Press Release

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

www.orphazyme.com
Company Registration No. 32266355

7 November 2017

ORPHAZYME RECEIVES ORPHAN DRUG DESIGNATION TO ARIMOCLOMOL FOR INCLUSION BODY MYOSITIS FROM THE U.S. FDA

Orphazyme A/S ("Orphazyme" or the "Company"), a Danish biotech company with a late stage orphan drug pipeline, today announced that the U.S. Food and Drug Administration ("FDA"), Office of Orphan Products Development, has granted an orphan drug designation to arimoclomol for the treatment of the orphan disease Inclusion Body Myositis ("IBM"). As described in the offering circular published by Orphazyme A/S on 6 November 2017, the Company applied to the FDA for the designation in September 2017.

The Company focuses, among others, on sporadic inclusion body myositis ("sIBM") and has already obtained an EU orphan drug designation for sIBM from the European Medicines Agency ("EMA"), which was granted in 2016.

About U.S. orphan drug designation

The United States Congress passed the Orphan Drug Act in 1983 to provide incentives for investment in treatments for rare conditions. Companies that receive orphan drug designation are entitled to several advantages, including the possibility of free of charge advice from the FDA and certain financial benefits, such as R&D tax credits (which is not part of the company's current plans) and exemptions or reductions in regulatory submission fees. If a drug candidate with orphan drug designation is approved by the FDA upon completion of clinical trials, it may receive orphan drug status providing the orphan drug with market exclusivity for seven years in the United States.

About sIBM

The disease is one of several neuromuscular diseases and is one of the most common wasting muscle disorders, affecting patients in the later stages of life, typically over the age of 50 years. The size of the patient population in Europe and the United States is not fully elucidated but has been conservatively estimated to be between 7,000 and 15,000 individuals. No effective treatment is currently available.

For additional information, please contact:

Anders Hinsby, CEO +45 31 44 31 39

About Orphazyme A/S

Orphazyme is a Danish biotech company with a late stage orphan drug pipeline, developing new treatment options for orphan protein misfolding diseases. The Company was founded in 2009 based on early scientific discovery in heat shock proteins ("HSPs"). Since inception, the Company has translated scientific discovery into a late stage clinical development programme. The Company is headquartered in Copenhagen and currently has 30 employees.

The lead candidate arimoclomol is in development as a potential treatment for four orphan diseases; two neuromuscular diseases, sporadic Inclusion Body Myositis ("sIBM") and Amyotrophic Lateral Sclerosis ("ALS"), and two lysosomal storage diseases, Niemann Pick type C ("NPC") and Gaucher disease.