

# HEALEY ALS Platform Trial - Master Protocol

**Status:** RECRUITING

## Eligibility Criteria

**Age:** 18 years and over

This study is NOT accepting healthy

**Healthy Volunteers:** volunteers

### Inclusion Criteria:

1. Sporadic or familial ALS diagnosed as clinically possible, probable, lab-supported probable, or definite ALS defined by revised El Escorial criteria. 2. Age 18 years or older. 3. Capable of providing informed consent and complying with study procedures, in the SI's opinion. 4. Time since onset of weakness due to ALS  $\leq$  24 months at the time of the Master Protocol Screening Visit. 5. Vital Capacity  $\geq$  50% of predicted capacity at the time of the Master Protocol Screening Visit measured by Slow Vital Capacity (SVC), or, if required due to pandemic-related restrictions, Forced Vital Capacity (FVC) measured in person. 6. Participants must either not take riluzole or be on a stable dose of riluzole for  $\geq$  30 days prior to the Master Protocol Screening Visit. 7. Participants must either not take edaravone or have completed at least one cycle (typically 14 days) of edaravone prior to the Master Protocol Screening Visit. 8. Participants must have the ability to swallow pills and liquids at the time of the Master Protocol Screening Visit and, in the SI's opinion, have the ability to swallow for the duration of the study. 9. Geographically accessible to the site.

### Exclusion Criteria:

1. Clinically significant unstable medical condition (other than ALS) that would pose a risk to the participant, according to SI's judgment (e.g., cardiovascular instability, systemic infection), or clinically significant laboratory abnormality or EKG changes. Clinically significant abnormal liver or kidney function is exclusionary. The following values \[alanine aminotransferase (ALT) or aspartate aminotransferase (AST)  $>$  3 times the upper limit of normal (ULN) or estimated Glomerular Filtration Rate (eGFR)  $<$  30 mL/min/1.73m<sup>2</sup>] are exclusionary regardless of clinical symptoms. 2. Presence of unstable psychiatric disease, cognitive impairment, dementia or substance abuse that would impair ability of the participant to provide informed consent, in the SI's opinion. 3. Active cancer or history of cancer, except for the following: basal cell carcinoma or successfully treated squamous cell carcinoma of the skin, cervical carcinoma in situ, prostatic carcinoma in situ, or other malignancies curatively treated and with no evidence of disease recurrence for at least 3 years. 4. Use of investigational treatments for ALS (off-label use or active participation in a clinical trial) within 5 half-lives (if known) or 30 days (whichever is longer) prior to the Master Protocol Screening Visit. 5. Exposure at any time to any gene therapies under investigation for the treatment of ALS (off-label use or investigational). 6. If female, breastfeeding, known to be pregnant, planning to become pregnant during the study, or of child-bearing potential and unwilling to use effective contraception, for the duration of the trial and for 3 months, or as specified in each RSA, after discontinuing study treatment. 7. If male of reproductive capacity, unwilling to use effective contraception for the duration of the trial and for 3 months, or as specified in each RSA, after discontinuing study treatment. 8. Anything that would place the participant at increased risk or preclude the participant's full compliance with or completion of the study, in the SI's opinion. 9. If a participant is being re-screened, the disqualifying condition has not been resolved, or the mandatory wash-out duration has not occurred.

## Conditions & Interventions

### Interventions:

DRUG: Zilucoplan, DRUG: Verdiperstat, DRUG: CNM-Au8, DRUG: Pridopidine, DRUG: SLS-005 Trehalose, DRUG: ABBV-CLS-7262, DRUG: DNL343, DRUG: NUZ-001

### Conditions:

Amyotrophic Lateral Sclerosis

### Keywords:

ALS, Placebo-Controlled, Double-Blind, Master Protocol, Lou Gehrig's Disease

## More Information

**Contact(s):** HEALEY Center for ALS at Massachusetts General Hospital - healeyalsplatform@mgh.harvard.edu

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### IRB

**Number:** HM20022250

**System ID:** NCT04297683

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