HEALEY ALS Platform Trial

Weekly Q&A – Dec 9, 2021
Guest Speaker

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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

3:1 Randomization within each Regimen

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

Zilucoplan

Placebo

Verdiperstat

Placebo

CNM-Au8

Placebo

Pridopidine

Placebo

Shared Placebo

Open Label Extension

Open Label Extension

Open Label Extension

Open Label Extension
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
- 158 individuals were randomized within Regimen D

309 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 12/9/21
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

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3:1 Randomization within each Regimen

Regimen A

Regimen B

Regimen C

Regimen D

Regimen E

Zilucoplan

Verdiperstat

CNM-Au8

Pridopidine

Trehalose

Shared Placebo

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Placebo

Placebo

Placebo

Placebo

Placebo

(n=160 for each regimen)

(n=120 for active drug; n=40 for placebo)
Plans for Expansion: 22 New Sites Coming Soon

- 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 16\textsuperscript{th} - Biostatistics with Lori Chibnik, PhD, MPH
Dec 23\textsuperscript{rd} - No Webinar
Dec 30\textsuperscript{th} - No Webinar

Weekly webinar registration: [QR Code]

ALS Link sign-up: [QR Code]