Cereno Scientific

Cereno Scientific Receives FDA Clearance to Initiate Global Phase IIb Trial of CS1 in Pulmonary Arterial Hypertension (PAH)

Cereno Scientific (Nasdaq First North: CRNO B), an innovative biotech pioneering treatments to enhance and extend life for people with rare cardiovascular and pulmonary diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted clearance to initiate the company's Phase IIb trial of its lead drug candidate CS1 for the treatment of pulmonary arterial hypertension (PAH). The FDA's decision enables Cereno to advance toward first patient in (FPI) in Q2 2026, with top-line data anticipated around Q4 2028, subject to enrollment timelines. The clearance follows constructive regulatory interactions and builds on the favorable safety, tolerability and encouraging disease-modifying signals observed in the Phase IIa study. CS1 has also been granted Orphan Drug Designation and Fast Track designation in the U.S.

"Our goal with CS1 is to address the underlying mechanisms that drive PAH, not simply manage symptoms," said Rahul Agrawal, CMO and Head of R&D at Cereno Scientific. "The planned Phase IIb trial is designed to determine the optimal dose for a Phase III trial and to assess CS1's potential to meaningfully reduce pulmonary vascular resistance and improve functional capacity when added to today's standard therapies. Building on the encouraging signals observed in our Phase IIa study, we believe CS1 could offer a differentiated and disease modifying approach for patients living with this rare and fatal disease."

The Phase IIb trial is formally titled "A Phase 2b, Double-Blind, Randomized, Placebo-Controlled, Dose-Finding Study, to Compare the Efficacy and Safety/Tolerability of CS1 Versus Placebo When Added to Standard of Care for the Treatment of Pulmonary Arterial Hypertension (PAH)." It is a global, multicenter trial enrolling approximately 126 patients with PAH who are stable on background therapy. During the first 36 weeks of treatment, participants will be randomized to receive once-daily CS1 capsules at one of two dose levels or matching placebo. At Week 36, all participants will be re-randomized: those initially receiving CS1 will be assigned either to continue on their CS1 dose or switch to placebo, while those initially receiving placebo will be assigned to one of the two CS1 dose groups. This design ensures that all participants will receive active treatment at some point of the trial and allows CS1's previously observed disease-modifying signals to be further evaluated in a larger, controlled trial. All participants will then continue in a second treatment period and follow-up. The total study duration is 60 weeks, including screening and follow-up. The study will evaluate the effect of CS1 on pulmonary vascular resistance (PVR) at Week 36 via right-heart catheterization, changes in 6-minute walk distance at Week 36, and a range of additional evaluations including measures of heart function, biomarker changes, clinical worsening, patient-reported outcomes, and pharmacokinetics. This dose-finding trial is expected to be conducted across 10–12 countries in the U.S., Europe and South America at approximately 65 investigative sites.

"Receiving FDA clearance to proceed with our Phase IIb study is a significant catalyst for Cereno," said Sten R. Sörensen, CEO at Cereno Scientific. "This milestone strengthens the commercial potential of CS1, supports our positioning as a leader in epigenetic modulation for rare cardiovascular diseases, and creates additional momentum in our partnering discussions.

With Fast Track designation and global site participation, we see clear opportunities to accelerate our path toward a potential disease-modifying therapy that can enhance and extend the lives of patients with PAH."

Cereno Scientific is now advancing global study-start activities, including site selection, regulatory submissions in participating countries, and operational readiness with its selected CRO partner. First patient in (FPI) is planned for Q2 2026, with top-line results anticipated around Q4 2028, subject to enrollment timelines. These activities mark the start of the largest and most comprehensive clinical program undertaken by the company to date.

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This information is information that Cereno Scientific AB is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact person set out above, at 22:25 (CET) on December 8, 2025.

About PAH

PAH is a rare, progressive and life-threatening disease characterized by high blood pressure in the pulmonary arteries that leads to right heart failure and premature death. Current standard treatments mainly focus on managing symptoms, leaving a significant unmet need for disease-modifying therapies that can change the course of disease and improve long-term outcomes.

About CS1

CS1 is an orally administered histone deacetylase inhibitor (HDACi) in development as a well-tolerated, disease-modifying therapy for pulmonary arterial hypertension (PAH) with favorable safety profile. Acting through epigenetic modulation, CS1 targets key disease-driving mechanisms such as vascular remodeling, fibrosis and inflammation. CS1 has shown disease-modifying potential in early clinical evaluation and is being evaluated as an add-on (on top of standard-of-care) therapy with the potential to improve outcomes for patients with high unmet medical needs. It has been granted Orphan Drug Designation (ODD) in both the U.S. and the EU and received Fast Track designation from the U.S. FDA in August 2025, underscoring its potential to address a serious condition with high unmet medical need.

CS1 has first-in-class potential and is currently in Phase II clinical development.

About Cereno Scientific AB

Cereno Scientific is pioneering treatments to enhance and extend life. The company's innovative pipeline offers disease-modifying drug candidates to empower people suffering from rare cardiovascular and pulmonary diseases to live life to the fullest.

Lead candidate CS1 is an HDAC inhibitor that works through epigenetic modulation and represents a novel therapeutic approach by targeting the root mechanisms of the pulmonary arterial hypertension (PAH). CS1 is a well-tolerated oral therapy with a favorable safety profile that has shown encouraging efficacy signals of reverse vascular remodeling, improvement of right heart function and enhanced patient quality of life in a Phase IIa trial in patients with PAH. An Expanded Access Program enables patients that have completed the Phase IIa trial to gain access to CS1. CS014, a new chemical entity with disease-modifying potential, showed favorable safety and tolerability profile in a Phase I trial. CS014 is a HDAC inhibitor with a multimodal mechanism of action as an epigenetic modulator having the potential to address the underlying pathophysiology of rare cardiovascular and pulmonary diseases with high unmet needs such as idiopathic pulmonary fibrosis (IPF). Cereno Scientific is also pursuing a preclinical program with CS585, an oral, highly potent and selective prostacyclin (IP) receptor agonist that has demonstrated the potential to significantly improve disease mechanisms relevant to cardiovascular diseases. While CS585 has not yet been assigned a specific indication for clinical development, preclinical data indicates that it could potentially be used in indications like thrombosis prevention without increased risk of bleeding.

The Company is headquartered in GoCo Health Innovation City, in Gothenburg, Sweden, and has a US subsidiary; Cereno Scientific Inc. based in Kendall Square, Boston, Massachusetts, US. Cereno Scientific is listed on the Nasdaq First North (CRNO B). The Company's Certified Adviser is DNB

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