Press release
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Cereno Scientific

Cereno Scientific Submits Phase IIb Trial Protocol for CS1 in Pulmonary Arterial Hypertension (PAH) to the U.S. FDA

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 the submission of the clinical trial protocol for the planned global Phase IIb trial of its lead drug candidate CS1 to the U.S. Food and Drug Administration (FDA). The submission marks an important milestone, moving the company closer to advancing CS1 into its next clinical phase and toward bringing a novel therapeutic approach to patients living with pulmonary arterial hypertension (PAH).

The planned Phase IIb trial is designed to further evaluate the safety, tolerability and efficacy of CS1, a histone deacetylase inhibitor (HDACi) in development as an oral treatment targeting the root mechanisms of PAH through epigenetic modulation. The Phase IIb trial will build on the results from the completed Phase IIa trial, where CS1 demonstrated a favorable safety and tolerability profile together with encouraging efficacy signals including reverse vascular remodeling, improved right heart function, and enhanced patient quality of life. The new global, multicenter, placebo-controlled trial will be conducted in collaboration with a leading international contract research organization (CRO). Regulatory interactions in other key regions will follow as part of the global start-up preparations.

“The submission of the protocol marks a major milestone and underscores the focus, commitment and outstanding collaboration across our team and global CRO,” said Rahul Agrawal, CMO and Head of R&D of Cereno Scientific. “This protocol has been developed in alignment with the FDA’s feedback from our recent Type C meeting, and we now look forward to the agency’s review ahead of initiating the trial.”

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.

“This milestone moves us closer to our goal of delivering a first-in-class therapy that targets the root mechanisms of PAH through epigenetic modulation,” said Sten R. Sörensen, CEO of Cereno Scientific. “With CS1, we have an opportunity to potentially change the treatment paradigm for patients facing this devastating disease and, in doing so, create meaningful long-term benefits for both patients and shareholders.”

Following the FDA's standard 30-day review, Cereno Scientific anticipates clearance to proceed with the trial. The Phase IIb trial is planned to begin during H1 2026 as part of the company's global development program for CS1.

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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 full.

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 Carnegie Investment Bank AB, certifiedadviser@carnegie.se. More information can be found on www.cerenoscientific.com.