Design of the Phase 3, Randomised, Placebo-Controlled Trial of oral Arimoclomol in Early Amyotrophic Lateral Sclerosis - ORARIALS-01 (NCT:03491462)

Claus Sundgreen (1), Thomas Blaettler (2), Richard Bennett (1), Dror Rom (2), Peter M. Andersen (3), Joanne Wuu (4), Michael Benatar (4)

1) Orphazyme A/S, Copenhagen, Denmark. 2) Proso� Clinical Inc, Huntingdon Valley, PA, USA. 3) Department of Pharmacology and Clinical Neuroscience, Umeå University, Sweden. 4) Department of Neurology, University of Miami, FL, USA.

BACKGROUND

Clinical

Exploratory efficacy assessment of the open-label extension part of a phase 2 trial of arimoclomol in sporadic ALS showed a slowing effect in ALSFRS-R compared to placebo (12-month period).

METHODS

Inclusion criteria are based on analysis of the PRO-ACT database identifying patients with relatively homogeneous progression over an observation period of 12–18 months.

The primary endpoint is the measurement of the Combined Assessment of Function and Survival (CAFS) (3) in the arimoclomol treatment arms as compared to placebo after 18 months.

RESULTS & CONCLUSION

- The last subject (n=245) was enrolled on 17th July 2019.
- The final follow-up will be week 78.
- The primary endpoint will be assessed after 18 months.
- The trial has been approved by the FDA (NCT03491462).

DESIGN

Eligible patients are those aged ≥18 years who meet the revised El Escorial criteria for clinically possible, clinically probable, clinically definite ALS, or have familial ALS caused by a known pathogenic mutation.

About Orphazyme

Orphazyme A/S is a Danish biopharmaceutical company with a late-stage drug pipeline, developing new treatment options for orphan protein-misfolding diseases. The lead program, arimoclomol, recently reported positive results from a phase IIb study (NCT03746587) in N3A mutant amyotrophic lateral sclerosis (ALS).

For more information, please visit www.orphazyme.com.