



## Update #2 on the AIDNPC clinical programme *(arimoclomol in treatment of Niemann-Pick disease type C)*

Conference call with patient organizations (14 JAN '16)

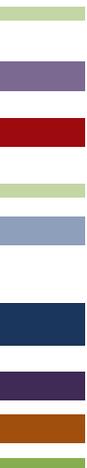
### SUMMARY

- Polish patient organization joins the TC series
- 001 Study: Observation Study is open and many sites now recruiting
- 001 Study: Enrolment consideration and study information
- Meeting with the FDA on January 12<sup>th</sup>



***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 patient will receive three-times daily oral treatment with the study drug in a placebo-controlled manner. This study is not yet recruiting.***



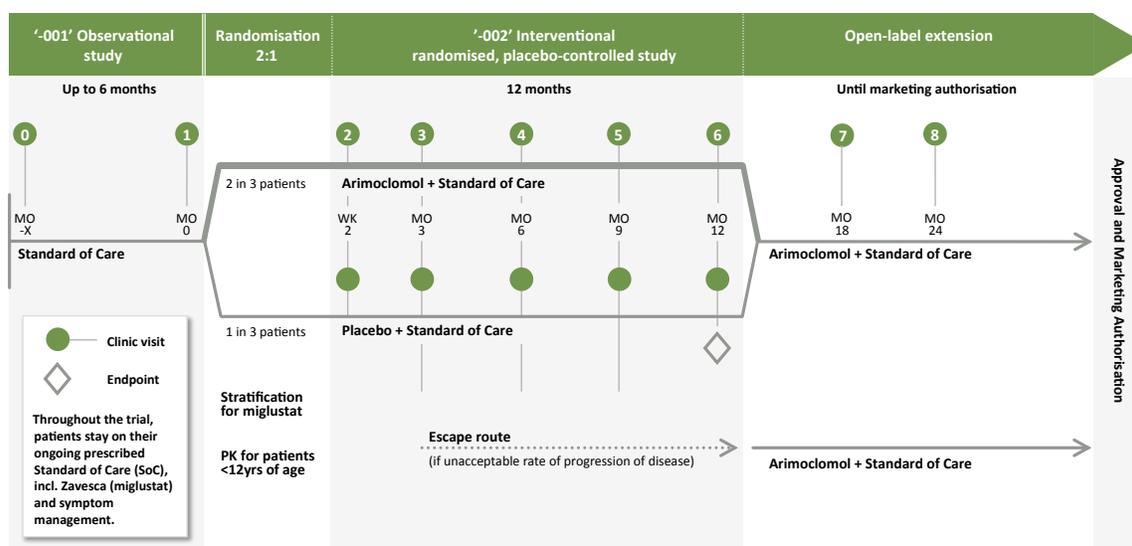
## Polish patient organization joins the TC series

We are pleased to have the Polish NPC Association (Stowarzyszenia Chorych na Niemann-Picka) join this TC series. The organisation is very active and is key the success of recruiting Polish patients to the Warsaw site.

## The '-001' Observational Study is open

Currently, the Observational Study (the '001' Study) is open and many sites (see above) are now recruiting for this study. The protocol for the Interventional Study (the '002' Study) is currently being filed with authorities, and we expect the first clinical sites to be ready to open for the 002 study by Summer. The best way to track the current enrolment status and contact information of individual clinical sites in the AIDNPC programme is to follow the entry for the '001' study at [www.ClinicalTrials.gov](http://www.ClinicalTrials.gov), using identifier NCT02435030.

Below is a graphic illustrating the design and time line of the full AIDNPC clinical trial programme:



## Many sites now recruiting

The following 8 sites (out of the 16 total) are activated and recruiting patients. A total of 16 patients have now been enrolled (two more were enrolled after the TC). Sites in France, Italy and Switzerland remain delayed as a result of unexpectedly lengthy contract negotiations and/or local ethics committee deliberations

London, UK  
Birmingham, UK  
Mainz, Germany

Copenhagen, Denmark  
Barcelona, Spain  
Warszawa, Poland

Monza, Italy  
Udine, Italy

## Enrolment consideration



As was discussed during the conference call, enrolment in the '-001' Observational Study is not obligatory for the opportunity to join the '-002' Interventional Study. That is, there is an option for patients to enter the therapeutic Interventional Study directly (also on [www.ClinicalTrials.gov](http://www.ClinicalTrials.gov): NCT02612129), without first having been enrolled in the Observational Study. Patients and physicians must keep in mind that the patients joining the '-001' study already now will have preference to enter the 002 study ahead of the patients choosing to wait for the '-002' study to open in their country.

### **Study information**

Orphazyme publishes as much information as possible to permit patients and physicians to make their decision about participation in the AIDNPC programme. The Interventional Study (the '002' Study) is described on [www.ClinicalTrials.gov](http://www.ClinicalTrials.gov): NCT02612129).

For UK patients only, the [www.AIDNPC.com](http://www.AIDNPC.com) web site also describes the study.

### **Meeting with the FDA**

Orphazyme had a meeting with the FDA on January 12th for the purpose of preparing an IND. One goal is to align EU and US regulators to conduct a 'global' trial with clinical sites on both sides of the Atlantic, allowing patients' data to be combined for analysis and approval. The meeting with the FDA was very positive and has brought the company closer to its goal of including a US arm in the AIDNPC programme, ultimately serving also American NP-C patients and their families. The US agency's attitude was supportive, showing interest in and willingness to support Orphazyme's initiative for an EU-US coordinated clinical programme in NP-C.

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

### **Next call:**

The next AIDNPC conference call is schedule for Thursday February 25<sup>th</sup> at 15h EDT.

