Agios Outlines 2026 Strategic Priorities and Key Milestones to Accelerate Rare Disease Portfolio Growth

January 12, 2026

- AQVESME™ (mitapivat) U.S. commercial launch in thalassemia underway following December 2025 FDA approval
- Pre-sNDA meeting with FDA for mitapivat in sickle cell disease anticipated in first quarter of 2026, with planned U.S. regulatory submission to follow
- Company progressing early- and mid-stage pipeline in multiple high-value indications
- Clear path to profitability through the company's existing commercial presence in thalassemia and PK deficiency, with potential to achieve over $1 billion in peak global sales

CAMBRIDGE, Mass., Jan. 12, 2026 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (Nasdaq: AGIO), a commercial-stage biopharmaceutical company focused on delivering innovative medicines for patients with rare diseases, today announced its 2026 strategic priorities and key milestones anticipated during the year. Members of the company's management team will present this update at the 44 th Annual J.P. Morgan Healthcare Conference on Wednesday, January 14, 2026, at 8:15 a.m. PT / 11:15 a.m. ET.

"In 2025, Agios delivered another year of strong and consistent execution across our portfolio, marking meaningful progress toward our goal of becoming a sustainable and diversified rare disease company," said Brian Goff, Chief Executive Officer, Agios. "Last year culminated in the historic U.S. approval of AQVESME™ (mitapivat), our pyruvate kinase (PK) activator and the only medicine approved to treat anemia in adults with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia. This approval brings a new, disease-modifying oral option to people living with this debilitating and deadly rare blood disorder.

"Entering 2026, the company is at an important inflection point. We will deliver a high-impact U.S. launch of AQVESME in thalassemia, seek to expand our PK activation franchise into additional high-value indications such as sickle cell disease and lower-risk myelodysplastic syndromes, and advance our promising early-stage pipeline with the potential to further diversify across hematologic and rare diseases. We also remain focused on disciplined capital allocation and operational efficiency to support our long-term sustainability. With strong momentum and a clear roadmap, Agios enters the year positioned to deliver transformative innovation and meaningful impact for patients living with rare diseases," Mr. Goff added.

Anticipated 2026 Milestones
Thalassemia

- In December 2025, the U.S. Food and Drug Administration (FDA) approved AQVESME for the treatment of anemia in adults with alpha- or beta-thalassemia. AQVESME is the only FDA-approved medicine for anemia in both non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia.
- Agios expects AQVESME to become available in the U.S. in late January 2026 following implementation of the AQVESME Risk Evaluation and Mitigation Strategy (REMS) program. Commercial launch activities are already underway and will continue throughout the year.

Sickle Cell Disease

- Topline results from the RISE UP Phase 3 trial of mitapivat in sickle cell disease were reported in November 2025. Agios anticipates having a pre-supplemental New Drug Application (sNDA) meeting with the FDA in the first quarter of 2026, and plans to submit a U.S. marketing application for mitapivat in sickle cell disease following that engagement.
- Enrollment in the Phase 2 sickle cell disease trial of tebapivat, Agios' more potent, once-daily oral PK activator, was initiated in 2025. Agios expects to report topline results from this trial in the second half of 2026.

Lower-Risk Myelodysplastic Syndromes (LR-MDS)

- Enrollment in the Phase 2b LR-MDS trial of tebapivat was completed in 2025. Agios expects to report topline results from this trial in the first half of 2026.

Polycythemia Vera (PV)

- Agios expects to report topline results from a Phase 1 healthy volunteer trial of AG-236, a small interfering RNA (siRNA) targeting TMPRSS6 as a potential treatment for PV, in the first half of 2026.

Phenylketonuria (PKU)

- With dosing completed in the Phase 1 single- and multiple-ascending-dose trial of AG-181, a phenylalanine hydroxylase (PAH) stabilizer, in healthy volunteers, Agios expects to initiate a Phase 1b proof-of-mechanism trial of AG-181 in patients with PKU in the first half of 2026 and to confirm proof of mechanism in the second half of 2026.

Presentation at 44th Annual J.P. Morgan Healthcare Conference

Members of Agios' management team will present at the 44 th Annual J.P. Morgan Healthcare Conference on Wednesday, January 14, 2026, at 8:15 a.m. PT / 11:15 a.m. ET. The live webcast will be accessible on the Investors section of the company's website (www.agios.com) under the "Events & Presentations" tab. A replay of the webcast will be archived on the company's website for at least two weeks following the presentation.

About AQVESME™ (mitapivat)
U.S. INDICATION
AQVESME is indicated for the treatment of anemia in adults with alpha- or beta-thalassemia.

U.S. IMPORTANT SAFETY INFORMATION
BOXED WARNING: HEPATOCELLULAR INJURY

AQVESME can cause serious hepatocellular injury. Measure liver laboratory tests (ALT, AST, alkaline phosphatase and total bilirubin with fractionation) at baseline and every 4 weeks for 24 weeks and then as clinically indicated. Avoid use of AQVESME in patients with cirrhosis. Discontinue AQVESME if hepatic injury is suspected.
Because of the risk of hepatocellular injury, AQVESME is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called the AQVESME REMS.

WARNINGS AND PRECAUTIONS

Hepatocellular Injury
AQVESME can cause hepatocellular injury. Avoid use of AQVESME in patients with cirrhosis. In patients with thalassemia treated with AQVESME, liver injury with and without jaundice has been observed within the first 6 months of exposure. Obtain liver tests (including ALT, AST, alkaline phosphatase, total bilirubin with fractionation) prior to the initiation of AQVESME, then every 4 weeks for the first 24 weeks, and as clinically indicated thereafter. Interrupt AQVESME if clinically significant increases in liver tests are observed or alanine aminotransferase is >5 times the upper limit of normal (ULN). Complete a comprehensive evaluation to rule out other causes of liver injury when drug-induced liver injury is suspected. Discontinue AQVESME if hepatocellular injury due to AQVESME is suspected.

Symptoms and signs of early liver injury may mimic those of thalassemia. Advise patients to report new or worsening symptoms of loss of appetite, nausea, right-upper-quadrant abdominal pain, vomiting, scleral icterus, jaundice, or dark urine while on AQVESME treatment.

During the double-blind period, 2 of 301 patients (0.66%) with thalassemia treated with AQVESME experienced adverse reactions suggestive of hepatocellular injury. Three additional patients experienced adverse reactions suggestive of hepatocellular injury during the open-label extension periods after switching from placebo to AQVESME. Of these 5 patients, 2 had serious liver injury requiring hospitalization, including 1 patient who developed jaundice (peak bilirubin 32 mg/dL). Another patient developed jaundice (peak bilirubin 4 mg/dL) without requiring hospitalization. These reactions were characterized by a time to onset within the first 6 months of treatment with peak elevations of alanine aminotransferase of >5×ULN with or without jaundice. All patients discontinued treatment with AQVESME, and these reactions improved upon treatment discontinuation.

AQVESME REMS
AQVESME is available only through a restricted program under a REMS called the AQVESME REMS because of the risk of hepatocellular injury.

ADVERSE REACTIONS
The most common adverse reactions among patients taking AQVESME were headache and insomnia.

DRUG INTERACTIONS

- Strong CYP3A Inhibitors and Inducers: Avoid concomitant use.
- Moderate CYP3A Inhibitors: Avoid concomitant use.
- Moderate CYP3A Inducers: Consider alternatives that are not moderate inducers. If there are no alternatives, see full Prescribing Information for recommended dosage for drug interactions with moderate CYP3A inducers.
- Sensitive CYP3A Substrates, including hormonal contraceptives: Avoid concomitant use with substrates that have narrow therapeutic index.
- CYP2B6, CYP2C, and UGT1A1 Substrates: Monitor patients for efficacy of the substrates with narrow therapeutic index.
- P-gp Substrates: Monitor patients for adverse reactions of the substrates with narrow therapeutic index.

HEPATIC IMPAIRMENT
Avoid use of AQVESME in patients with cirrhosis (Child-Pugh Class A, B, or C).

Please see full Prescribing Information for AQVESME, including Boxed Warning.

About Agios: Fueled by Connections to Transform Rare Diseases™
At Agios, our vision is to redefine the future of rare disease treatment. Fueled by connections, we build trusted partnerships with communities – collaborating to develop and deliver innovative medicines that have the potential to transform lives. With a foundation in hematology, we combine biological expertise with real-world insights to advance a growing pipeline of rare disease medicines that reflect the priorities of the people we serve. Agios is a commercial-stage biopharmaceutical company headquartered in Cambridge, Massachusetts. To learn more, visit www.agios.com and follow us on LinkedIn and X.

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 Agios' products, including AQVESME™; Agios' plans for future meetings with, or submissions to, regulators, including the FDA; its plans for the development of mitapivat, tebapivat, AG-236 and AG-181, and its strategic plans and focus. The words "anticipate," "expect," "goal," "hope," "intend," "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: 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' results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; 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' 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 establish and maintain key collaborations; uncertainty regarding any royalty payments related to the sale of its oncology business or any milestone or royalty payments related to its in-licensing of AG-236, and the uncertainty of the timing of any such payments; uncertainty of the results and effectiveness of the use of Agios' cash and cash equivalents; 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.

Contacts:

Investor Contact
Morgan Sanford, Vice President, Investor Relations
Agios Pharmaceuticals
morgan.sanford@agios.com

Media Contact
Eamonn Nolan, Senior Director, Corporate Communications
Agios Pharmaceuticals
eamonn.nolan@agios.com

Source: Agios Pharmaceuticals, Inc.