



MULTICENTER STUDY WITH PLACEBO GROUP TO STUDY THE EFFECT OF LITHIUM IN COMBINATION WITH RILUZOLE IN VOLUNTEERS WITH AMYOTROPHIC LATERAL SCLEROSIS (IRB # 15491)

Amyotrophic lateral sclerosis (ALS) is a rare, neurodegenerative disorder that results in progressive wasting and paralysis of voluntary muscles. In this double blind, randomized, placebo-controlled clinical trial, researchers will evaluate the safety and effectiveness of the drug lithium given in combination with riluzole, a drug commonly used to treat ALS, compared to a placebo given in combination with riluzole.

Approximately 250 participants will be recruited from multiple centers, in the US and Canada, that belong to the Northeast ALS Consortium (NEALS) and the Canadian ALS Clinical Trials and Research Network (CALs). Enrollment will occur in stages. Initially 84 participants will be enrolled in the trial. An interim analysis using available data will occur after the 84th participant is enrolled. During this time, the Data and Safety Monitoring Board (DSMB) appointed by the National Institutes of Health (NIH) may decide to stop the trial for efficacy or futility reasons or to stop enrollment and request that follow-up continue with the 84 participants already enrolled in the trial, or the DSMB may decide to continue enrollment.

Participants will be randomized to one of two arms of the study. Arm one will receive lithium and riluzole. Arm two will receive riluzole and placebo (an inactive substance). All participants will be receiving riluzole.

After screening and randomization, participants will be followed every 4 weeks for the first 12 weeks.

Subsequent in-person visits will occur every 8 weeks with a final visit at week 52. Between in-person visits, telephone interviews will take place every 4 weeks to administer the ALS Functional Rating Scale—Revised (ALSFRS-R) questionnaire. A follow-up telephone interview will occur at week 56 (off study medication) to review adverse events. The primary outcome measure is disease progression as measured by the ALSFRS-R questionnaire.

Participants randomized to placebo whose disease progresses will be crossed over to lithium for the remaining period of the study (up to 52 weeks total).

Duration of the study for participants is 56 weeks which includes 52 weeks of treatment and a follow up telephone interview at week 56.

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