

**Company announcement**  
No. 18/2019

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## **Orphazyme to prepare for filing of arimoclomol in US for Niemann-Pick disease Type C (NPC)**

- *US Food and Drug Administration (FDA) agrees to Orphazyme's plan to file New Drug Application (NDA) in US*
- *On track to file NDA and Marketing Authorisation Application (MAA) in NPC in both US and EU in H1 2020*

**Copenhagen, July 21, 2019** – Orphazyme A/S (ticker: ORPHA.CO), a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, today announces that it has had a positive meeting with the FDA and remains on track to submit an NDA for arimoclomol for NPC in H1 2020.

Anders Hinsby, Chief Executive Officer of Orphazyme, said: *"We are very pleased with the collaborative interactions we have had with the FDA who provided us with thorough guidance on data presentation in the NDA for arimoclomol in the US for NPC. The valuable advice we have received from health authorities in both the US and Europe increases our optimism that we may soon be able to provide an important treatment option for NPC, a severely debilitating and fatal disease that predominantly affects children."*

Following the encouraging regulatory agency feedback, Orphazyme plans to introduce an Early Access Program for NPC in the fall of 2019, to further accelerate access to treatment with arimoclomol for people living with NPC.

As previously disclosed, Orphazyme also intends to file an MAA in Europe in H1 2020.

### **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](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 1 and three phase 2 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 press release 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 press release 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.