Clinical update: Orphazyme expands NPC study to three sites in the USA

Orphazyme's clinical programme AIDNPC, investigating the orally administered small molecule arimoclomol as a treatment for Niemann-Pick disease type C, is close to opening in America.

Orphazyme announce today that the participating investigators are: Prof Marc Patterson at Mayo Clinic, Minnesota; Dr Maria Escola of Children's Hospital of Pittsburgh of UPMC and Dr Paul Harmatz at UCSF Benioff Children's Hospital Oakland.

Orphazyme runs the Phase II/III 'NPC-002' trial to investigate the use of arimoclomol as a treatment for Niemann-Pick disease type C. This multi-centre, double-blind, placebo-controlled interventional study, will enroll approximately 46 Niemann-Pick disease type C patients at 19 sites across Europe and the USA. Participants 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.

The interventional AIDNPC programme

The interventional study is a double-blind, controlled study, in which patients will receive either arimoclomol or placebo in addition to their ongoing standard of care treatment for a 12-month period. Patients will be randomized 2:1, which means that two patients will receive arimoclomol for every one patient receiving placebo. Neither the patients nor the treating physicians will know whether they are receiving placebo or arimoclomol.

After the 12-month intervention study every patient will be offered arimoclomol.

The primary endpoint of the study is disease progression rate, which, supported by biochemical readouts, will provide the basis for an application for market authorisation at the end of the study.

Inclusion criteria

Participants in the AIDNPC trial programme must be between 2 and 18 years of age and diagnosed with Niemann-Pick disease type C, presenting with at least one neurological symptom. For information of further inclusion & exclusion criteria, please refer to www.ClinicalTrials.gov (identifier: NCT02612129).

About arimoclomol

Arimoclomol is a new chemical entity with a proven safety record in humans: seven 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.

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 consists of more than 45 diseases, including Niemann-Pick disease type C, and often affect children, most of whom are 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.

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