Company announcement
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Orphazyme's arimoclomol receives US Fast Track designation in sporadic Inclusion Body Myositis

Copenhagen, Denmark, December 18, 2019 – Orphazyme A/S (ORPHA.CO), a biopharmaceutical company pioneering Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, today received Fast Track designation from the US Food and Drug Administration (FDA) for the development of arimoclomol for the treatment of sporadic Inclusion Body Myositis (sIBM).

Thomas Blaettler, Chief Medical Officer, commented, "We are highly encouraged to receive Fast Track status from the FDA for arimoclomol in sIBM. This is our second Fast Track Designation for arimoclomol, and it strongly underlines the great potential of our investigational drug".

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 more frequent interactions with the FDA.

Results from the phase 2/3 trial with arimoclomol for sIBM are expected in the first half of 2021. Following a strong recommendation from the European Medicines Agency (EMA), the trial protocol is amended to omit an interim analysis (originally scheduled for H1 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 sIBM
Sporadic Inclusion Body Myositis (sIBM) is a progressively debilitating muscle-wasting disease. sIBM is characterized by a build-up of protein aggregates and atrophy of muscle cells, which leads to weakness and over time severe disability. The estimated prevalence of sIBM is 45.6 per million or 40,000 patients in the USA and Europe. There are no approved treatments for sIBM. Arimoclomol has been granted Orphan Drug Designation (EU and USA) for the treatment of sIBM.

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.