Cereno Scientific reports significant progress and a timeline adjustment in the Phase II study of CS1 in rare disease PAH

Cereno Scientific (Nasdaq First North: CRNO B), a company developing innovative treatments for common and rare cardiovascular disease, today announced an update on the progress of the Phase II study of CS1 in pulmonary arterial hypertension (PAH). The company reports significant progress in the study, however, a slower recruitment pace than estimated during the last months and a longer start-up phase for two new clinics have affected the study timeline. The updated study timeline now expects study completion and top-line results during Q2 2024.

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. To date, 32 patients have been enrolled in the study; 8 of those did not, after consent, meet all study criteria to continue in the study; 20 patients have received the CardioMEMS HF System implantation; 19 patients have been randomized to drug therapy; and 16 patients have completed the study, one of which terminated early due to non-drug related issues.

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.

“We are highly encouraged by the remarkable findings in the reported Patient Case as well as the positive findings from the DQCR that our drug candidate CS1 might be able to document substantial clinical benefits in this fatal disease. We are working hard to complete the recruitment of 30 patients to randomize to treatment with CS1 in the study. We have seen significant progress in the last period, however, there are still some patients to recruit. We currently have a number of additional patients identified at our current sites and our
two new sites to be activated and are optimistic about the execution of the study based on this updated timeline,” said Björn Dahlöf, CMO of Cereno.

“I am excited to have seen the positive events of the study unfold with the remarkable clinical results in the Patient Case and subsequently the positive findings seen in the DQCR and the data we have seen so far have certainly raised our expectations about the possible benefits of CS1 therapy in this disease with such a strong unmet need for new and better therapeutic options. Although we now have to adjust our timeline of reporting top-line results to Q2 next year, I am happy to see significant progress with as many as 16 patients who already have completed the study and indications that the pace of recruitment seem to be picking up in Q4,” said Sten R. Sörensen, CEO of Cereno.

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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 07.59 (CET) on November 17, 2023.

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 with CS1. 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.