



Update #10 on the AIDNPC clinical programme *(arimoclomol in treatment of Niemann-Pick disease type C)*

Conference call with patient organizations (22 SEPTEMBER '16)

SUMMARY

- Z** 002 Study: Recruitment into the 002 Interventional Study
- Z** 002 Study: Scientific paper on arimoclomol published



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 002 Interventional 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. In the '-001' Study, 2 more patients enrolled in the last weeks (1 in Rome and 1 in Zaragoza) for a total of 35 patients that now have enrolled to date at 12 European sites. In the '-002' Study, still a total of 3 patients have been enrolled to date at the following site:

Copenhagen, Denmark

The process of getting the go-ahead from all required regulators and hospital committee in any of the countries and for any of the sites is a multi-step process that often involve back-and-forth with questions to satisfy the individual boards. Varying calendars and deadline schedules for these also play into the timelines.

Orphazyme reports that it is progressing well with all the authorities and announced last week at the 23rd Family Conference in Bedfordshire, UK, that expectations are that the Birmingham site will be opened on September 28th and that the GOSH site in London on October 13th. The company further expects to have all sites, including at least two new sites in the US, to open for access to the '-002' Study during the remainder of the year, allowing patients currently in the '-001' Study to transfer and new patients to enrol.

To track enrolment status and obtain detailed contact information for individual clinical sites in the AIDNPC programme, visit www.ClinicalTrials.gov and use identifier [NCT02612129](https://clinicaltrials.gov/ct2/show/study/NCT02612129).

Scientific paper on arimoclomol published

Orphazyme is pleased –after an extended review process at the publisher– to now be able to share a more in depth scientific explanation for the rationale behind the use of arimoclomol as a potential treatment for Niemann-Pick type C disease. The article, published in the prestigious Science Translation Medicine, is a supplemental piece of information helping patients and families in making decisions about potential new treatment options and any participation in clinical trials. Go to <http://stm.sciencemag.org/content/8/355/355ra118> to read or get a PDF copy.

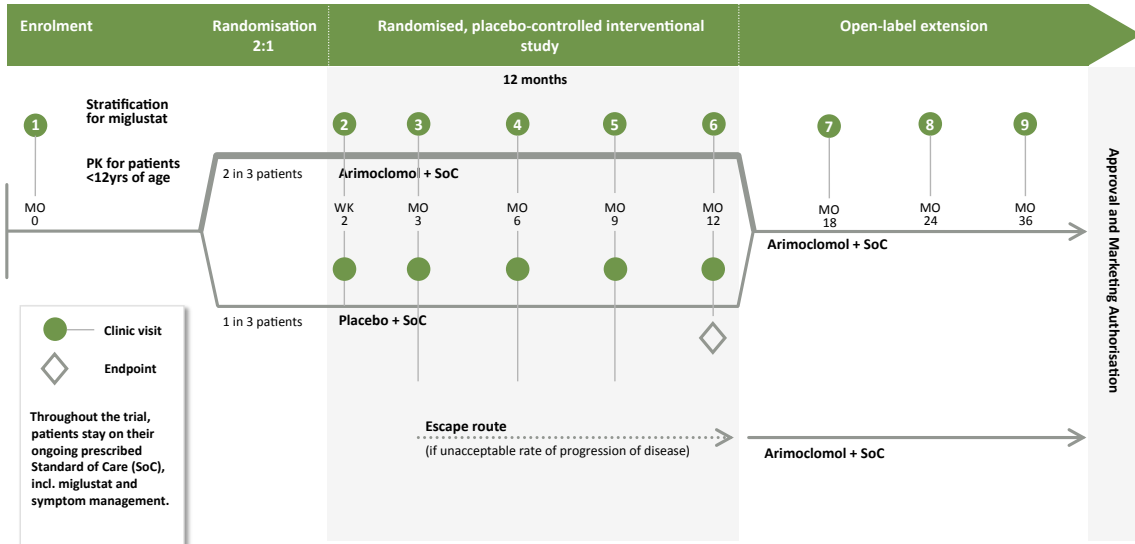


Image from <http://stm.sciencemag.org/> on 8 September 2016

Clinical design overview

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 call is schedule for Thursday October 27th at 15h EDT.

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

