

Orphazyme submits European Marketing Authorisation Application for arimoclomol for treatment of Niemann-Pick disease Type C

- *Follows U.S. Food and Drug Administration (FDA) acceptance of New Drug Application (NDA) with Priority Review in September 2020*
 - *Regulatory decisions expected in 2021*

Copenhagen, Denmark, November 9, 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, today announces it has submitted a Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for its investigational product candidate arimoclomol for the treatment of Niemann-Pick disease Type C (NPC).

This follows Orphazyme's U.S. NDA seeking approval for arimoclomol for the treatment of NPC that is under Priority Review with the FDA (PDUFA date: March 17, 2021). Arimoclomol has been granted Orphan Drug Designation in Europe and the U.S. Additionally, arimoclomol has FDA Fast Track and Breakthrough Therapy Designations for NPC as well as Rare Pediatric Disease Designations in the U.S.

Kim Stratton, Chief Executive Officer, said, *"This filing in Europe is a significant milestone for Orphazyme as we work toward our first potential approvals of arimoclomol in major markets. There are few options today that can address the devastating effects of NPC, and we are hopeful we can address an important need for this community. We look forward to working with EMA as they complete their review of our application."*

For additional information, please contact

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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 U.S. (ORPH) and Nasdaq Copenhagen (ORPHA).

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, sIBM, and ALS. Arimoclomol has received orphan drug designation (ODD) for NPC, sIBM, 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, sIBM and ALS. In addition, arimoclomol has received breakthrough therapy designation (BTD) and rare-pediatric disease designation (RPDD) from the FDA for NPC.

About 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, including relating to the terms of the proposed offering, the Extraordinary General Meeting and the completion of the proposed offering. 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.