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Press Release:

FDA grants Fast Track designation for the Phase III clinical investigation of arimoclomol as a treatment of Niemann-Pick disease type C

Copenhagen, Denmark, June 27, 2016. Orphazyme ApS announced today that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for the investigation of arimoclomol intended for the treatment of Niemann-Pick disease type C. The FDA's Fast Track program is a process designed to facilitate the development, and expedite the review of drugs to treat serious conditions and fill an unmet medical need. The purpose is to get important new drugs to the patient earlier.

"*The Fast Track designation is really good news for everyone involved in the AIDNPC trial, not least the sufferers of Niemann-Pick disease type C who deserve that every effort is made to expedite development of promising new therapies,*" says Orphazyme ApS CEO Anders Hinsby.

The Fast Track designation comes three weeks after the FDA gave a positive review of the AIDNPC Investigational New Drug (IND) application, allowing the trial to proceed at full speed. The opening of at least two clinical sites in the US is now in preparation. Orphazyme expects to announce the participating clinical sites in the near future.

For further information:
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About the AIDNPC Clinical Programme
AIDNPC consists of two clinical studies: An Observational Study followed by an Interventional Study. The Observational Study was only conducted in Europe and is nearing completion. European and US patients are now being enrolled directly into the Interventional Study. During the Interventional Study patients will receive orally administered arimoclomol or placebo at a 2:1-ratio, meaning that two patients will receive arimoclomol for every patient receiving placebo, three times daily for 12 months. Every patient 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 system 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 diseases. This family of genetic disorders includes Niemann-Pick disease type C and consists of more than 45 diseases, often affecting children, most of whom are currently untreatable. 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,