Company announcement
No. 50/2020

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

www.orphazyme.com
Company Registration No. 32266355

Orphazyme announces U.S. FDA acceptance and Priority Review of New Drug Application for arimoclomol for Niemann-Pick disease Type C

- *If approved, arimoclomol would become the first approved therapy in the U.S. for people with Niemann-Pick disease Type C (NPC)*
- *FDA has set target PDUFA action date of March 17, 2021*

Copenhagen, Denmark, September 16, 2020 – Orphazyme A/S (ORPHA.CO), a late-stage biopharmaceutical company pioneering the Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, today announced that the U.S. Food and Drug Administration (FDA) has accepted, with Priority Review, the company's New Drug Application (NDA) for arimoclomol for the treatment of NPC.

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. This designation shortens the review period from the standard 10 months to six months from the acceptance of the NDA. The FDA has set a target action date of March 17, 2021 under the Prescription Drug User Fee Act (PDUFA) and has indicated that it does not currently plan to hold an advisory committee meeting to discuss the application.

Kim Stratton, Chief Executive Officer, Orphazyme, said, "*The filing acceptance marks a significant milestone in our journey towards our first potential approval of arimoclomol for NPC, a devastating and often fatal disease for which there is no approved therapy in the U.S. We look forward to collaborating with the FDA as they complete their review of this NDA to address the unmet medical need in NPC, and meanwhile are working to expand our U.S. activities in preparation for potential commercial availability next year.*"

"Acceptance of the arimoclomol filing by the FDA is another major step forward in the effort to bring a treatment to people affected by Niemann-Pick disease Type C and is a reflection of the commitment of the entire community coming together to support promising research," said Joslyn Crowe, Executive Director of the National Niemann-Pick Disease Foundation.

Arimoclomol has been granted FDA Fast Track and Breakthrough Therapy Designations for NPC, as well as Orphan Drug and Rare Pediatric Disease Designations. Orphazyme expects to file a Marketing Authorisation Application (MAA) with the European Medicines Agency (EMA) for arimoclomol in NPC in H2 2020.

For additional information, please contact

Orphazyme A/S

Anders Vadsholt, CFO +45 28 98 90 55

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) in order 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), sporadic Inclusion Body Myositis (sIBM) and Gaucher disease. Orphazyme is headquartered in Denmark and has operations in the U.S. and Switzerland. Orphazyme's shares are listed on 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 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, sIBM, and ALS.

About Niemann-Pick disease Type C
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, including the FDA's target action date of March 17, 2021 under the PDUFA, the FDA's statement that it is currently not planning to hold an advisory committee meeting for the Company's NDA application for arimoclomol for NPC and planned expansion in the U.S. and timing of potential commercial availability. 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.