Investor News
No. 04/2020

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

www.orphazyme.com
Company Registration No. 32266355

Orphazyme to collaborate with The Michael J. Fox Foundation on Parkinson's disease research

Copenhagen, Denmark, July 17, 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 announces that it will join The Michael J. Fox Foundation's (MJFF) Parkinson's Disease Research Tools Consortium. The group brings together experts from the medical community and industry to identify and develop new pre-clinical tools to address unmet research needs and accelerate experiments. Orphazyme's drug candidate arimoclomol has potential across a range of neurodegenerative diseases, including GCase-deficient Parkinson's disease (PD).

"Our preclinical and clinical research activities have given the team at Orphazyme a deep understanding of neurodegenerative conditions, including PD. We look forward to sharing our knowledge of targeting heat shock proteins and lysosomal function with the consortium," said Thomas Kirkegaard Jensen, Orphazyme Founder and Chief Scientific Officer. "The Michael J. Fox Foundation is a highly respected non-profit organisation with an ambitious agenda to address PD. We are proud to join this group of industry leaders and PD experts and build on our commitment to find potential solutions for this devastating disease."

Mark Frasier, PhD, Senior Vice President, Research Programs at MJFF, says, "We welcome Orphazyme to the Parkinson's Disease Research Tools Consortium. The company's unique expertise will help us create even more useful tools and contribute to the positive momentum in Parkinson's research."

About Parkinson's disease
PD occurs when brain cells that make dopamine, a chemical that coordinates movement, stop working or die. It can cause resting tremor, slowness, stiffness, and walking balance and memory problems. PD is a lifelong and progressive disease. Mutations in the gene *GBA*, which lead to misfolded/deficient lysosomal enzyme glucocerebrosidase (GCase), present the greatest genetic risk in PD and therapeutics targeting this pathway may have potential in such forms of PD. Orphazyme's research is focused on exploring the potential role of Heat-Shock Proteins, which help to correct and restore protein misfolding and improve lysosomal function, in disorders such as GCase deficient PD.

About the MJFF Parkinson's Disease Research Tools Consortium
The consortium brings together industry researchers to discuss unmet research needs and priorities in PD. The group leverages combined resources to advance the development of high-quality preclinical research tools and ensure their widespread availability.

For additional information, please contact

Orphazyme A/S

Anders Vadsholt, CFO +45 28 98 90 55

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, 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.

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 the clinical development and potential benefits of arimoclomol for Gaucher disease, NPC, sIBM and ALS, 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.