Cereno Scientific move to request “compassionate use” of CS1 for treatment of patients with rare disease PAH

Cereno Scientific (Nasdaq First North: CRNO B), a company developing innovative treatments for common and rare cardiovascular disease, today announced that the company will submit a request for expanded access to investigational drug CS1 for use as a treatment outside of a clinical trial, sometimes called “compassionate use.” The initiative is prompted by a request from an investigator in the ongoing Phase II study of CS1. Cereno will submit a request to the FDA under the ‘Expanded Access to Investigational Drugs for Treatment Use’ requesting expanded access to CS1 which initially will be limited to patients who have completed the Phase II study in PAH.

The Expanded Access is a potential pathway for a patient with a serious or immediately life-threatening disease to gain access to an investigational medical product for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available. If Cereno’s request for expanded access to CS1 is authorized by the FDA and subsequently approved by the local hospital sites’ ethics committees, patients having completed the Phase II study will, in consultation with the investigator, have the option to continue with the CS1 drug therapy. The expanded access would provide Cereno with the ability to collect efficacy and safety data on extended long-term exposure to CS1 in patients with pulmonary arterial hypertension (PAH) under a formal FDA-approved protocol, which could provide support to later applications to the FDA such as fast-track designation/breakthrough therapy and to obtain the IND acceptance to start a Phase III study with CS1.

“I am excited with the prospect of being able to support these patients for an extended period outside of our 12-week clinical trial as PAH truly is a debilitating and fatal disease. For Cereno, an expanded access to CS1 for patients having completed the study would provide value for our development program with CS1 in PAH. “Compassionate use” would allow us to collect more data on the usage of CS1 in this patient population over an extended period of time; data, which could add insight and value to our program for CS1 in its clinical development journey towards market approval. This is a high-priority activity for our clinical team at Cereno and we are aiming to submit the request to the FDA as soon as we are ready in Q4,” said Sten R. Sörensen, CEO of Cereno. “The ‘compassionate use’ request, combined with the previously reported Patient Case and positive findings of the DQCR, has further strengthened our belief in a positive outcome of the Phase II study and, ultimately, CS1’s potential as a game-changer for patients with PAH.”

The Phase II study of CS1 in the rare disease PAH is actively running at 9 specialist clinics in the US with two new clinics currently in late-stage start-up process. The company has earlier this year reported positive findings from the ongoing study suggesting a potential positive effect of drug candidate CS1 in patients with the severe rare disease PAH. First, a patient
case study performed on the first patient having completed the study at a specific clinic showed remarkable efficacy data. In 12 weeks of treatment with CS1, the patient showed a 30% reduction in pulmonary pressure and a 20% increase in cardiac output. The patient’s overall functional status was changed from NYHA/WHO functional class II to I at the end of the treatment period, meaning that she had next to normal functional physical capacity with CS1. In addition, Cereno reported in October 2023 that a Data Quality Control Review (DQCR) was concluded with positive findings. The data quality of the CardioMEMS measurements was found satisfactory with adherence to study protocol and with timely data transfers from the patient’s home to the clinic. Efficacy findings showed a clinically meaningful reduction of pulmonary pressure in several patients, included in the data quality control, of a similar or greater magnitude as in the Patient Case. The review included data obtained by the CardioMEMS HF System from the first 16 patients enrolled in the study and the reported findings can be read in full in a previous announcement and below.

For further information, please contact:

Tove Bergenholt, Director IR & Communications
Email: tove.bergenholt@cerenoscientific.com
Phone: +46 732-366 246

Sten R. Sörensen, CEO
Email: sten.sorensen@cerenoscientific.com
Phone: +46 73-374 03 74

About the DQCR initiative

The Data Quality Control Review (DQCR) initiative was performed in October 2023 with the aim of correcting potential deviations from the set protocol or identifying issues around data transfer from the patient’s home to the clinic to increase standardization of the data and also obtain an early indication of CS1’s efficacy. The DQCR was performed on blinded data regarding the individual patient dosing. The review included data obtained by the CardioMEMS HF System from the first 16 patients enrolled in the Phase II study.

Key findings from the DQCR:

1. The DQCR concluded no concerning issues with digital data transfer and patient/physician protocol adherence.
2. The DQCR shows several patients with a reduction in mPAP of similar or greater magnitude as the initial Patient Case as measured with CardioMEMS HF System over time (AUC mmHg days). This indicates a clinically meaningful efficacy potential with CS1 in reducing mPAP in patients with PAH on top of standard-of-care drug therapy.
3. The DQCR shows that more than 60% of patients on CS1, all doses included, have a sustained reduction in mPAP evaluated as the AUC.
4. Reductions of mPAP (AUC) as so far seen in several patients in this study are clinically meaningful for patients with PAH.
5. The DQCR indicates an efficacy response compatible with a dose-response pattern. As the analysis was performed with dosages blinded, the final assessment of a dose-response relationship will need to await unblinding of the data at the end of the study.

6. The DQCR indicates an early onset of action with drug therapy of CS1 as measured by the reduction of mPAP. This early onset was observed already after 3 weeks for several patients.

7. The DQCR showed a sustained reduction of mPAP in the 2-week follow-up period after the 12-week period of therapy with CS1 was discontinued.

The Phase II study will continue to completion without any changes to the study protocol. The DQCR findings are not based on data from all patients participating in the Phase II study and some patients in this analysis have not completed the full study period. The final results of the study may differ from the findings in this DQCR and shall not in any way be seen as a guarantee regarding the outcome and conclusions of the upcoming final Phase II study results.

About the Phase II study of CS1

The Phase II study of CS1 in the rare disease pulmonary arterial hypertension (PAH) is actively recruiting patients at 9 specialist clinics in the US, and two new clinics are in the process of opening. An investigator-initiated patient case study performed on the first patient having completed the study at the clinic the investigator was based showed remarkable efficacy data. In 12 weeks of treatment with CS1, the patient showed a 30% pulmonary pressure reduction and a 20% increase in cardiac output. The patient’s overall functional status was changed from NYHA/WHO functional class II to I at the end of the treatment period, meaning that she had next to normal functional physical capacity. A data quality control initiative was performed confirming the utility of the CardioMEMS HF System (Abbott Inc.) and showed that CS1 has a clinical meaningful reduction of pulmonary pressure, a key marker of the PAH disease burden. The initial findings are, however, not a guarantee of the final study result. The study is designed to randomize 30 PAH patients and the top-line result of the Phase II study is estimated to be reported in Q2 2024.

About Cereno Scientific AB

Cereno Scientific develops innovative treatments for common and rare cardiovascular disease. The lead drug candidate, CS1, is a HDAC (histone deacetylase) inhibitor that acts as an epigenetic modulator with pressure-reducing, reverse-remodeling, anti-inflammatory, anti-fibrotic and anti-thrombotic properties. A Phase II study is ongoing to evaluate CS1’s safety, tolerability, and efficacy in patients with the rare disease pulmonary arterial hypertension (PAH). A collaboration agreement with global healthcare company Abbott allows Cereno to use their cutting-edge technology CardioMEMS HF System in the study. Two initiatives performed during the ongoing Phase II study have shown positive findings suggesting the potential clinical benefit of CS1 in PAH patients. These initial findings are, however, not a guarantee of the final study results that are expected in Q2 2024. Cereno also has two promising preclinical drug candidates in development through research collaborations with the University of Michigan. Investigational drug CS014 is a HDAC inhibitor in development as a treatment for arterial and venous thrombosis prevention. The innovative drug candidate represents a groundbreaking approach to antithrombotic treatment potentially without the associated increased risk of bleeding in humans. CS014 is a new chemical entity with a multi-fold mechanism of action as an epigenetic modulator – regulating platelet activity, fibrinolysis, and clot stability for the prevention of thrombosis without increased risk of bleeding as documented in preclinical studies. Drug candidate CS585 is a prostacyclin receptor agonist that has been documented in preclinical studies to target the IP receptor for prevention of thrombosis without increased risk of bleeding. The company is headquartered in Gothenburg, Sweden, and has a US subsidiary Cereno Scientific Inc. based in Kendall Square in Boston, Massachusetts, US. Cereno is listed on the Nasdaq First North (CRNO B). More information on www.cerenoscientific.com.