# Update #5 on the AIDNPC clinical programme

*(arimoclomol in treatment of Niemann-Pick disease type C)*

**Conference call with patient organizations (28 APR '16)**

## SUMMARY

- 001 Study: Recruitment progressing well — 32 patients enrolled at 12 sites
- 002 Study: Timing of the '-002' Interventional Study start
- 002 Study: Filing of IND in the US and of CTAs in Europe

***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 open. US patients are not able to join the 001 study.***
- ***The '-002' Interventional Study, where patients will receive three-times daily oral treatment with the study drug in a placebo-controlled manner. This study is not yet recruiting.***

## 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' Study was presented. A total of 32 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 |

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

## Timing of the '-002' Interventional Study start

Due to recent changes in the design of the clinical trial, including the introduction of a placebo group, Orphazyme has informed participating sites that there will be a slight delay.

The delay to the start of the Interventional Study of the AIDNPC Programme (the '002' study) is due to a change of study design and to the associated FDA and EMA regulatory processes that are ensuring all aspects of safety and ethics. The delay will affect only some of the patients in a few countries (UK, Spain and Denmark), and each of these particular patients are now being informed by the relevant Principal Investigators.

### The AIDNPC Clinical Programme

The AIDNPC clinical programme will appropriately assess the therapeutic response of Arimoclomol in Niemann-Pick disease type C. Arimoclomol is a small-molecule inducer of heat shock proteins. It is an orally available experimental drug that readily crosses the blood-brain barrier.

This initial phase of the AIDNPC Programme is an Observational Study (the '001' study), lasting for a period of up to six months. Following this, the second phase of the AIDNPC Programme, the Interventional Study (the '002' study), will be open to all patients that complete the Observational Study and to new patients that fulfil the in- and exclusion criteria.

In the Observational Study, two skin biopsies will be taken at entry to the study, at beginning of the '002' study, and, thereafter, biopsies will only be required after 6 and 12 months.

The Interventional Study is a prospective, randomised, double-blind, placebo controlled therapeutic study in patients with confirmed diagnosis of Niemann-Pick disease type C (NP-C). This will be a multi-centre clinical programme involving clinical centres in EU. Two thirds of the participating patients will receive arimoclomol and one third the placebo — nobody will know who will actually receive the active drug or placebo, as this process is randomised (by chance) and blinded.

At the start of the Interventional Study, patients below 12 years of age will have a cannula fitted to enable small blood samples (6 in total) to be easily taken over a period of eight hours, in order to assess the concentration of the drug in the blood.

## Regulatory process for the 002 Study

Orphazyme reported that the regulatory process relating to the 002 Study is progressing well and according to plan.

Orphazyme intends to file an IND with the FDA, permitting use of the same protocol, and permitting that the combined generated data be used for registration of the medicine with both the EMA and the FDA. Once the US IND is open, Orphazyme plans to have a couple of sites in the US identified and participating in the 002 Study. Several sites are currently being evaluated, with the help of Orphazyme's Scientific Advisory Board and the NNPDF.

In both the US and Europe, the regulatory process allows for the competent authorities to pose questions to ensure compliance with all aspects of safety and ethics, which could affect the timeline site-by-site and country-by-country.

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

## Next call:

The next AIDNPC conference call is schedule for Thursday May 26th at 15h EDT.

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