

Orphazyme establishes U.S. headquarters in Chicago as the company prepares for commercialization

- *Global executive team expanding to support growth plans with three U.S.-based leaders*

Copenhagen, Denmark, Chicago, Illinois, USA, December 2, 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 announced it has established its U.S. headquarters in Chicago.

Orphazyme already has recruited more than 30 U.S. employees and recently named three U.S.-based leaders to its global executive team. Molly Painter, U.S. President, is heading the launch and commercial operations in the critical market. Terri Stevens is Chief Business Officer, responsible for global strategy, corporate development, and business development & licensing. Molly Carey Poarch has joined as Global and U.S. Head of Corporate Communications.

"We have established a U.S. headquarters in Chicago to allow us to more closely engage with our partners and community members as we work together to pursue innovation for debilitating neurodegenerative diseases based on our pioneering science," said Kim Stratton, Chief Executive Officer. *"Our U.S. growth will be fueled by the team of industry experts we have been assembling. We are excited to add three key U.S.-based leaders to our corporate executive team as we expand to support our ambition of serving rare disease communities around the world."*

The U.S. team, which is focused on the U.S. regulatory review and preparing for the company's first potential commercial launch in 2021, includes legal, commercial, finance, advocacy relations, regulatory, and medical affairs functions. Orphazyme's global headquarters is in Copenhagen, Denmark.

Brendan Reilly, whose Chicago City Council district includes the office, said, *"I am proud to welcome Orphazyme's U.S. headquarters to Chicago's 42nd Ward. Orphazyme will bring more than 30 full-time jobs and join a growing community of healthcare companies that call Chicago home. I look forward to their continued growth and progress in the biopharmaceutical field."*

"I am excited to welcome Orphazyme, a fresh and innovative biopharmaceutical to Chicago's robust group of health care companies. I believe their methodology will make leeway in the fight against rare neurodegenerative diseases while bringing job growth to the city," said State Senator Mattie Hunter.

Orphazyme announced in September that the U.S. Food and Drug Administration (FDA) has accepted for review the company's New Drug Application (NDA) for arimoclomol for the treatment of Niemann-Pick disease Type C (NPC) and designated it for Priority Review. The FDA has set a target action date of March 17, 2021 under the Prescription Drug User Fee Act (PDUFA).

NPC is a rare, relentlessly progressive, neurodegenerative disease that can have a devastating impact on the lives of patients and their families. There are no currently approved treatments in the United States for NPC. Arimoclomol has received FDA Fast-Track and Breakthrough Therapy Designations for NPC, as well as Orphan Drug and Rare Pediatric Disease Designations. In November, the company also submitted a Marketing Authorisation Application to the European Medicines Agency for arimoclomol in NPC.

In addition to the pending applications for NPC, the company has ongoing pivotal clinical trials underway to understand arimoclomol's potential for treating Amyotrophic Lateral Sclerosis (ALS) and Inclusion Body Myositis (IBM), and following the recently released results of a Phase 2 trial the company intends to establish a pivotal trial in Gaucher disease.

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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.