Agios Enters Exclusive Global License Agreement with Oscotec to Develop and Commercialize Next-Generation SYK Inhibitor Cevidoplenib

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- Agios obtains exclusive global rights to novel, late-stage, next-generation, oral SYK inhibitor
- Agreement diversifies Agios' rare hematology portfolio with expansion into immune thrombocytopenia, unlocking up to $1 billion in peak U.S. sales potential
- Oscotec will receive $25.0 million upfront, with future payments tied to development, regulatory, and commercial milestones, as well as tiered royalties on future net sales
- Agios will host investor conference call and webcast today at 8:00 a.m. ET

CAMBRIDGE, Mass., June 01, 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 an agreement with Oscotec, a clinical-stage drug discovery and development company focused on immunology and oncology, headquartered in South Korea, to license the exclusive global rights to cevidoplenib, a novel, next-generation, oral spleen tyrosine kinase (SYK) inhibitor.

Agios will focus on advancing cevidoplenib for the treatment of immune thrombocytopenia (ITP), a rare autoimmune blood disorder in which the immune system destroys platelets, leading to low platelet counts and an increased risk of bleeding. The goal of treatment in ITP is to reduce the risk of bleeding events by safely achieving stable platelet levels while minimizing the burden of treatment-related toxicities. Globally, ITP affects an estimated 200,000 individuals, including 90,000 adults diagnosed in the U.S.

Cevidoplenib is a highly selective SYK inhibitor designed to prevent the harmful autoantibody-mediated destruction of platelets – the key driver of ITP. It was also designed to address the limitations of first-generation SYK inhibitors, offering the potential for an improved tolerability profile that supports long-term, manageable care for patients. Cevidoplenib has received orphan drug designation from the U.S. Food and Drug Administration (FDA) for the treatment of ITP.

"Cevidoplenib is a next-generation SYK inhibitor uniquely designed to potentially offer improved tolerability and durability compared to first-generation SYK inhibitors. Backed by clinically meaningful Phase 2 data, we believe cevidoplenib has the opportunity to become a best-in-class treatment option for ITP," said Brian Goff, Chief Executive Officer, Agios. "Licensing this promising medicine is a natural extension of our therapeutic focus and expertise, expanding and diversifying our rare hematology portfolio into ITP – an autoimmune blood disorder with an urgent need for new treatment options. This agreement also aligns with our disciplined approach to capital allocation, enabling us to advance this novel medicine while remaining firmly focused on executing our 2026 strategic priorities."

Cevidoplenib has been evaluated in a global, randomized, 12-week Phase 2 trial assessing efficacy, safety, and dose response in adults with persistent or chronic ITP. The primary endpoint was platelet response, defined as platelet count ≥30,000/μL and doubling the platelet count compared to average platelet count during screening at any visit during the 12-week treatment period and without the use of rescue medication. While this novel primary endpoint did not achieve statistical significance, durable and clinically meaningful platelet responses were observed in the cevidoplenib arm compared with placebo across multiple secondary endpoints that align with primary endpoints used in ITP registrational trials. Additionally, cevidoplenib was well tolerated in the Phase 2 trial. Based on these results, Agios expects to advance cevidoplenib into Phase 3 development for ITP in the first half of 2028, following completion of additional chemistry, manufacturing, and controls (CMC) development work.

Under the terms of the agreement, Agios will obtain exclusive global rights to develop and commercialize cevidoplenib across all indications and will assume full responsibility for future development and commercialization costs. Oscotec will receive a $25.0 million upfront payment and is eligible to receive up to $140.0 million in development and regulatory milestones for up to three indications in the U.S. and Europe, as well as commercial milestone payments and royalties ranging from high single digit to mid-teen on future net sales. Oscotec retains the option to secure exclusive development and commercialization rights to cevidoplenib in South Korea following the release of Phase 3 trial results.

Agios continues to expect its 2026 operating expense guidance to be approximately flat compared to 2025, excluding the $25.0 million upfront payment to Oscotec.

Conference Call Information
Agios will host a conference call and live webcast today, June 1, 2026, at 8:00 a.m. ET to discuss this agreement. 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 available on the company's website approximately two hours after the event.

About Immune Thrombocytopenia
Immune thrombocytopenia (ITP) is a rare autoimmune blood disorder characterized by immune-driven loss of platelets, a type of blood cell essential for normal blood clotting. In ITP, the immune system mistakenly produces autoantibodies that mark platelets for destruction and, in some cases, also produces autoantibodies against the precursor cells responsible for producing platelets. The net effect of these actions is a reduction of platelet levels in circulation, leading to an increased risk of bleeding.

ITP affects both children and adults and, while it may initially present as an acute condition, it often persists and becomes a chronic condition in many adult patients. Globally, ITP affects an estimated 200,000 individuals, including 90,000 adults diagnosed in the U.S. Of these, roughly 50,000 have chronic ITP and require treatment for their symptoms. Approximately 24,000 of these patients do not respond to initial treatments and must progress to second-line treatment or beyond. This later-line population urgently needs new treatment options, as many patients eventually relapse or become refractory to existing therapies.

Current standard-of-care therapies are focused on increasing platelet counts and reducing bleeding risk; however, they are often associated with limitations, including delayed onset of response, lack of durable efficacy, and class-specific adverse events, such as decreased blood counts and an increased risk of infections.

About Cevidoplenib in Immune Thrombocytopenia
Cevidoplenib is a next-generation, oral spleen tyrosine kinase (SYK) inhibitor designed to prevent autoantibody-mediated platelet destruction, a key driver of disease in immune thrombocytopenia (ITP). SYK is a signaling enzyme involved in antibody-driven immune cell activation; by targeting SYK-dependent pathways, cevidoplenib is intended to reduce antibody-mediated platelet clearance and help restore platelet counts. Its selective targeting of SYK can also potentially support improved tolerability and durability for long-term use compared to first-generation SYK inhibitors. Cevidoplenib has been studied in multiple global clinical trials and remains under investigation; it has not been approved by regulatory authorities for any indication.

About Phase 2 Cevidoplenib Trial in Immune Thrombocytopenia
The Phase 2 trial (NCT04056195) of cevidoplenib was a global, randomized, double-blind, placebo-controlled, parallel-dose study conducted in adults with persistent or chronic immune thrombocytopenia (ITP).

60 patients with platelet counts <30,000/μL who had relapsed after or were refractory to at least one prior therapy were enrolled across multiple regions, including the U.S., Europe, and South Korea. Patients were randomized 1:2:2 to receive placebo, cevidoplenib 200 mg twice daily, or 400 mg twice daily for 12 weeks. The median age was 60 years (range: 23-86). Stable background ITP therapies were permitted, and the study population was heavily pretreated, with 68.3% of patients having received three or more prior lines of therapy. Most patients had severe thrombocytopenia at baseline, with 68.3% having platelet counts <15,000/μL. Additionally, 63.3% were nonresponders to prior therapies and 81.7% had relapsed disease.

The primary endpoint was platelet response, defined as platelet count ≥30,000/μL and doubling the platelet count compared to average platelet count during screening at any visit during the 12-week treatment period and without the use of rescue medication. While this novel primary endpoint did not achieve statistical significance, durable and clinically meaningful platelet responses were observed in the cevidoplenib arm compared with placebo across multiple secondary endpoints that align with primary endpoints used in ITP registrational trials. Durable platelet responses with cevidoplenib were observed across secondary endpoints measuring at least two consecutive platelet counts ≥30,000/μL and ≥50,000/μL.

Cevidoplenib was well tolerated in the Phase 2 trial. The most commonly reported treatment-related adverse events included transient elevations in liver enzymes and gastrointestinal events, and no new safety signals were identified.

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.

Available Information about Agios
To achieve broad dissemination, Agios may disclose information to the public through a variety of disclosure channels including press releases, SEC filings, and public conference calls and webcasts. Some of the information distributed through these disclosure channels may be considered material information. Investors and others should note that Agios plans to use its website (www.agios.com) as a distribution channel to announce and give notice of Agios' upcoming events and presentations (including, but not limited to, presentations at medical or healthcare conferences). Such information, which may be deemed material, will be available on the Investors section of the company's website under the "Events & Presentations" tab. In addition, you may sign up to automatically receive email alerts about Agios' upcoming events and presentations ("Calendar Alerts") by visiting the "Email Alerts" option under the "IR Resources" tab of the Investors section of the company's website and submitting your email address.

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 Agios' exclusive license agreement with Oscotec; the potential benefits of cevidoplenib; 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 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; 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 or cevidoplenib, 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