

**Company announcement**  
No. 14/2018

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

www.orphazyme.com  
Company Registration No. 32266355

## **Orphazyme expects to announce results of full data set for Niemann-Pick disease Type C (NPC) Phase II/III trial in Q1 2019**

**Copenhagen, December 10, 2018** – Orphazyme A/S, a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, today announced that the Company expects to announce the results of the full data set for the clinical Phase II/III trial in NPC in Q1 2019.

On September 28, 2018, Orphazyme announced top-line data showing a treatment benefit in favor of arimoclomol, even if statistical significance was not reached. A subsequent evaluation of the data has motivated a further thorough data review and Orphazyme has initiated a data verification process that will be performed before completing the final statistical analysis of the trial.

Thomas Blaettler, Chief Medical Officer, said, “In this pivotal trial every single data point counts, which has led us to conduct our analysis at the highest level of granularity.”

The comprehensive data verification process is on-going and we expect to communicate the results of the full data set in Q1 2019, instead of in Q4 2018 as previously communicated.

This company announcement does not impact the 2018 financial guidance published in the Annual Report 2017 on March 15, 2018.

### **For additional information, please contact**

#### **Orphazyme A/S**

Anders Hinsby, CEO +45 31 44 31 39

#### **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](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.

**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.