ORPHAZYME

Press Release:

FDA gives clearance to arimoclomol study for treatment of Niemann-Pick disease type C

Copenhagen, Denmark, June 06, 2016; Orphazyme has received a "Study May Proceed" letter from the FDA on Orphazyme's protocol for arimoclomol as a new treatment for Niemann-Pick disease type C (NP-C), the AIDNPC intervention study

This makes the US IND[1] effective, complementing the corresponding European CTAs[2] submitted for the same protocol.

Orphazyme runs an international, multi-centre, double-blind intervention study enrolling approximately 46 NP-C patients to be treated with three daily oral doses of arimoclomol or placebo. The objective of the study is to determine the efficacy and safety of arimoclomol in the treatment of NP-C.

Orphazyme is working to include two distinguished US clinical sites in the AIDNPC clinical programme. AIDNPC is a global programme, and now, with the addition of two sites in the US, Orphazyme will enrol patients into the interventional study at 18 sites in nine countries.

Christina Guldberg, Director of Clinical Operations at Orphazyme, says: "*We have more than 30 patients enrolled in Europe in the initial observational part of the study programme. We will start the interventional part and treat the first patients with arimoclomol in June. We are very pleased that the IND is now open so that we can start the preparations for welcoming US patients directly into the interventional study*".

"There is high unmet need for new therapies to treat sufferers of NP-C, a debilitating and life-threatening disease. With this trial, we continue our work to help the patients with a drug that has an oral route of administration and readily crosses the blood brain barrier in order to treat this disease," says Christine í Dali MD, VP of Clinical Development.

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About the AIDNPC Clinical Programme
The AIDNPC programme consists of two studies: An Observational Study followed by an Interventional Study. The Observational Study is a natural history study that has only been conducted in Europe. In the Interventional Study, European and US NP-C patients will receive oral arimoclomol or placebo three times daily for 12 months. All patients will subsequently be invited to join the open-label extension phase of the study, where everyone will receive arimoclomol.

About Arimoclomol
Arimoclomol is a small molecule that is taken orally and distributes throughout the body, including the brain. Arimoclomol acts by inducing the cells' own heat shock proteins, a cell-protective systems involved in maintaining proper protein folding and quality as well as lysosomal function in the cells. Arimoclomol is safe and well-tolerated, which has been established extensively in Phase I and Phase II clinical trials.

About Orphazyme
Orphazyme ApS is a Danish biopharmaceutical company, which develops paradigm-changing medicines for the treatment of genetic diseases. The lead program is in development as a treatment for lysosomal storage disease. This family of genetic disorders includes NP-C and consists of more than 45 diseases, often affecting children, most of which are currently untreatable and often fatal. Orphazyme started enrolling in AIDNPC in the fall of 2015, and will start dosing patients in a double blinded placebo-controlled Phase 3 study in June 2016. Orphazyme is backed by leading European VCs. The strong

investor syndicate includes Novo A/S, Sunstone Capital, Aescap Venture, Kurma Partners and Idinvest Partners. For more information, please visit www.orphazyme.com,

[1] IND: Investigational New Drug Application (USA)
[2] CTA: Clinical Trial Application (Europe)