ORPHAZYME COMPLETES ENROLMENT OF PATIENTS FOR PHASE 3 CLINICAL TRIAL IN NIEMANN-PICK TYPE C DISEASE

Copenhagen, 11 May 2017

Orphazyme ApS, a Danish biotech company developing novel therapies for the treatment of protein misfolding diseases, today announced the completion of enrolment for its lead program within Niemann-Pick type C disease.

Niemann-Pick type C disease (NPC) is one of several fatal lysosomal storage diseases and is affecting around 1 in 150,000 newborns. Although symptom onset and progression is highly variable, NPC is most often diagnosed in childhood and adolescence. Impaired breakdown and recycling of lipids in cells of affected individuals leads to malfunction and demise of cells in the brain and other organs, which results in seizures, progressive loss of muscle control and intellectual capacity.

The trial is a multicentre, prospective, double-blind and placebo controlled interventional study. The purpose of this trial is to assess the efficacy and safety of arimoclomol, compared to placebo, in the treatment of Niemann-Pick type C disease, when it is administered in addition to the patient's current prescribed best standard of care.

CEO Anders Hinsby said: “Enrolment of this trial has truly been a joint effort with the dedicated NPC patient community and physicians. We are grateful for commitment of the trial participants and their families and the hard work of the involved investigators. Our hope is that arimoclomol will dramatically improve the lives of those suffering from NPC.”

The primary endpoint of the study is the change in disease severity in patients treated with arimoclomol compared to placebo, which, supported by biochemical readouts, will provide the basis for an application for market authorisation at the end of the study. The trial is estimated to complete in the second quarter 2018.

Arimoclomol is a new chemical entity with a very favorable safety and tolerability record in humans: eight Phase I clinical studies have been conducted in healthy volunteers. Arimoclomol is administered orally, three times daily and can be easily dissolved in liquids or food for best possible patient comfort and compliance.

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About Orphazyme ApS

Orphazyme ApS is a Danish biotech company developing paradigm-changing medicines for the treatment of protein misfolding diseases with well-characterised mechanisms of disease. The lead program is in development as a treatment for the lysosomal storage disease Niemann-Pick type C. This is one of a family ~50 debilitating genetic disorders that often affect children, most of whom are currently untreatable. Orphazyme is owned by an investor syndicate comprising Novo A/S, Sunstone Capital, Aescap Venture, Kurma Partners, LSP, ALS investment fund and Idinvest Partners. For more information, please visit www.orphazyme.com