveta Milanova to the role of chief commercial officer, bringing two decades of experience in rare disease commercial strategy and global market access

Anticipated 2023 Milestones

- *Thalassemia:* Complete enrollment of the Phase 3 ENERGIZE and ENERGIZE-T studies of PYRUKYND® by mid-year
- *Pediatric PK Deficiency:* Enroll at least half of patients in the Phase 3 ACTIVATE-kids and ACTIVATE-kidsT studies of PYRUKYND® by year-end
- *Sickle Cell Disease:* Announce data readout from Phase 2 portion of RISE UP study of PYRUKUND® and go/no-go to Phase 3 decision by mid-year
- *Lower-risk Myelodysplastic Syndromes (LR-MDS):* Complete enrollment of Phase 2a study of novel PK activator AG-946 by year-end
- *Earlier-stage Pipeline:* File investigational new drug (IND) application for PAH stabilizer for the treatment of phenylketonuria (PKU) by year-end

Agios 2026 Vision

By 2026, Agios' vision is to establish a classical hematology franchise with PYRUKYND ® approvals across PK deficiency, thalassemia and sickle cell disease; expand its portfolio by advancing AG-946 and the preclinical pipeline as well as through disciplined business development aligned with the company's core therapeutic focus areas and capabilities; and achieve cash-flow positivity. Agios provided a roadmap of additional significant potential catalysts between 2024 and 2026 to enable the realization of this vision, as follows:

2024

- Data readout from Phase 3 ENERGIZE study of PYRUKYND® in adults with non-transfusion-dependent thalassemia (first

half of 2024)
- Data readout from Phase 3 ENERGIZE-T study of PYRUKYND® in adults with transfusion-dependent thalassemia (second half of 2024)
- Data readout from Phase 2a study of AG-946 in LR-MDS

2025

- Potential FDA approval for PYRUKYND® in thalassemia
- Data readout from Phase 3 portion of RISE UP study of PYRUKYND® in sickle cell disease, pending go/no-go decision in 2023
- Data readouts from Phase 3 ACTIVATE-kids and ACTIVATE-kidsT studies of PYRUKYND® in pediatric PK deficiency

2026

- Potential FDA approval for PYRUKYND® in sickle cell disease
- Potential FDA approval for PYRUKYND® in pediatric PK deficiency
- Achieve cash-flow positivity

Presentation at 41st Annual J.P. Morgan Healthcare Conference
Agios will webcast its corporate presentation from the 41st Annual J.P. Morgan Healthcare Conference on Wednesday, January 11 at 7:30 a.m. PT. A live webcast of the presentation can be accessed under “Events & Presentations” in the Investors section of the company’s website at agios.com. A replay of the webcast will be archived on the Agios website for at least two weeks following the presentation.

About Agios
Agios is a biopharmaceutical company that is fueled by connections. The Agios team cultivates strong bonds with patient communities, healthcare professionals, partners and colleagues to discover, develop and deliver therapies for rare diseases. In the U.S., Agios markets a first-in-class pyruvate kinase (PK) activator for adults with PK deficiency, the first disease-modifying therapy for this rare, lifelong, debilitating hemolytic anemia. Building on the company's leadership in the field of cellular metabolism, Agios is advancing a robust clinical pipeline of investigational medicines with programs in alpha- and beta-thalassemia, sickle cell disease, pediatric PK deficiency and MDS-associated anemia. In addition to its clinical pipeline, Agios has multiple investigational therapies in preclinical development and deep scientific expertise in classical hematology. 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 PYRUKYND® (mitapivat), AG-946 and its PAH stabilizer; Agios’ plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®, AG-946 and its PAH stabilizer; Agios’ strategic vision and goals, including its key milestones for 2023 and potential catalysts through 2026; 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. For example, there can be no guarantee that any product candidate Agios 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; the failure of Agios to receive milestone or royalty payments related to the sale of its oncology business, the uncertainty of the timing of any receipt of any such payments, and the uncertainty of the results and effectiveness of the use of proceeds from the transaction with Servier; 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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