PRESS RELEASE
26 June 2026 07:45:00 CEST

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Cereno Scientific Reports EAP Analyses with CS1 in PAH: Majority of Completing Patients Achieved Stable or Improved Clinical Status over 12 Months

Cereno Scientific (Nasdaq First North: CRNO B), a biotech pioneering treatments to enhance and extend life for people with rare cardiovascular and pulmonary diseases, today reported further analyses from the 12-month Expanded Access Program (EAP) with its lead drug candidate CS1 in pulmonary arterial hypertension (PAH). Over 12 months, the majority of patients who completed treatment maintained or improved their functional and biomarker status in a progressive disease, with CS1 showing a continued favorable safety and tolerability profile. These observations support CS1's potential as a well-tolerated therapy with favorably safety and disease-modifying effects and strengthens confidence ahead of the planned global Phase IIb trial starting in June 2026.

The EAP was conducted under a formal FDA protocol and was initiated at the request of patients and physicians to enable continued treatment with CS1 following the Phase IIa trial. It enrolled ten patients who had completed that trial. The program's primary objective of evaluating long-term safety and tolerability was met, with no unexpected safety concerns and no deaths reported.

Of the ten patients enrolled, six completed 12 months of treatment. Four patients did not complete the full treatment period, none were due to reasons related to CS1. Two patients discontinued CS1 after developing atrial fibrillation and subsequently being prescribed anticoagulation therapy by their treating physicians. Because anticoagulant use was an exclusion criterion under the EAP protocol, these patients were required to stop CS1 treatment. Atrial fibrillation is a known condition in PAH, particularly in later stages of disease, and was not considered related to CS1. One patient withdrew consent, and one was lost to follow-up. Importantly, no discontinuation was assessed as related to CS1.

Clinical observations over 12 months

Patients did not roll over directly from the Phase IIa trial into the EAP as originally planned. The interval between completion of Phase IIa and initiation of EAP treatment ranged from six to 25 months, during which patients received changes to their standard PAH therapy, and CS1 was given mainly on stable background therapy. The results are presented as real-world clinical observations of individual patient responses, not as controlled efficacy results.

Among the six patients who completed 12 months of treatment:

- Five of six had stable (4) or improved (1) NYHA/WHO functional class
- Five of six had stable (1) or improved (4) NT-proBNP
- Three of six improved six-minute walk distance
- Three of six had stable (1) or improved (2) REVEAL Risk Score 2.0
- Three of five evaluable patients had stable (2) or reduced (1) mean pulmonary arterial pressure, measured by CardioMEMS

In a disease such as PAH that typically progresses despite available treatment, maintained or improved clinical status across these measures over a year of treatment is considered supportive and clinically relevant, and is consistent with the profile seen in the Phase IIa trial.

The EAP also showed that CS1 could be administered alongside currently approved PAH therapies, including concomitant treatment with sotatercept. This supports the feasibility of evaluating CS1 as an add-on therapy in a modern PAH treatment setting and is directly relevant to the design of the Phase IIb trial.

“What is particularly meaningful to me is that these additional analyses provide important long-term clinical context for CS1 in PAH. The EAP further reinforces the favorable safety and tolerability profile observed in the Phase IIa trial and shows that patients completing 12 months of treatment were generally stable or improved across several clinically relevant measures. While conclusions regarding efficacy cannot be drawn from this small, non-controlled study, maintaining clinical stability over time in a progressive and fatal disease such as PAH is especially encouraging. These findings provide valuable momentum and support our continued conviction in the potential of CS1 as we advance the program into the planned Phase IIb study,” said Rahul Agrawal, CMO and Head of R&D of Cereno Scientific.

Relevance to CS1’s disease-modifying mechanism

CS1 is an HDAC inhibitor acting as an epigenetic modulator, in development as a potentially disease-modifying therapy that targets the underlying disease mechanisms of PAH, including vascular remodeling, fibrosis and inflammation.

PAH is a progressive disease in which patients deteriorate over time despite available treatment. In this context, maintained or improved clinical status in real-world observations over 12 months treatment is clinically relevant, particularly when seen alongside a favorable long-term safety and tolerability profile as well as being combined with other standard therapy. This is a clinical signal that is expected of a disease-modifying therapy. The EAP findings are supportive of this profile and consistent with the observations in the previous Phase IIa trial.

“I have observed patients continue treatment with CS1 over a longer period in a real-world clinical setting in the EAP. In my experience, CS1 has been well tolerated, including when used alongside background PAH therapies. For patients with PAH, where long-term disease management is critical, these observations are encouraging and support further evaluation of CS1 as a potential new treatment option,” said Dr. Jason Guichard, Prisma Health-Upstate, investigator in the EAP and Phase IIa trial of CS1 in PAH.

Limitations

The EAP was open-label, enrolled a small number of patients and was not designed or powered to demonstrate efficacy; no conclusions regarding efficacy can be drawn. Since patients did not roll over directly from the Phase IIa trial, and intervening changes to background therapy means the data is presented as real-world clinical observations rather than controlled efficacy results. The exploratory Fluidda imaging sub-study included only three patients; no consistent imaging results were observed and no conclusions can be drawn.

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The Phase IIb trial

“The EAP has delivered what it was designed to provide: important long-term safety and tolerability data, together with additional real-world clinical observations that strengthen our understanding of CS1 in PAH. I am pleased to see these findings build on the encouraging Phase IIa results and reinforce the potential of CS1 as an oral, once-daily, disease-modifying therapy. We are entering the next stage of development with strengthened confidence as we prepare to initiate the global Phase IIb study in June 2026,” said Sten R. Sörensen, CEO of Cereno Scientific.

The planned global, placebo-controlled Phase IIb study will enrol approximately 126 patients across approximately 65 investigational sites in 10–12 countries in North America, Europe and South America. Developed in dialogue with the FDA, the study will evaluate the safety, tolerability and efficacy of CS1 when added to standard of care, while identifying the optimal dose for Phase III.

*To help investors better understand the EAP results, we have also published an explanatory article on our website: **https://www.cerenoscientific.com/en/newsroom/stories**.*

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About CS1
CS1 is Cereno Scientific's lead drug candidate, an HDAC inhibitor acting through epigenetic modulation with a novel therapeutic approach targeting underlying disease-driving mechanisms in pulmonary arterial hypertension (PAH), including vascular remodeling, fibrosis and inflammation. In a completed Phase IIa trial, CS1 demonstrated a favorable safety and tolerability profile together with efficacy signals suggesting improvements in right heart function, functional class and patient quality of life. The data also provided early signs consistent with reverse vascular remodeling, a finding that supports CS1's potential to address underlying drivers of disease progression in PAH. Long-term follow-up data from the completed 12-month Expanded Access Program (EAP) confirmed a favorable safety and tolerability profile over up to 15 months of treatment experience, consistent with observations in the Phase IIa trial, and showed that a majority of patients completing treatment maintained or improved clinical status. CS1 is being developed as an oral, once-daily potentially disease-modifying treatment for PAH. CS1 has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) and the European Commission for the treatment of PAH, as well as FDA Fast Track designation.

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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 in a Phase IIa trial in patients with PAH, including improvements in right heart function, functional class and patient quality of life, with early signs consistent with reverse vascular remodeling. An Expanded Access Program confirmed CS1 to be well-tolerated with a favorable safety profile over 12 months of treatment and showed that a majority of patients completing treatment maintained or improved clinical status. CS014 is a new chemical entity and HDAC inhibitor with a multimodal mechanism of action as an epigenetic modulator having the potential to address the underlying pathophysiology of a range of cardiovascular and pulmonary diseases with high unmet needs. CS014 showed favorable safety and tolerability profile in Phase I, development focus for Phase II is pulmonary hypertension associated with interstitial lung disease (PH-ILD). Cereno Scientific is also advancing the preclinical program CS585, an oral, highly potent and selective prostacyclin (IP) receptor agonist shown to prevent thrombosis without increased bleeding risk, currently being evaluated in antiphospholipid syndrome (APS).

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**.