

HEALEY ALS Platform Trial

Regimen I

NUZ-001

Developed by Neurizon Therapeutics

Investigational products included in the HEALEY ALS Platform Trial are selected by a team of experts after careful review of the study drug and the science supporting its treatment potential in Amyotrophic Lateral Sclerosis (ALS). Each regimen evaluates one study drug.

Regimen I is testing an experimental medication called NUZ-001. The randomized controlled trial for this regimen (the initial period when there is a chance of receiving placebo) will involve in-person study visits every 4 to 8 weeks; about 5-6 visits total over the course of 36 weeks.

In order to enroll in this trial, participants must:

- Be within 24 months of weakness symptom onset due to ALS
- Have a Vital Capacity score greater than or equal to 50% of predicted value
- Be able to safely swallow pills and liquids

View full list of
eligibility criteria on
ClinicalTrials.gov
<https://bit.ly/4hhIGSg>



About Regimen I

Regimen I is a Phase 2/3 trial enrolling approximately 160 participants to evaluate the safety and efficacy of NUZ-001 as a potential treatment for ALS. This regimen involves biomarker analysis and cerebrospinal fluid (CSF) collection to assess the effects of NUZ-001.

3:1 Active Drug to Placebo Ratio

Participants who enroll in this regimen have a 3 in 4 (75%) chance of being assigned to active study drug and a 1 in 4 (25%) chance of being assigned to placebo during the initial 36-week randomized controlled trial (RCT) period.

Active Treatment Extension (ATE)

Participants will continue into the ATE for NUZ-001 after completing the 36-week RCT. During ATE, all participants will receive the active study drug. The ATE for Regimen I is designed to last approximately 36 weeks or until the primary (topline) results of the RCT are available.

View map and contact
info for participating
research centers

<https://bit.ly/3lICv9t>



For general questions about the trial
Contact the Patient Navigator

healeysplatform@mgh.harvard.edu
833-425-8257 (HALT ALS)

Q&A for Regimen I



Your study team can help answer any questions you have about the possible benefits and risks of participating in research.

Q: How is the study drug administered?

A: NUZ-001 or placebo is taken by mouth once a day after a meal. The number of tablets (pills) that a participant will take each day is based on their body weight.

Q: What does NUZ-001 do?

A: NUZ-001 aims to slow ALS disease progression and protect motor neuron health by decreasing harmful protein buildup inside cells. In ALS, motor neurons are damaged in part because harmful proteins, such as TDP-43, build up inside the neurons and disrupt normal cell function. NUZ-001 strengthens the cell's own internal natural clearance systems, switching on pathways that help clear away toxic protein buildup and support motor neuron survival.

Q: Has NUZ-001 been studied before?

A: Yes. In Australia, a Phase 1 clinical trial studied NUZ-001 in 12 people living with ALS. The trial included an initial study period followed by continued access to the investigational drug. Some participants received NUZ-001 for over three years, giving researchers valuable longer-term safety information. NUZ-001 was generally well tolerated with few side effects reported, and the Phase 1 study provided information that supported moving forward into the HEALEY ALS Platform Trial.

Q: What side effects might be expected?

A: In a previous study of NUZ-001 in people living with ALS, some participants experienced dry mouth, constipation, increased hair growth, or raised liver enzymes. NUZ-001 is an experimental medication, so the side effects are still being studied. Participant safety will be closely monitored, and your Platform Trial study team will help manage any possible side effects.

Stay Connected to the Platform Trial

Investigational products will be added to the HEALEY ALS Platform Trial through support by pharma, foundation partners, philanthropy, federal, and other fundraising initiatives.

**Register to attend
Platform Trial Q&A
Webinars**



<https://bit.ly/3DvkJTa>

**Visit the 'Study Drugs'
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about each regimen**



<https://bit.ly/31EKT98>

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