Company announcement
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Orphazyme's arimoclomol receives US Fast Track Designation in Amyotrophic Lateral Sclerosis

Copenhagen, Denmark, May 22, 2020 – Orphazyme A/S (ORPHA.CO), a biopharmaceutical company pioneering Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, announces that the Company has received Fast Track Designation from the US Food and Drug Administration (FDA) for the development of arimoclomol for the treatment of Amyotrophic Lateral Sclerosis (ALS).

Kim Stratton, Chief Executive Officer, said, "This is the third Fast Track Designation that arimoclomol has received from the FDA, which further underlines the potential of our investigational drug, the seriousness, and high unmet medical need in the diseases that we are targeting. We are continuing to evaluate arimoclomol in a phase 3 clinical trial in ALS and look forward to topline results from the trial in H1 2021".

Arimoclomol has also received Fast Track status from the FDA for the treatment of Niemann-Pick disease Type C (NPC) and sporadic Inclusion Body Myositis (sIBM).

Fast Track is a designation by the FDA of an investigational drug for expedited review to facilitate development of drugs which treat a serious or life-threatening condition and fill an unmet medical need. Fast Track status entails eligibility for Accelerated Approval and Priority Review if certain criteria are met, as well as Rolling Review, which means that a company can submit completed sections of its Biologic License Application (BLA) or New Drug Application (NDA) for review by the FDA, rather than waiting until every section is completed before the entire application can be reviewed.

Orphazyme initiated a phase 3 trial in August 2018 with arimoclomol in ALS. Topline results from this trial are expected in H1 2021.

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 ALS
Amyotrophic Lateral Sclerosis (ALS) is a rare, rapidly progressive, and always fatal neurodegenerative disease. Protein misfolding and aggregation in motor neurons are important contributors to the disease process, which ultimately leads to paralysis of skeletal muscles as well as the muscles that enable breathing. The patient population in Europe and the United States is estimated to be approximately 50,000 patients. Currently, there are only limited treatment options available. Arimoclomol has been granted Orphan Drug Designation (EU and USA) for the treatment of ALS.

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.