



Update #8 on the AIDNPC clinical programme *(arimoclomol in treatment of Niemann-Pick disease type C)*

Conference call with patient organizations (28 JULY '16)

SUMMARY

- z 001 Study: Recruitment has reached 33 patients enrolled at 12 sites
- z 002 Study: Recruitment has started — 3 patients enrolled at 1 site
- z 002 Study: Study design and patient considerations



The AIDNPC clinical trial programme consists of two studies:

- *The '-001' Observational Study, where patients can join the programme early and participate in a natural history study. This study is running in several European countries. US patients are not able to join the 001 study.*
- *The '-002' Interventional Study, is currently opening up in countries where approval has been obtained. Here, patients will receive three-times daily oral treatment with the study drug in a placebo-controlled manner. Not all sites are yet able to enroll.*



Recruitment into the 001 Study

At the latest end-of-the-month AIDNPC telephone conference hosted by the sponsor, Orphazyme ApS, the following update on the recruitment into the '-001' and '-002' Studies was presented. Unchanged since late June, a total of 33 patients have been enrolled to date at the following 12 sites:

London, UK	Copenhagen, Denmark	Udine, Italy
Birmingham, UK	Barcelona, Spain	Roma, Italy
Mainz, Germany	Warszawa, Poland	Milano, Italy
München, Germany	Monza, Italy	Bern, Switzerland

For the site in Zaragoza, Spain, the hospital contract is still missing a single signature, and the contract for the Paris and Montpellier sites in France have been readied for signature. For Napoli, Italy, the contract is signed and the site is ready.

Recruitment into the 002 Study

Also unchanged, a total of 3 patients have been enrolled to date at the following site:

Copenhagen, Denmark

Citing summer vacations and awaiting replies from health authorities, no substantial updates on the regulatory front were provided.

To re-cap, the only country/site to approve start of the trial has been Denmark. Here, two patients have rolled over from the '-001' Study, and one patient has enrolled directly into the '-002' Study. In the UK, a new centralized HRA (Health Research Authority) approval procedure has been back-logged and is part of the reasons for a delay in getting the two British sites activated in the '-002' Study. Ethics approval has been granted in Poland, while the health authorities there remain to deliver approval of the trial.

To track enrolment status and obtain detailed contact information for individual clinical sites in the AIDNPC programme, visit www.ClinicalTrials.gov:

- For the '-001' Observational Study, use identifier NCT02435030
- For the '-002' Interventional Study, use identifier NCT02612129

Study design and patient considerations

On the back of questions from patient organisations, Orphazyme provided the following feedback on the '-002' study design and its effects on patients and the clinical sites. Patients will self-administer the study medication three times daily according to instructions; either orally or via feeding tube. The capsule contents can be dissolved in certain foodstuff. The study medication contains either arimocloamol (randomly assigned to 2 for every 3 patients) or placebo. The amount of arimocloamol administered is dependent on the patient's weight. The blinded part of the trial will last for 12 months, after which all patients will be receiving



arimoclomol in an open-label extension of the trial. This extension will continue until A) a conclusion of no effect is reached, or B) until the drug becomes available on the patient's home market.

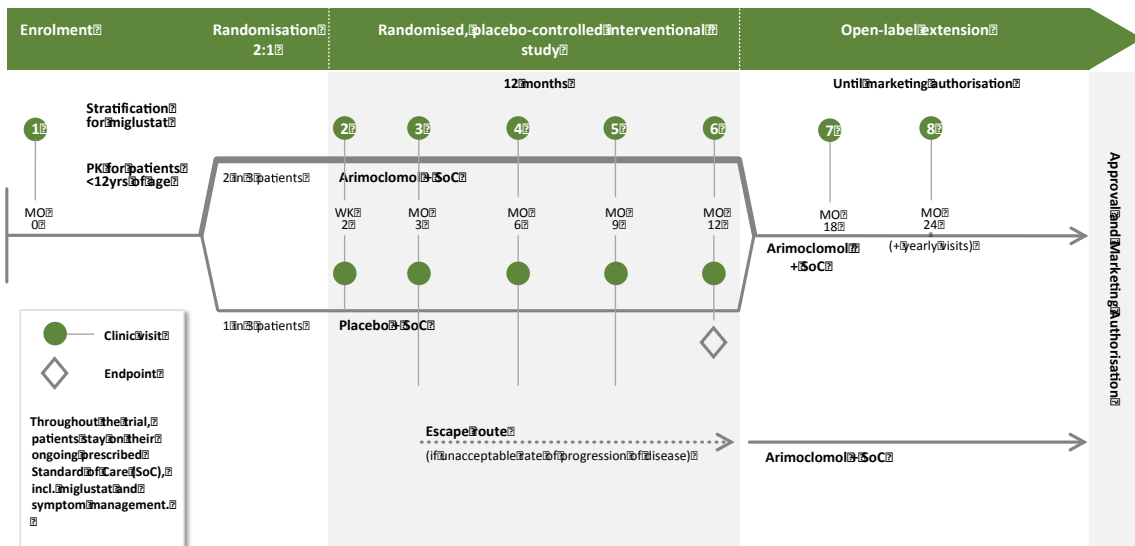
Addressing concerns over the number of patients that any single clinical site can reasonably manage, due to the extensive testing that each patient undergoes at each visit, Orphazyme explained that due to patient visits taking place only every 3 months, the investigators have the flexibility to space visits to accommodate a significant number of patients at any single site.

Aware that many patients do not live close to participating sites, or even in the same country, full travel and lodging support is provided to enrolled patients that are referred to a participating clinical site from another sites where the patient normally is seen by his/her treating physician.

While inclusion and exclusion criteria and other basic information is available at ClinicalTrials.gov, patients interested in participating in the trial should always discuss this with their own doctor, and then meet with the principal investigator at a participating site, to ensure that a full disclosure and professional explanation of all implications of participation in the trial is provided by a team member with a full understanding of the clinical protocol.

The graphic below illustrates the design of the '-002' Study, including number and timing of patients visits to the sites. An escape route is provided for patients that experience an unacceptable rate of progression of the disease.

Study design CT-ORZY-NPC-002



We encourage the sharing of above information with the patient community.

Next call:

The next AIDNPC conference call is schedule for Thursday August 25th at 15h EDT.

Visit the AIDNPC Clinical Programme website: www.AIDNPC.com

