Company announcement
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Arimoclomol for NPC receives Rare Pediatric Disease Designation

Copenhagen, Denmark, January 19, 2018 – Orphazyme A/S, a Danish biotech company listed on Nasdaq Copenhagen (TICKER: ORPHA.CO), with a late-stage, orphan-drug pipeline, today announced that arimoclomol has been granted rare pediatric disease designation by the US Food and Drug Administration (FDA) for the treatment of Niemann-Pick disease Type C (NPC).

NPC is a genetic disease affecting around 1 in 120,000 newborns. Although the time of symptoms onset is variable, NPC is most often diagnosed in childhood and adolescence. Progressive neurological pathology is the hallmark of NPC and is responsible for disability and premature death in most patients. Arimoclomol, an orally available small molecule, is currently being investigated in a clinical Phase II/III trial as a potential treatment for NPC.

Anders Hinsby, CEO of Orphazyme, says: *"We are very pleased with the FDA's continued support for the development of therapies for children with rare diseases. Arimoclomol has now been granted orphan drug, fast track, and rare pediatric disease designations by the FDA, which underpin the high unmet need in NPC, providing acknowledgement of the arimoclomol program. We look forward to continue working closely with the FDA in our effort to provide a treatment option for patients suffering from NPC."*

With the rare pediatric disease designation, the FDA provides incentive for the development of treatments for rare pediatric diseases. A drug qualifies for this category if the entire prevalence of the disease in the US is below 200,000 and if more than 50% of the patients are between 0-18 years.

If a drug candidate with a rare pediatric disease designation receives marketing approval in the US, the FDA will, if certain criteria are met, issue a Priority Review Voucher to the sponsor company. This voucher can be redeemed to provide Priority Review of a subsequent marketing application for a different product.

For additional information, please contact

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About NPC
Niemann-Pick Type C (NPC) is a rare, genetic, and progressive disease that impairs the body's ability to move cholesterol and other lipids inside the cells. This results in the accumulation of lipids within the body's tissue, including the brain tissue, causing damage to the affected areas. The symptoms upon the onset of NPC vary from fatality during the first months after birth to a progressive disorder not diagnosed until adulthood. The disease affects neurologic and psychiatric functions as well as various internal organs.

About arimoclomol

Arimoclomol is a new chemical entity with a very favorable safety and tolerability record in humans: Seven Phase I clinical studies have been conducted in healthy volunteers. Arimoclomol is administered orally, three times daily, and can be easily dissolved in liquids or food for best possible patient comfort and compliance. Orphazyme has obtained orphan drug designation for arimoclomol in NPC, ALS, and sIBM from both the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

About Orphazyme A/S

Orphazyme is a Danish biotech company listed on Nasdaq Copenhagen (ticker: ORPHA.CO) with a late-stage drug pipeline, developing new treatment options for orphan protein-misfolding diseases. The company was founded in 2009 based on early scientific discovery in heat-shock proteins. The company is headquartered in Copenhagen and currently has 35 employees. The lead candidate arimoclomol is in development as a potential treatment for four orphan diseases; two neuromuscular diseases, sporadic Inclusion Body Myositis (sIBM) and Amyotrophic Lateral Sclerosis ("ALS"), and two lysosomal storage diseases, Niemann-Pick Type C (NPC) and Gaucher disease. For more information, please visit www.orphazyme.com.