



January 19, 2018

Dear NNPDF and the Niemann-Pick Type C Community,

We are pleased to inform you that arimoclomol, an investigational medicine currently under study in the Phase 2/3 clinical research stage, has been granted Rare Pediatric Disease Designation by the US Food and Drug Administration (FDA) for the treatment of Niemann-Pick disease Type C (NPC). This designation is granted for diseases that are rare and serious or life-threatening in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years old. A drug qualifies for this designation if the prevalence of the disease in the US is below 200,000 people and if more than 50% are between the ages of 0-18 years old.

We are very pleased with the FDA's continued support for the development of therapies for children with rare diseases. Arimoclomol has previously been granted Orphan Drug and Fast Track Designation by the FDA, which underscores the urgent unmet medical need in NPC. Along with the Rare Pediatric Disease Designation, these programs are intended to facilitate development and expedite review of drugs to treat serious and life-threatening conditions where there is an unmet medical need so that an approved product can reach the market expeditiously.

We look forward to continue working closely with the FDA and the NPC community in our effort to provide a treatment option for those suffering from this disease.

Sincerely yours,

Anders Hinsby
CEO Orphazyme