

Company announcement
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Orphazyme to prepare for filing of arimoclomol in Europe for Niemann-Pick disease Type C (NPC)

- *Expected submission of Marketing Authorization Application (MAA) in H1 2020*

Copenhagen, June 7, 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 received constructive advice from the European Medicines Agency (EMA)'s Scientific Advice Working Group in response to the request for advice submitted to the agency. After receiving this feedback, Orphazyme confirms its intention to file the MAA for NPC in the first half of 2020.

Anders Hinsby, Chief Executive Officer, said: "We are pleased with the advice received from EMA, which provides good guidance on topics that we will take into careful consideration as we continue the preparation of a Marketing Authorization Application for arimoclomol in Europe. NPC is a severely debilitating and fatal disease that predominantly affects children and we are committed to bringing arimoclomol to patients suffering from this devastating disease".

Additional data, including 12-month open-label extension data, will become available during H2 2019 and must be included in the MAA.

Orphazyme plans to meet with the US Food and Drug Administration (FDA) to discuss the pathway forward during the Summer and will provide an update after it has received written comments from the FDA.

For additional information, please contact

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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 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.