Company announcement
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Enrollment of first patient in Phase II clinical trial for Gaucher disease

Copenhagen, June 21, 2018 – Orphazyme A/S, a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, today announced that the first patient has received the first dose in a Phase II clinical trial for Gaucher disease.

Anders Hinsby, Chief Executive Officer, said: "*With the enrollment of the first patient in the Gaucher disease trial, we reach another important milestone in the development of our pipeline this year. Glucocerebrosidase deficiency leads to severe neurological manifestations in many patients with Gaucher disease. With this trial we move a step closer to a treatment option for these patients.*"

The trial takes place at clinical sites in India and will include approximately 40 patients. Patients will be randomized 1:1:1:1 into four treatment arms – active treatment at three different doses and placebo. The patients will receive arimoclomol or placebo-controlled treatment for six months. Following the placebo-controlled period, the placebo group will be re-randomized into one of the three active treatment groups for a six-month extension.

The primary endpoint in this trial is measurement of the enzyme chitotriosidase, which is a known and well-characterized marker of Gaucher disease. Measurements will be performed in samples from cerebrospinal fluid and blood to assess arimoclomol's potential effect on both neurological and peripheral disease.

Results from the Phase II trial are expected in H1 2019.

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 and lysosomal dysfunction. 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 glucocerebosidase (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.