

Dear Niemann-Pick type C Community,

We are pleased to inform you that on November 16th Orphazyme A/S was listed on the Nasdaq OMX exchange in Copenhagen through an Initial Public Offering (IPO).

Orphazyme decided to become a publicly traded company to raise the capital necessary to complete all the clinical and nonclinical studies along with the manufacturing validation work required by regulatory authorities. This will enable us to file a New Drug Application with the FDA and a Marketing Authorization Application with the EMA for the approval of our investigational product arimoclomol for the treatment of Niemann-Pick type C.

We plan to also use the net proceeds from the IPO to advance the development of arimoclomol for the potential treatment of additional serious rare misfolded protein diseases, where like NPC, there remains a high unmet medical need. However, our mission remains unchanged and the NPC community can be assured that the patients and their families affected by NPC will continue to be our number one priority.

Now that we have the necessary funds to generate the data and documentation required for the regulatory submissions for NPC, we will expand our organization, push even harder, and more intensely focus on executing the necessary development and regulatory activities to ensure arimoclomol becomes available to the patients with NPC.

NPC remains our lead and most advanced program with the Phase 3 trial of arimoclomol fully enrolled and ongoing. 50 patients are enrolled in the trial for participation in the 12-month randomized placebo-controlled treatment period of the trial. We can't thank these patients and families enough for their involvement to help make the trial possible, as well as for their courage in advancing science for the community as a whole.

The first patient was enrolled into the Phase 3 trial in June 2016. Of the 50 patients enrolled, five patients have already completed their 12-month treatment period, and these patients have all opted to roll over to the open-label extension of the trial where all are receiving arimoclomol under continued treatment. As the last patient was enrolled in May 2017, we expect to have top-line results of this Phase 3 trial in the autumn of 2018.

We are honoured to have been working with the NPC Community since 2010, and we believe the IPO will ensure that we are in the best possible position to take the next part of the journey together. Appreciating the urgent need to bring arimoclomol to patients with NPC, and assuming the Phase 3 trial results will be positive, we recognize our responsibility to achieve this goal.

Sincerely yours,

Anders Hinsby
CEO, Ophazyme

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