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**Update #14 on the AIDNPC clinical programme**

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

**Conference call with patient organizations (23 FEB ’17)**

SUMMARY

* 002 Study: Recruitment into the 002 Interventional Study
* Orphazyme at the WORLD conference
* New TC meeting frequency once study is fully recruited
* 002 Study: Clinical design overview

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***The AIDNPC clinical trial programme consists of two studies:***

* ***The ‘-001’ Observational Study is no longer recruiting.***
* ***The ‘-002’ Interventional Study, is currently opening 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 ‘-002’ Study was presented. Over the last month, 3 more patients have enrolled in ‘-002’ Study, for a the total of 25 patients now enrolled to date at the 8 European sites that so far have opened for ‘-002’ enrolment. The two US sites have opened for enrolment. Of these partients, 18 patients have rolled over from the ‘-001’ Study, while 7 are enrolled directly into ‘-002’. Awaiting to roll over from the ‘-001’ Study is 9 more patients at sites where the last legal and regulatory hurdles are being addressed. To date, the following sites have been activated for the ‘-002’ Study:

Copenhagen, Denmark

Birmingham, UK

Great Ormond St, UK

Mainz, Germany

Münich, Germany

Paris, France

Barcelona, Spain

Warsaw, Poland

Rochester, MN-USA

Oakland, CA-USA

The total number of patients planned to be enrolled is 46.

Orphazyme informs that it continues to work to getting all sites ready and fully recruited as soon as possible, and the company expects all patients to have been recruited by early Spring.

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

Orphazyme presented at the WORLD conference (San Diego)

Dr. Thomas Kirkegaard Jensen, Orphazyme’s CSO, presented at the 13th Annual WORLD Symposium in San Diego US (13/2–17/2 2017), and the company was pleased to meet and interact with stakeholder from all over the world.

New TC meeting frequency once study is fully recruited

With the AIDNPC study close to being fully recruited, Orphazyme proposed a change of frequency for the now-monthly teleconference calls providing updates. Once the last patient has been enrolled, the updates could be provided on a quarterly basis. The company invites the patient organisations to share their views on the proposed change in TC frequency.

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.



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

Next call:

The next AIDNPC call is schedule for Thursday March 30th 2017 at 15h EDT.

Visit the AIDNPC Clinical Programme website: [www.AIDNPC.com](http://www.AIDNPC.com)