Thank you for joining the weekly webinar, Happy New Year! We are admitting audience members from the waiting room. Please allow a few moments for the webinar to begin.
This trial is dedicated to all people living with ALS, their families, and friends. Your partnership in research is what keeps us filled with passion, dedication, and the commitment to develop new treatments for ALS.

This breakthrough trial would not be possible without your participation.

Every research participant, whether on the active drug or placebo, plays a critical role in making the hope of finding a cure for ALS a reality.

Thank You
Common Protocol and Shared Infrastructure Allow for Operational and Scientific Efficiencies

Regimen: Active Study Drug + Matching Placebo

1 Protocol (Phase 2/3)
1 single IRB
Central Governance

7 Regimens
70+ Enrolling Sites
~1300 Participants
The HEALEY ALS Platform Trial is a perpetual trial to provide decisive answers and direction with efficient execution.
The objective of each regimen is to provide a go / no go decision to inform the clinical development program of the regimen study drug.

**Primary Endpoint (Placebo-Controlled Period)**
Change from baseline through week 24 in disease severity as measured by the ALSFRS-R total score and survival.

**Safety, Secondary, and Exploratory Endpoints**
(respiratory function, muscle strength, survival, biomarkers + regimen-specific endpoints)
Each regimen is compared to the shared placebo dataset, which keeps growing as new regimens are added.

**Participant Flow**

- Enroll in HEALEY ALS Platform Trial
- Randomization
- Shared infrastructure and common protocol allow sharing of placebo data

**KEY ELIGIBILITY CRITERIA**

- Sporadic or familial ALS
  - (possible, probable, lab-supported probable, or definite by revised EEC)
- Time since weakness onset ≤ 3 years
- Slow vital capacity ≥ 50% of predicted
- Able to swallow
- Either not take or be on stable dose of riluzole for ≥ 30 days
- Either not take or have completed at least one cycle of edaravone
- Either not take or have started Relyvrio/Albrioza ≥ 30 days prior to screening
The platform trial is a unique opportunity to move ALS biomarkers and new outcome measures forward.

**DNA** – whole genome sequencing

**Neurofilaments** – for all regimens + regimen-specific biomarkers based on MOA

**Home Spirometry** – critical during the pandemic

**Speech Analysis** – emerging digital biomarker

Additional biomarkers/outcome measures considered for upcoming and future regimens (e.g., new patient-reported outcomes - ROADS; PBMCs for stem cell generation)
Providing research access across a diverse network of 70+ NEALS sites

Contact a study team near you to discuss enrollment opportunities

https://bit.ly/3g2NZr5
Enrollment Update: Regimen F and Regimen G

516 Participants consented to Master Protocol since RGF and RGG initiated

438 Participants assigned to RGF or RGG

392 Participants randomized within RGF or RGG

Thank You
for your partnership in ALS research

(as of 1/4/24)
New Year, New Webinars, New Format!

Tune in for updates about Expanded Access and EAPs! **on the 2nd Thursday of each month**

https://bit.ly/3r6Nd2L
Patient Navigation
Central resource for people living with ALS

Phone: 833-425-8257 (HALT ALS)
E-mail: healeyalsplatform@mgh.harvard.edu

Weekly webinar registration:
https://bit.ly/3r6Nd2L

ALS Link sign-up:
https://bit.ly/3o2Ds3m

Upcoming Webinars:
January 11th - EAP discussion with guest speakers from the NIH
January 18th - Pridopidine (Regimen D) update with Prilenia Therapeutics
January 25th - CNM-Au8 (Regimen C) update with Clene Nanomedicine