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Orphazyme completes rolling submission of New Drug Application to U.S. FDA for arimoclomol for Niemann-Pick disease Type C

- *Company expects to submit MAA for arimoclomol for NPC to European Medicines Agency in the second half of 2020*
- *Arimoclomol progressing through registrational trials in two additional indications - ALS and sIBM*

Copenhagen, Denmark, July 20, 2020 – Orphazyme A/S (ORPHA.CO), a late-stage biopharmaceutical company pioneering the Heat-Shock Protein response in order to develop and commercialize novel therapeutics for the treatment of neurodegenerative orphan diseases, today announced that it has completed the rolling submission of its New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for its investigational therapy arimoclomol for the treatment of Niemann-Pick disease Type C (NPC).

Arimoclomol has received FDA Fast-Track and Breakthrough Therapy Designations for NPC, as well as Orphan Drug and Rare Pediatric Disease Designations. The rolling NDA submission allowed Orphazyme to submit critical portions of its NDA to the FDA as they were completed. Following completion of submission, the FDA has up to 60 days to determine whether to accept the application for review.

"*Data from the randomized, controlled, clinical trial of arimoclomol for Niemann-Pick disease Type C support the positive effect of this agent in stabilizing neurologic progression of the disease, specifically in subgroups of patients over four years of age, and in those also taking miglustat*," said Marc Patterson, MD, Professor of Neurology, Pediatrics and Medical Genetics, Mayo Clinic Children's Center in Rochester, MN. "*The data show clear evidence of target engagement, specifically an elevation of Heat-Shock Protein levels, with encouraging changes in biomarkers of excess lipid storage. These data support the role of enhanced Heat-Shock Protein 70 expression in Niemann-Pick disease Type C and may have applications in other lysosomal disorders*."

"*The NPC patient community's need for disease-modifying therapy could not be more urgent as there are no FDA-approved treatments for this malicious disease*," said Justin Hopkin, MD, Board Chair of the National Niemann-Pick Disease Foundation (NNPDF). "*On behalf of the NNPDF and the Niemann-Pick community, we would like to extend our heartfelt thanks to Orphazyme for their tireless commitment to improving the lives of NPC patients and congratulate them in their completed NDA submission for arimoclomol to the FDA as a treatment for NPC*."

Orphazyme expects to submit a Marketing Authorisation Application (MAA) with the European Medicines Agency (EMA) for arimoclomol in NPC in the second half of 2020.

Kim Stratton, Chief Executive Officer of Orphazyme, said, "*The rapid completion of the rolling submission of arimoclomol in NPC brings us one step closer to the potential approval of a new treatment option that can address a substantial unmet need for patients with NPC. We look forward to working together with the FDA as they review our application. If approved, arimoclomol would be the only FDA-approved treatment for NPC in the U.S. This exciting step adds to the momentum at Orphazyme, as we make progress in our commercial preparations in the U.S. and other major markets ahead of potential approval in NPC, and as we progress arimoclomol through clinical trials in additional indications with unmet medical needs – Amyotrophic Lateral Sclerosis (ALS), sporadic Inclusion Body Myositis (sIBM), and Gaucher disease."*

For additional information, please contact

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About Orphazyme A/S
Orphazyme is a biopharmaceutical company pioneering the Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases. The company is focused on developing therapies for diseases caused by protein misfolding, protein aggregation, and lysosomal dysfunction. Arimoclomol, the company's lead candidate, is in clinical development for four orphan diseases: Niemann-Pick disease Type C (NPC), Gaucher Disease, sporadic Inclusion Body Myositis (sIBM), and Amyotrophic Lateral Sclerosis (ALS). The Denmark-based company is listed on Nasdaq Copenhagen (ORPHA.CO). For more information, please visit www.orphazyme.com.

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 or naso-gastrically, 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, sIBM, and ALS. Arimoclomol has received orphan drug designation (ODD) for NPC, sIBM, and ALS in the U.S. and EU. Arimoclomol has received fast-track designation (FTD) from the U.S. Food and Drug Administration (FDA) for NPC, sIBM 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
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, including whether arimoclomol for the treatment of NPC is approved by the FDA as well as the timing of the MAA submission for arimoclomol for NPC and the clinical development and potential benefits of arimoclomol for NPC, ALS, sIBM and Gaucher disease, 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.