Orphazyme Clinical Trial Hits Full Enrollment

Orphazyme has fully enrolled its Phase 3 clinical study of arimoclomol in amyotrophic lateral sclerosis (ALS). The Danish biopharma company hit full enrollment ahead of schedule due in large part to the efforts of the global ALS community who put out the word to people living with ALS.

When proteins become misfolded during cell stress, they clump together (or aggregate) causing toxicity and cell death. Protein misfolding and aggregation in motor neurons are important contributors to the disease process in ALS.

Arimoclomol works by amplifying the production of heat shock proteins (HSPs) during cell stress. HSPs may rescue defective misfolded proteins and clear proteins that have aggregated, like TDP-43.

The ALS Association funded earlier trials of arimoclomol.

The Orphazyme study is investigating arimoclomol in people living with ALS at 30 centers of excellence in North America and Europe to determine the efficacy and safety of this potential new oral therapy.

Results of the study are anticipated in the first half of 2021. An open-label extension study will be offered to participants who complete the 76-week trial.

Arimoclomol has been granted Orphan Drug Designation in the United States for the treatment of ALS. Orphan drugs are pharmaceutical agents developed to treat diseases that are extremely rare and would not be profitable for a manufacturer to produce without financial assistance from the government. The rare conditions treated by such drugs are referred to as orphan diseases.

Orphazyme, a biopharmaceutical company with offices in Copenhagen, Denmark, and Newton, MA, USA is dedicated to developing treatments for people living with rare diseases such as ALS.

For more information, visit Orphazyme: https://www.orphazyme.com/news-feed/2019/7/18/orphazyme-completes-enrollment-in-phase-3-trial-evaluating-arimoclomol-in-amyotrophic-lateral-sclerosis