

Publication of manuscript on preclinical Proof-of-Concept of arimoclomol in Gaucher disease

Copenhagen, November 27, 2018 – Orphazyme A/S, a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, today announced that a manuscript providing preclinical Proof-of-Concept for its lead compound, arimoclomol, in Gaucher disease was published in the peer-reviewed scientific journal [EBioMedicine](#). The manuscript details how the heat-shock amplifier, arimoclomol, improves the refolding, maturation, and lysosomal activity of glucocerebrosidase (GCase), the enzyme deficient in Gaucher disease and linked to Parkinson’s disease.

A range of biological and biochemical assays were used to assess the effect of arimoclomol on GCase activity in *ex vivo* systems of fibroblasts and neuronal-like cells from Gaucher disease patients. Data showed that arimoclomol induced relevant Heat-Shock Proteins (HSPs), such as HSP70, and enhanced the folding, maturation, activity, and correct cellular localization of mutated GCase across several genotypes, including common mutations in primary cells from Gaucher disease.

Thomas Kirkegaard Jensen, Co-Founder and Chief Scientific Officer, says: “We are excited that the excellent work of our scientists and close collaborators is now available for all to read. The findings provide a cornerstone of our lysosomal disease programs and ambition to provide a treatment for all patients suffering from these devastating diseases”.

To read the full-length article, please visit the Media section on Orphazyme’s website: www.orphazyme.com

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About Orphazyme A/S

Orphazyme is a biopharmaceutical company focused on bringing novel treatments to patients living with life-threatening or debilitating rare diseases. Our research focuses on developing therapies for diseases caused by misfolding of proteins, including lysosomal storage diseases. Arimoclomol, the company’s lead candidate, is in clinical development for four orphan diseases: Niemann-Pick disease Type C, Gaucher disease, sporadic Inclusion Body Myositis, and Amyotrophic Lateral Sclerosis. 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 been studied in seven Phase I and three Phase II trials. Arimoclomol is in clinical development for NPC, Gaucher disease, sIBM, and ALS.

About Gaucher

Gaucher disease is a genetic, progressively debilitating lysosomal storage disease caused by mutations leading to defective glucocerebrosidase (GCase) protein. As a consequence, lipids that are normally cleared by the lysosome build-up in tissues and organs, including the bone marrow, liver, spleen, and sometimes brain, and drive the disease pathology. The estimated number of people affected with Gaucher disease in the USA and Europe combined is 10,000-15,000. Effective treatments are available for the systemic manifestations of the disease – however, no therapies are available for the neurological symptoms.

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.