Company announcement
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Orphazyme completes enrollment in phase 3 trial evaluating arimoclomol in Amyotrophic Lateral Sclerosis

• *Headline results from full analysis expected in H1 2021*

Copenhagen, Denmark, July 18, 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 completed enrollment in its phase 3 trial evaluating arimoclomol for the treatment of Amyotrophic Lateral Sclerosis (ALS) ahead of schedule. Headline results from the full analysis remain on track for the first half of 2021.

The trial completed enrollment sooner than anticipated thanks to support from the global ALS community and the participation of hundreds of people living with ALS and their care partners. An interim analysis of the data is no longer necessary due to the proximity of the final data sets to the planned interim analysis.

Michael Benatar, MD, PhD, University of Miami, Lead International Coordinating Investigator, said: "*It is thanks to the tireless efforts of investigators and coordinators at participating centers across the world that we have been able to reach this important milestone, bringing us one step closer to definitively learning whether arimoclomol is beneficial for patients living with ALS*."

Thomas Blaettler, MD, Chief Medical Officer, said: "*The completion of enrollment for Orphazyme's pivotal ALS trial is another great milestone for our clinical development program. We are confident that the trial design and 18-month trial duration will maximize our ability to demonstrate efficacy. By omitting the interim analysis, we also save statistical power for the final analysis of the trial, further enhancing potential for success. We are excited about the possibilities of arimoclomol as a potential new therapy in ALS and now look forward to reporting trial results in the first half of 2021.*"

Calaneet Balas, President & CEO of the ALS Association and Chairwoman of the International Alliance of ALS/MND Associations, said: "*We are grateful to the hundreds of people living with ALS around the world for participating in this trial. We urgently need new treatments for ALS and we look forward to the results.*"

The phase 3 trial of arimoclomol for ALS is a 76-week, randomized, placebo-controlled trial being conducted at 30 centers of excellence in North America and Europe. A total 213 participants have been randomized 2:1 to arimoclomol and placebo and up to an additional 18 individuals on stable treatment with edaravone may participate in the US. Patients completing the trial will be offered participation in an open-label extension trial.

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.

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 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.