

Orphazyme moves towards clinical development in Niemann-Pick type C

Orphazyme has identified and evaluated two candidate products through preclinical development for Niemann-Pick type C. One is the recombinant human protein HSP70 and the other is an orally available small molecule, Arimoclomol that works as an inducer of heat shock proteins, including HSP70.

Both product candidates have produced encouraging biochemical and disease-modifying effects in studies in the Niemann-Pick type C mouse model performed in collaboration with Prof. Frances Platt (University of Oxford). Both compounds have successfully completed toxicity studies and are now enabled for clinical trials. In the third quarter of 2014 Orphazyme will select the product candidate for a clinical Niemann-Pick type C trial. We expect to start the multi-centre clinical trial in patients in the first quarter of 2015. Final inclusion criteria and participating clinical sites are still to be confirmed.

It is a priority for Orphazyme to ensure the clinical and regulatory feasibility of our program in continuous dialogue with patient organizations and national regulatory agencies including EMA and the FDA.

Orphazyme's Chief Scientific Officer and Founder, Dr. Thomas Kirkegaard Jensen, and Chief Medical Officer Dr. Carlos R. Camozzi, MD, will present the preclinical results and supply further information about the planned clinical trial at the Niemann-Pick Disease Group (NPDG) UK meeting, 26-28. September 2014.

We look forward to sharing the exciting data and plans with the patient community.

Kindest regards,

Anders M. Hinsby, Orphazyme Chief Executive Officer