Healey ALS Platform Trial

Weekly Q&A – Dec 2, 2021
Guest Speaker

Neil Thakur, PhD
Chief Mission Officer
The ALS Association
A rapid and fundamental transformation in the ALS experience, until we find cure

**Longer Lives**
- Life-extending treatments made accessible to ALL people with ALS
- Prevention of harms associated with ALS

**Improved Quality of Life**
- People with ALS empowered to live their lives as they want
- Greater engagement and autonomy
- Reduced or prevented physical, emotional and financial burdens

**Preventing Loved Ones from Getting ALS**
- Genetic screening to identify those at risk
- Prevention of new cases of ALS

https://www.als.org/whatever-it-takes
How we get there

Find New Treatments & Cures
- More clinical trials and participation with research
- More focused research

Optimize Current Treatments & Care
- Improve and deliver state of the art care
- Improve assistive technology

Prevent or Delay Harms Associated with ALS
- Identify risk factors
- Treat as early as possible
- Discover new preventative treatments
The ALS Association’s Research Program Currently Funds Widely Across the ALS Ecosystem

- Biology and Genetics of ALS
- Preclinical Drug Discovery
- Risks and Causes of ALS
- Biomarkers Development
- Phase I
- Phase II
- Phase III
- Infrastructure, Specimens, Data, Tools, Training

Managing ALS
- Phase IV Medication
- Assistive Technology
- Telehealth/telemedicine
- Family/caregiver burden
- Access/Quality of Care
- Natural History
- Surveys

~150 Active Projects, 12 countries around the world, $50M research commitment
The Healey ALS Platform trial makes part of the pipeline faster

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Managing ALS
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The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

Screening

Regimen A
(n=160 for each regimen)

Regimen B

Regimen C

Regimen D
(n=120 for active drug; n=40 for placebo)

3:1 Randomization within each Regimen

Regimen Assignment

Zilucoplan
Placebo

Open Label Extension

Verdiperstat
Placebo

Open Label Extension

CNM-Au8
Placebo

Open Label Extension

Pridopidine
Placebo

Open Label Extension

Shared Placebo

Regimen D
Regimens A, B and C completed enrollment!

- 162 individuals were randomized within Regimen A
- 167 individuals were randomized within Regimen B
- 161 individuals were randomized within Regimen C
- 156 individuals were randomized within Regimen D

299 have entered the Open Label Extension (OLE)

>800 people with ALS signed Informed Consent for the Platform Trial

“I’m looking forward to helping find a cure for ALS.“
- Platform trial participant

Thank You

This breakthrough trial would not be possible without your partnership

as of 12/2/21
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

Screening

Regimen Assignment

(n=160 for each regimen)

Regimen A

Regimen B

Regimen C

Regimen D

Regimen E

3:1 Randomization within each Regimen

(n=120 for active drug; n=40 for placebo)

Zilucoplan

Placebo

Verdiperstat

Placebo

CNM-Au8

Placebo

Pridopidine

Placebo

Trehalose

Placebo

Shared Placebo

Open Label Extension
54 sites enrolling, 20 more selected

- Atrium Health
- Cleveland Clinic
- George Washington University
- University of California, San Francisco
- Yale University
- Indiana University
- Stony Brook University
- Georgetown University
- University of Arkansas
- University of Pittsburgh
- University of Southern California
- University of Utah
- Augusta University
- University of Cincinnati
- Virginia Commonwealth University
- Swedish Medical Center
- Las Vegas Clinic
- Kaiser, Los Angeles
- Lehigh Valley Health Network
- St. Alphonsus Regional Medical Center
- Hackensack University
- Essentia Health

Note: Only those sites highlighted in blue are actively enrolling at this time. Additional sites will be activated on a rolling basis.
Patient Navigation
Central resource for people living with ALS

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

- Enrollment updates shared weekly
- Over 1,000 questions about ALS research answered live
- Over 13,000 viewers from 43 countries
- Special guests and topics added each week

Weekly webinar registration:

ALS Link sign-up:
Send us webinar ideas!

**Upcoming Guest Speakers:**

Dec 9th- Nicholas Olney, MD (Providence ALS Clinic, OR)

Dec 16th- Biostatistics with Lori Chibnik, PhD, MPH

Dec 23rd- No Webinar

Dec 30th- No Webinar

Weekly webinar registration:

ALS Link sign-up: