Company announcement
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Orphazyme announces enrollment of first patient in Phase III clinical trial of arimoclomol for ALS

- *Primary objective is to determine efficacy of chronic treatment with arimoclomol compared to placebo over 76 weeks*
- *Results expected in H1 2021*

Copenhagen, August 10, 2018 – Orphazyme A/S, a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, today announced that the first patient has received the first dose in its Phase III clinical trial of arimoclomol for Amyotrophic Lateral Sclerosis (ALS).

Thomas Blaettler, Chief Medical Officer, said: "*The enrollment of the first patient in our Phase III trial for ALS represents a major milestone for the company. Arimoclomol has shown substantial promise in previous trials and has a favorable safety and tolerability profile. This is a truly devastating disease and we will work to expeditiously advance the Phase III trial with the prospect of making a new therapeutic option available to the patients in the shortest possible timeframe"*.

Michael Benatar, MD, PhD, University of Miami, Principal Investigator of a prior Phase II trial of arimoclomol in SOD1 ALS and Lead International Coordinating Investigator, said: "I am delighted to see arimoclomol advance into Phase III testing and that the eligible population has been broadened to include all patients with ALS. Based on the mechanism of action of the drug and our understanding of the underlying biology of ALS, there is good reason to believe that all patients with ALS might benefit from this therapeutic approach".

Lucie Bruijn, PhD, MBA, Chief Scientist at the ALS Association, said: "*On behalf of the patients struck by this devastating disease, I am delighted to see this Phase III trial up and running. The ALS Association invested in the early clinical trials and are pleased to see this moving forward to a Phase III study and to seeing the potential of arimoclomol unfold*".

The randomized and placebo-controlled Phase III trial in ALS is being conducted in North America and Europe and will enroll 231 patients, to be randomized in a 2:1 ratio receiving either arimoclomol or placebo for up to 76 weeks. Patients completing the trial will be offered participation in an open-label extension trial.

The primary objective of the present trial is to determine the efficacy of chronic treatment with arimoclomol compared to placebo over 76 weeks in subjects with ALS as assessed with a combined assessment of function and survival (CAFS). Important secondary endpoints include survival, change in ALSFRS-R, and slow vital capacity (SVC).

A total of 30 sites are planned in the US, Canada and Europe.

Results from the Phase III 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 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 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.