

## Orphazyme endorses patient advocacy consensus guidelines, highlights importance of collaboration with patient communities

- *Orphazyme supports consensus guidelines to direct interactions with rare disease patient advocacy organizations*
- *Company extends heartfelt gratitude and respect to patient organization partners during Niemann-Pick Disease Awareness Month*

**Copenhagen, October 15, 2018** – Orphazyme A/S, a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, today announced the company's endorsement of published guidelines to support interactions with rare disease patient advocacy organizations.

Published in the Orphanet Journal of Rare Diseases earlier this year, [\*Principles for interactions with biopharmaceutical companies: The development of guidelines for patient advocacy organizations in the field of rare diseases\*](#), is a landmark article for the rare disease community. Toni Mathieson, Chief Executive of Niemann-Pick UK (NPUK), an organization that works in partnership with Orphazyme, is a co-author on the paper.

Anders Hinsby, Chief Executive Officer of Orphazyme, said: *"Close collaboration with patient organizations is essential to bringing new therapies to people living with rare diseases. We have been privileged to work with Niemann-Pick disease patient organizations, such as NPUK, for nearly a decade. The knowledge and experience shared by these groups have been instrumental in guiding our path."*

Anders added, *"The standards and recommendations put forth in this paper will further guide our interactions with non-profit organizations to ensure transparent, ethical partnerships as we increase our engagement with the communities representing Inclusion Body Myositis (IBM), Amyotrophic Lateral Sclerosis (ALS), and Gaucher disease."*

Paul Merrigan, Chief Commercial Officer and President of Orphazyme US, said: *"People living with rare disorders and their families are the authorities on these diseases; we rely on the expertise of patient organization partners to guide our actions. For these reasons, our first US employee was hired to create a patient advocacy relations program, built on the foundation of Orphazyme's longstanding commitment to collaborating with patient communities."*

Orphazyme colleagues who oversee engagement with patient communities will attend the National Organization of Rare Diseases (NORD) Rare Summit this week in Washington, D.C. We welcome the opportunity to meet with patient organizations working to support NPC, ALS, IBM, and Gaucher disease communities.

**For additional information, please contact****Orphazyme A/S**

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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](http://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 NPC**

Niemann-Pick disease Type C (NPC) is a 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 build-up in tissues and organs, including the brain, and drive the disease pathology. The estimated prevalence of NPC in the USA and Europe combined is 1,000-2,000. There are no approved treatments for NPC in the USA and only one approved product in Europe. Arimoclomol has been granted Orphan Drug Designation (EU and USA), Rare Pediatric Disease Designation (USA), and Fast Track designation (USA) for the treatment of NPC.

**About sIBM**

Sporadic Inclusion Body Myositis (sIBM) is a progressively debilitating muscle-wasting disease. sIBM is characterized by a build-up of protein aggregates and atrophy of muscle cells, which leads to weakness and over time severe disability. The estimated prevalence of sIBM is 24.8-45.6 per million or 17,000-31,000 patients in the USA and Europe. There are no approved treatments for sIBM. Arimoclomol has been granted Orphan Drug Designation (EU and USA) for the treatment of sIBM.

**About ALS**

Amyotrophic Lateral Sclerosis (ALS) is a rare, rapidly progressive, and always fatal neurodegenerative disease. Protein misfolding and aggregation in motor neurons are important contributors to the disease process, which ultimately leads to paralysis of skeletal muscles as well as the muscles that enable breathing. The patient population in Europe and the United States is estimated to be approximately 50,000 patients. Currently, there are only limited treatment options available. Arimoclomol has been granted Orphan Drug Designation (EU and USA) for the treatment of 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.