Agios Announces Key Anticipated 2024 Milestones Across Rare Disease Portfolio

January 8, 2024

– Industry-leading PK Activator Franchise Has Demonstrated Clinical Efficacy in Four Hematological Diseases, Including New Positive Phase 3 Data in Non-Transfusion-Dependent Thalassemia –

– Company Expects Four Additional Phase 3 Readouts by the End of 2025, with Potential FDA Approvals in Thalassemia in 2025 and Sickle Cell Disease in 2026 –

– Strong Cash Position Expected to Support Completion of Ongoing Programs and Disciplined Pipeline Expansion at Least into 2026 –

CAMBRIDGE, Mass., Jan. 08, 2024 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a leader in the field of cellular metabolism pioneering therapies for rare diseases, today announced its anticipated 2024 milestones and value-driving catalysts through 2026 that support the company's mission to transform patient outcomes in rare diseases. Agios will present at the 42nd Annual J.P. Morgan Healthcare Conference on Wednesday, January 10, 2024, at 7:30 a.m. PT, and a live webcast will be available at investor.agios.com.

"We were pleased to announce positive topline data from the Phase 3 study of our lead PK activator, mitapivat, in non-transfusion-dependent alpha- or beta-thalassemia last week, a segment of the population with no currently approved therapeutic options in the U.S. We look forward to data readouts from four additional Phase 3 studies across our industry-leading PK activator franchise by the end of 2025," said Brian Goff, chief executive officer at Agios. "This robust series of near-term catalysts positions Agios for potential launches of a first- and best-in-class therapy in thalassemia in 2025 and in sickle cell disease in 2026, and we look forward to maximizing the commercial opportunities ahead of us. Supported by our strong cash position, Agios is poised for significant progress in the next 12-24 months, and we look forward to the opportunity to deliver a novel oral treatment option for two additional hematologic diseases with high unmet need."

2023 Highlights

- *Thalassemia:* Completed enrollment in the Phase 3 ENERGIZE and ENERGIZE-T studies of mitapivat in non-transfusion-dependent and transfusion-dependent thalassemia, respectively
- *Sickle Cell Disease:* Announced positive data from the Phase 2 portion of the RISE UP study of mitapivat and dosed the first patients in the Phase 3 portion
- *Pediatric PK Deficiency:* Completed enrollment in the Phase 3 ACTIVATE kids-T study of mitapivat in children with PK deficiency who are regularly transfused. Enrolled more than half of patients in the Phase 3 ACTIVATE-kids study of mitapivat in children with pediatric PK deficiency who are not regularly transfused
- *Lower-risk Myelodysplastic Syndromes (LR-MDS):* Announced clinical proof-of-concept in Phase 2a study of AG-946, supporting continued development in Phase 2b
- *Earlier-stage Pipeline:* Filed an Investigational New Drug Application (IND) for PAH stabilizer for the treatment of phenylketonuria (PKU)
- *Business Development:* Announced exclusive worldwide license agreement with Alnylam for novel siRNA targeting TMPRSS6 for the potential treatment of polycythemia vera (PV)
- *Data Presentations:* Presented broad set of clinical and translational data at the 65th American Society of Hematology (ASH) Annual Meeting & Exposition, including positive data from the Phase 2 portion of the RISE UP study of mitapivat in sickle cell disease

Anticipated 2024 Milestones

- *Thalassemia:* Following the announcement of topline data from the Phase 3 ENERGIZE study last week, Agios plans to report topline data from the Phase 3 ENERGIZE-T study of mitapivat in transfusion-dependent thalassemia (mid-year) and submit a New Drug Application (NDA) for mitapivat in thalassemia (year-end)
- *Sickle Cell Disease:* Complete enrollment in the Phase 3 portion of the RISE UP study of mitapivat (year-end)
- *Pediatric PK Deficiency:* Complete enrollment in the Phase 3 ACTIVATE-kids study of mitapivat (mid-2024). Report topline data from Phase 3 ACTIVATE kids-T study (year-end)
- *Lower-risk Myelodysplastic Syndromes (LR-MDS):* Dose first patient in Phase 2b study of AG-946 (mid-year)
- *Earlier-stage Pipeline:* Dose the first patient in the Phase 1 study of PAH stabilizer for the treatment of PKU (H1 2024)

Four Additional Phase 3 Readouts and Two Potential New Indication Approvals Expected by End of 2026

2024

- Data readout from Phase 3 ENERGIZE study of mitapivat in non-transfusion-dependent thalassemia (announced January 3, 2024)
- Data readout from Phase 3 ENERGIZE-T study of mitapivat in transfusion-dependent thalassemia (mid-year)
- Data readout from Phase 3 ACTIVATE kids-T study of mitapivat in pediatric PK deficiency (year-end)

2025

- Data readout from Phase 3 portion of the RISE UP study of mitapivat in sickle cell disease
- Data readout from Phase 3 ACTIVATE kids study of mitapivat in pediatric PK deficiency
- Potential FDA approval for mitapivat in thalassemia

2026

- Potential FDA approval for mitapivat in sickle cell disease
- Potential FDA approval for mitapivat in pediatric PK deficiency

Presentation at 42nd Annual J.P. Morgan Healthcare Conference

Agios will webcast its corporate presentation from the 42nd Annual J.P. Morgan Healthcare Conference on Wednesday, January 10 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 the pioneering leader in PK activation and is dedicated to developing and delivering transformative therapies for patients living with 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 deep scientific expertise in classical hematology and leadership in the field of cellular metabolism and rare hematologic diseases, 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 is advancing a preclinical TMPRSS6 siRNA as a potential treatment for polycythemia vera (PV), and a preclinical PAH stabilizer as a potential treatment for phenylketonuria (PKU). 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, Agios’s PAH stabilizer and its novel siRNA Targeting TMPRSS6; Agios’ plans, strategies and expectations for its preclinical, clinical and commercial advancement of its drug development, including PYRUKYND®, AG-946, its PAH stabilizer and its novel siRNA Targeting TMPRSS6; Agios’ strategic vision and goals, including its key milestones for 2024 and potential catalysts through 2026; and the potential benefits of Agios’s strategic plans and focus. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “would,” “could,” “potential,” “possible,” “hope,” “strategy” and “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 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. Moreover, there can be no guarantee that any medicines ultimately commercialized by Agios will receive commercial acceptance. 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 pandemics or other public health emergencies 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; 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 key collaborations; uncertainty regarding any milestone or royalty payments related to the sale of Agios’ oncology business or its in-licensing of TMPRSS6 siRNA, and the uncertainty of the timing of any such payments; uncertainty of the results and effectiveness of the use of proceeds from the transaction with Servier; competitive factors; 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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