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Orphazyme US

Orphazyme to showcase data on arimoclomol in Niemann-Pick disease Type C during the 2021 Annual WORLD*Symposium*™

Chicago, USA, February 4, 2021 – Orphazyme US (ORPH), a late-stage biopharmaceutical company pioneering the Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, today announced plans to present a series of data on its investigational drug arimoclomol during the 17th Annual WORLD*Symposium* Scientific Meeting, to be held virtually on February 8-12, 2021.

"*The WORLDSymposium, while virtual this year, offers an important opportunity to present the mechanisms of the Heat-Shock Protein response and how that translates into potential therapeutic utility for lysosomal diseases such as Niemann-Pick disease Type C (NPC),*" said Daniel Gallo, Head, U.S. Medical, Orphazyme. "*We look forward to convening with the research community during the conference and to showcasing these data in support of arimoclomol.*"

Arimoclomol data being presented at the virtual WORLDSymposium are:

- **Persistent effect of arimoclomol in patients with Niemann-Pick disease Type C: 12-month results from an open-label extension of a pivotal phase 2/3 study**
  - Presented by Marc Patterson, M.D.
  - Wednesday, February 10, Poster 191

- **Pharmacokinetics properties of arimoclomol in Niemann-Pick disease Type C: Modest and not clinically relevant effect of bodyweight or age**
  - Presented by Thomas Anderson
  - Thursday, February 11, Poster 5

- **Rescue of NPC1 protein by the Heat-Shock response amplifier arimoclomol across multiple genotypes**
  - Presented by Nikolaj Petersen, Ph.D.
  - Thursday, February 11, Poster 197

- **Impacts and burden of NPC: A Patient and Caregiver Perspective**
  - Presented by Eugen Mengel, M.D.
  - Thursday, February 11, Poster 158

- **Validation of a short-form 5-domain Niemann-Pick disease type C clinical severity scale (5-domain NPCCSS)**
  - Presented by Marc Patterson, M.D.
  - Thursday, February 11, Poster 190

Arimoclomol is under Priority Review by the U.S. Food and Drug administration (FDA) for the treatment of NPC with a target action date of June 17, 2021. NPC is a rare, progressive, life-threatening, neurodegenerative disease for which there is no cure and no approved treatment in the United States. Arimoclomol is also under review in Europe for the NPC indication.

"*We continue to engage in productive dialogue with the FDA related to the NDA for arimoclomol,*" said Thomas Blaettler, Chief Medical Officer, Orphazyme. "*In parallel, we continue to enroll patients in our Early Access Program (EAP) in the US and we are initiating EAPs in European countries, including France and Germany. These activities advance Orphazyme's mission to bring an innovative treatment to rare disease communities with significant unmet need.*"

For additional information, please contact

Orphazyme US

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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), Inclusion Body Myositis (IBM) 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 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, 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 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.