

Company announcement

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Orphazyme initiates rolling submission of New Drug Application for arimoclomol with US FDA in Niemann-Pick disease Type C

- *EMA Marketing Authorisation Application submission expected in H2 2020*

Copenhagen, Denmark, May 29, 2020 – Orphazyme A/S (ORPHA.CO), a biopharmaceutical company pioneering the Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, today announces it has initiated the submission of its New Drug Application (NDA) for a rolling review by the US Food and Drug Administration (FDA) for arimoclomol, an investigational Heat-Shock Protein amplifier, for the treatment of Niemann-Pick disease Type C (NPC).

Arimoclomol has received FDA Fast Track and Breakthrough Therapy Designations for the treatment of NPC, in addition to Orphan Drug and Rare Pediatric Disease Designations. The FDA's rolling review allows the company to submit portions of the NDA to the FDA as they are completed. Orphazyme expects to complete submission of the remaining portions of the NDA to the FDA in the next couple of months.

Kim Stratton, Chief Executive Officer, said, *"We are very pleased to initiate this rolling submission for arimoclomol for the treatment of NPC, based on the promising results we have reported from clinical trials. Arimoclomol has the potential to significantly improve the lives of patients with this devastating disease and we look forward to working with the FDA with the goal of bringing this medicine to market. Orphazyme is continuing its commercial preparations for launch of arimoclomol in NPC in the US and other key markets, upon approval, as well as clinical development in three other indications where there is a significant unmet medical need: Amyotrophic Lateral Sclerosis (ALS), sporadic Inclusion Body Myositis (sIBM), and Gaucher disease."*

Orphazyme expects to submit a Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for arimoclomol in NPC in H2 2020.

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, including lysosomal storage diseases. 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 1 and three phase 2 trials. Arimoclomol is in clinical development for NPC, Gaucher disease, sIBM, and ALS.

About Niemann-Pick disease Type C

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. 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.