# Update #1 on the AIDNPC clinical programme

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

**Conference call with patient organizations (26 NOV '15)**

## SUMMARY

- 001 Study: Status information and general program information
- Anonymous patient contact
- 001 Study: Sites now recruiting
- Enrolment considerations
- Compassionate Use program not in the plans

***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.***

## Status information — '-001' Study:

Orphazyme informs that best way to track the enrolment status and contact information of individual clinical sites in the AIDNPC programme is to follow the entry for the Observational Study (the '001' study) at www.ClinicalTrials.gov (use identifier NCT02435030).

## General program information — For UK Patients

A web site dedicated to the AIDNPC clinical programme (www.AIDNPC.com) has been created for UK patients and has UK Ethics Committee approval. For inspiration, Orphazyme recommend this site to the patient organisations, but the company stresses that the site cannot be promoted to patients outside the UK. The company is more than willing to aid in providing additional and specific information and support (in any language) to any of the organisations requesting this.

## Anonymous patient contact

Permitting direct contact to Orphazyme by e-mail, an e-mail address has been set up where patients or physicians can write —while being anonymous to Orphazyme— with specific questions (either personal questions or on behalf of a group of patients). The address is: 'orphazymenpc@simbecorioncro.com'.

## Sites now recruiting — '-001' Study:

The following 8 sites (out of the 16 total) are activated and recruiting patients.

| | | |
|---|---|---|
| London, UK | Copenhagen, Denmark | Monza, Italy |
| Birmingham, UK | Barcelona, Spain | Udine, Italy |
| Mainz, Germany | Warszawa, Poland | |

## Enrolment consideration — '-001' and '-002'

Orphazyme told that there is an option for patients to enter the therapeutic Interventional Study directly (i.e., into the '002' Study; also on www.ClinicalTrials.gov: NCT02612129), and without first having been enrolled in the '001' Study. The company stresses that **it is important for patients** to keep in mind that enrolment in the AIDNPC programme is competitive. That is, all patients already enrolled in the '001' Study will be the first to be offered enrolment into the '002' Study. After that, and *only* if there is room for more patients in the '002' Study, will not-already-enrolled patients be able to enrol directly into the '002' Study. It is also important to keep in mind that not all countries are equally fast in approving new clinical studies.

Thus, in the countries where the '-002' Study will be late in getting local approval, patients waiting for '-002' Study to start in such countries risk that patients from

other countries could enrol ahead of them, and therefore prevent their own direct enrolment into Study '002'. Overall, it could be viewed as an advantage for patients to enrol into the '001' Study.

Participation in the '001' Observational Study provides important information about the disease severity and progression of the disease as well as the appropriateness of the selected assessment tools (e.g., NPC Disease Severity Scores and Quality-of-Life questionnaires). In addition, the '001' Study allows for an important validation of the unique biomarker assays developed specifically for the AIDNPC program. One of the possible outcomes of this biomarker research is the availability of a faster and simpler method in a small blood sample for the initial diagnosis (as compared to the current philipin staining of skin biopsies).

## Compassionate Use not in the plans

Orphazyme appreciates the strong interest in the possibility for 'Compassionate Use' and 'Named Patient Access' outside the current protocols for arimoclomol. However, it is important to keep in mind that, while the company has seen encouraging results from preclinical studies, it still needs to obtain results from a properly controlled clinical study to evaluate safety and efficacy in NPC patients. Not until such supportive results are available would it be advisable to embark on a compassionate-use program.

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

## Next call:

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