

**Press Release:****Orphazyme begins dosing of Niemann-Pick disease type C patients in arimoclomol AIDNPC interventional study**

**Copenhagen, Denmark, 15 June 2016.** Orphazyme announced today that the first two patients have been dosed with arimoclomol in the Phase III 'AIDNPC' Niemann-Pick disease type C clinical trial programme.

The first two patients in the NPC-002 Interventional Study of the AIDNPC clinical programme were enrolled at the Rigshospitalet site in Copenhagen, Denmark, marking a key milestone in the development of a new treatment option for Niemann-Pick disease Type C (NP-C) patients.

*"We have reached an important milestone in this pivotal registration study in Niemann-Pick disease type C,"* Christina Guldborg, Director of Clinical Operations at Orphazyme, says, *"and we are looking forward to enrolling patients at each of the participating clinical sites, as the '-002' study opens up in the other seven European countries and USA."*

Orphazyme runs the NPC-002 international, multi-centre, double-blind, placebo-controlled interventional study, enrolling approximately 46 Niemann-Pick disease Type C patients who will be treated with three daily oral doses of arimoclomol or placebo. The objective of the study is to determine the efficacy and safety of arimoclomol in the treatment of Niemann-Pick disease Type C.

To start the intervention study at all the 16 European sites, Orphazyme is seeking approval for clinical trial applications with eight national competent authorities and ethics committees. The 32 patients who are already participating in the observational study phase (NPC-001) will be invited into the treatment phase (NPC-002) as soon as the approvals are received in the respective jurisdictions.

*"On this day, we are very pleased to have included two patients in the interventional study. We are grateful to the two affected children and their families for their commitment and participation, and we are dedicated to working tirelessly with all patients, families and participating physicians to conduct the study expediently. The NPC-002 study will now progress to include more patients in the pursuit of a safe and efficacious oral drug for Niemann-Pick disease type C,"* adds Christine í Dali MD, VP of Clinical Development.

**For further information:**

Christine í Dali MD, VP Clinical Development at Orphazyme ApS

E-mail: [contact@orphazyme.com](mailto:contact@orphazyme.com)

**About the AIDNPC Clinical Programme**

The AIDNPC programme consists of two studies: An Observational Study followed by an Interventional Study. The Observational Study is a natural history study that has only been conducted in Europe. In the Interventional Study (NPC-002), European and US Niemann-Pick disease Type C patients will receive oral arimoclomol or placebo three times daily for 12 months. All patients will subsequently be invited to join the open-label extension phase of the study, where everyone will receive arimoclomol.

**About Arimoclomol**

Arimoclomol is a small molecule that is taken orally and distributes throughout the body, including the brain. Arimoclomol acts by inducing the cells' own heat shock proteins, a cell-protective system involved in maintaining proper protein folding and quality as well as lysosomal function in the cells. Arimoclomol is safe and well-tolerated, which has been established extensively in Phase I and Phase II clinical trials.

**About Orphazyme**

Orphazyme ApS is a Danish biopharmaceutical company, which develops paradigm-changing medicines for the treatment of genetic diseases. The lead program is in development as a treatment for lysosomal storage diseases. This family of genetic disorders includes Niemann-Pick disease Type C and consists of more than 45 diseases, often affecting children, most of which are often fatal and currently untreatable. Orphazyme is backed by leading European VCs. The strong investor syndicate includes Novo A/S, Sunstone Capital, Aescap Venture, Kurma Partners and Idinvest Partners. For more information, please visit [www.orphazyme.com](http://www.orphazyme.com).

