Update #7 on the AIDNPC clinical programme

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

Conference call with patient organizations (29 JUNE '16)

SUMMARY

- 001 Study: Recruitment has reached 33 patients enrolled at 12 sites
- 002 Study: Recruitment has started — 3 patients enrolled at 1 site
- 002 Study: "Study May Proceed" letter from the FDA
- 002 Study: Grant of 'Fast Track' designation by the FDA

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' Study was presented. 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

Orphazyme ApS has the following update on the recruitment into the '-002' Study. A total of 3 patients have been enrolled to date at the following site:

Copenhagen, Denmark

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.

Clinical Trial Applications were also been submitted in all other European countries where the trial takes place, and the authorities in some of these have responded with questions. It should be noted that the summer vacation time is likely to negatively affect the speed of approvals in several countries. 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. Orphazyme is working tirelessly to expedite the approvals in all countries and sites.

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 May Proceed" letter from the FDA

Orphazyme on June 15th announced its receipt of a positive opinion from the FDA in response to its submission of an IND (Investigation New Drug) application. As a result, Orphazyme is currently working to establish at least two clinical sites in the US that will be participating in the '-002' interventional study. As soon as Orphazyme is able to announce the names of the US sites and principal

investigators, it will also ensure that the NNPDF (the US patient support organization) will be able to support the local patient community in getting answers to questions.

Grant of 'Fast Track' designation by the FDA

June 27th Orphazyme announced that the FDA has positively reviewed the company's application of the '-002' Study in the AIDNPC clinical development programme and found that it meets the criteria for 'Fast Track' designation.

'Fast Track' is a designation by the US Food and Drug Administration of an investigational drug for expedited review to facilitate development of drugs, which treat a serious or life-threatening condition and fill an unmet medical need.

A drug development programme that receives Fast Track designation is eligible for 1) more frequent meetings and written correspondence with FDA to discuss the drug's development plan; 2) 'Accelerated Approval' if the drug demonstrates an effect on a surrogate or intermediate endpoint reasonably likely to predict clinical benefit; 3) 'Rolling Review', which means that a drug company can submit completed sections of its New Drug Application (NDA) for review by FDA, rather than waiting until all sections of the application are completed before the entire application can be submitted and reviewed.

Importantly, the designation also signifies that FDA is in agreement with the development plan for arimoclomol, which implies that an approval is more likely should the clinical trial result in positive results for efficacy.

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

Next call:

The next AIDNPC conference call is schedule for Wednesday July 28th at 15h EDT.

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