PHASE 3 CHAMPION-ALS CLINICAL TRIAL

Evaluating the efficacy and safety of ULTOMIRIS® (ravulizumab) as a potential treatment for adults living with familial or sporadic ALS (age 18 years and older).

Amyotrophic Lateral Sclerosis (ALS)

A progressive neurodegenerative disease that affects motor nerve cells in the brain and spinal cord.

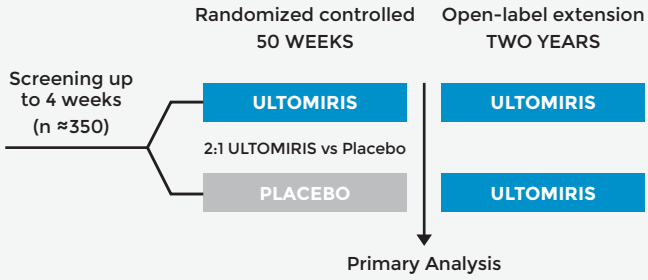
PRIMARY ENDPOINT

Change from baseline in ALS functional rating scale-revised (ALSFRS-R) score.

SECONDARY ENDPOINTS

- Ventilation assistance-free survival (VAFS)
- Change from baseline in respiratory capacity
- Change from baseline in muscle strength
- Change from baseline in neurofilament light chain (NfL) serum concentrations
- Safety

ULTOMIRIS ALS Phase 3 Trial Design



ULTOMIRIS for ALS

ULTOMIRIS is the first and only long-acting C5 complement inhibitor. The medication works by inhibiting the C5 protein in the terminal complement cascade, a part of the body's immune system. When activated in an uncontrolled manner, the complement cascade over-responds, leading the body to attack its own healthy cells. ULTOMIRIS has the potential to inhibit complement-mediated damage in people with ALS, which may slow disease progression based on pre-clinical data and the significant role complement activation is thought to play in other neuromuscular diseases.

Key Elements of CHAMPION-ALS

2:1 randomized

A 50-week, double-blind, 2:1 randomized, placebo-controlled and multi-center Phase 3 clinical study.

every 8 weeks

Enrolled participants will receive ULTOMIRIS or placebo every eight weeks, following an initial loading dose.

~350 adults
~90 trial sites

The study will enroll a broad ALS population from North America, Europe, and the Asia-Pacific region. Additional information about the study will be posted to clinicaltrials.gov and other global trial websites.

50 weeks
+2 year extension

After 50 weeks, participants may enroll in an open-label extension phase to receive ULTOMIRIS for up to two years.

ALEXION®

This study was designed in partnership with people living with ALS, leading ALS specialists and global regulatory agencies.