Company announcement
No. 73/2020
Inside Information

Orphazyme A/S
Ole Maaløes Vej 3
DK-2200 Copenhagen N

www.orphazyme.com
Company Registration No. 32266355

Orphazyme provides regulatory update on arimoclomol for NPC

Copenhagen, Denmark – December 27, 2020 – Orphazyme A/S (ORPHA.CO; ORPH), a late-stage biopharmaceutical company pioneering the Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, announced today the U.S. Food and Drug Administration (FDA) has extended the review period of the New Drug Application (NDA) for arimoclomol for the treatment of Niemann-Pick Disease Type C (NPC) by a standard extension period of three months. This extension is necessary for the FDA to complete its review. The updated Prescription Drug User Fee Act (PDUFA) target action date is June 17, 2021.

The FDA has confirmed the NDA remains under Priority Review, and the extension does not impede eligibility for a Pediatric Rare Disease Priority Review Voucher. The FDA grants Priority Review to applications for potential therapies that, if approved, could offer a significant improvement in safety or effectiveness, diagnosis, or prevention of serious conditions.

"*Orphazyme is working closely with the FDA to support the final review of the new drug application for arimoclomol,*" said Molly Painter, US President, Orphazyme. "*There is significant unmet medical need for the treatment of NPC, and we are committed to bringing arimoclomol to patients in the U.S. and Europe as soon as possible.*"

"*We have responded to all FDA information requests and submitted all outstanding information regarding the arimoclomol NDA for NPC,*" said Thomas Blaettler, Chief Medical Officer, Orphazyme. "The Phase 3 trials for Amyotrophic Lateral Sclerosis and Inclusion Body Myositis remain on track for read-out in the first half of 2021 and we look forward to providing an update on our progress."

Arimoclomol has received FDA Fast-Track and Breakthrough Therapy Designations for NPC, as well as Orphan Drug and Rare Pediatric Disease Designations. If approved in the US, arimoclomol will be the first and only approved medicine for NPC, a rare, relentlessly progressive, neurodegenerative disease with an estimated incidence of one in 100,000 live births. In November 2020, the company also submitted a Marketing Authorisation Application to the European Medicines Agency for arimoclomol in NPC.

For additional information, please contact

Orphazyme A/S

Copenhagen: Anders Vadsholt, CFO +45 28 98 90 55
Chicago: Molly Carey Poarch +1 773-770-6888

About Orphazyme A/S
Orphazyme is a late-stage biopharmaceutical company pioneering the Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases. The company is harnessing amplification of Heat-Shock Proteins (or HSPs) to develop and commercialize novel therapeutics for diseases caused by protein misfolding, protein aggregation, and lysosomal dysfunction, including lysosomal storage diseases and neuromuscular degenerative diseases. Arimoclomol, the company's lead candidate, is in clinical development for four orphan diseases: Niemann-Pick disease Type C (NPC), Amyotrophic Lateral Sclerosis (ALS), Inclusion Body Myositis (IBM) and Gaucher disease. Orphazyme is headquartered in Denmark and has operations in the U.S., Switzerland, France and Germany. Orphazyme's shares are listed on Nasdaq U.S. (ORPH) and Nasdaq Copenhagen (ORPHA.CO).

About arimoclomol
Arimoclomol is an investigational drug candidate that amplifies the production of Heat-Shock Proteins (HSPs). HSPs can rescue defective misfolded proteins, clear protein aggregates, and improve the function of lysosomes. Arimoclomol is administered orally, crosses the blood-brain barrier, and has now been studied in seven phase 1, four phase 2 and one pivotal phase 2/3 trial. Arimoclomol is in clinical development for NPC, Gaucher Disease, IBM, and ALS. Arimoclomol has received orphan drug designation (ODD) for NPC, IBM, and ALS in the US and EU. Arimoclomol has received fast-track designation (FTD) from the U.S. Food and Drug Administration (FDA) for NPC, IBM and ALS. In addition, arimoclomol has received breakthrough therapy designation (BTD) and rare-pediatric disease designation (RPDD) from the FDA for NPC.

About Niemann-Pick disease Type C (NPC)
Niemann-Pick disease Type C (NPC) is a rare, genetic, progressively debilitating, and often fatal neurovisceral disease. It belongs to a family known as lysosomal storage diseases and is caused by mutations leading to defective NPC protein. As a consequence, lipids that are normally cleared by the lysosome accumulate in tissues and organs, including the brain, and drive the disease pathology. We estimate the incidence of NPC to be one in 100,000 live births and the number of NPC patients in the United States and in Europe to be approximately 1,800 individuals. There are no approved treatments for NPC in the U.S.

Forward-looking statement
This company announcement may contain certain forward-looking statements. Although the Company believes its expectations are based on reasonable assumptions, all statements other than statements of historical fact included in this company announcement about future events are subject to (i) change without notice and (ii) factors beyond the Company's control. These statements may include, without limitation, any statements preceded by, followed by, or including words such as "target," "believe," "expect," "aim," "intend," "may," "anticipate," "estimate," "plan," "project," "will," "can have," "likely," "should," "would," "could", and other words and terms of similar meaning or the negative thereof. Forward-looking statements are subject to inherent risks and uncertainties beyond the Company's control that could cause the Company's actual results, performance, or achievements to be materially different from the expected results, performance, or achievements expressed or implied by such forward-looking statements. Except as required by law, the Company assumes no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in the forward-looking statements, even if new information becomes available in the future.