HEALEY ALS Platform Trial

Weekly Q&A – Feb 23, 2023
HEALEY ALS Platform Trial Goals:
- Screen new drugs rapidly and efficiently
- Get solid answers
- Determine next steps
Schema for Each Regimen

- **Screen for eligibility**
- **Randomization 3:1**
- **Placebo**
- **Active**

**Screening Period**

**Randomized Period**

**Open-Label Extension Period**

(Active Treatment Extension)

- **24 weeks**
The HEALEY ALS Platform Trial is a unique opportunity to advance science

**DNA** – whole genome sequencing

**Neurofilaments** – for all regimens

**Biomarkers** (Blood, Urine, CSF) – several drug-specific biomarkers

**Speech Analysis** – emerging digital biomarker

**Home Spirometry** – critical during the pandemic

Additional biomarkers/outcome measures are being considered for upcoming regimens (e.g., new patient-reported outcomes; PBMCs for stem cell generation)
The positive results on speech and bulbar function, and on overall function and breathing in people earlier in disease course, are very encouraging and deserve further investigation in a phase III trial. This was a successful phase II trial where we learned a tremendous amount that will inform the follow-on clinical trials of pridopidine in ALS.

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Director, Sean M. Healey & AMG Center for ALS, Massachusetts General Hospital
Upcoming Webinars about Regimen D

Webinar for Participants in Regimen D

Monday, February 27th
Platform Trial sites will communicate details to their participants.

Community Webinar (Open to Public)

Monday, March 6th at 10:00 am Eastern Time
Register here: https://bit.ly/3KQ3q6L
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:
March 2nd- Weekly Q&A
March 9th- Weekly Q&A