HEALEY ALS Platform Trial

Weekly Q&A – May 5, 2022

Healey Center
Sean M. Healey & AMG Center for ALS at Mass General
Guest Speaker

Peter Foss
President, Advisory Board
ALS Finding a Cure
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

Open Label Extension

Open Label Extension

Open Label Extension

Open Label Extension
What is TREHALOSE?

- Trehalose is a disaccharide composed of 2 glucose molecules linked together.
- Found extensively in nature.
- Humans do not make trehalose, but can metabolize it.
- Oral trehalose is not absorbed due to breakdown by gut trehalase’s (<0.5%).
- Trehalase’s are found in the gut, kidney and liver and breakdown trehalose to the 2 glucose molecules.
- In this study trehalose is administered IV to bypass the gut trehalase enzymes.
- Trehalose penetrates muscle and brain.

Link to watch recorded science webinar: https://bit.ly/3L4hrdB
Study Results Timelines – Regimens A-D

*** Note: COVID-19 might impact these estimates

Q1 Q2 Q3 Q4

LPLV
(Last Participant Last Visit in the Randomized, Placebo Controlled Period)

Database Lock

Top Line Results

Study Reports

Expected LPLV: March-July 2022 (depending on the regimen and OLE choice)
Expected Database Lock: July-Oct 2022
Top Line Results: Aug-Nov 2022
Study Reports: Nov-Dec 2022

as of 5/5/22
Enrollment Updates (as of May 5, 2022)

- **51** individuals have signed informed consent
- **33** individuals have been randomized within Regimen E

Thank You

This breakthrough trial would not be possible without your participation.

Your **partnership** in research is what keeps us filled with passion, dedication, and the commitment to uncover new promising treatments for ALS.

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.
31 Sites Currently Activated for Regimen E

(as of 5/5/22)

Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!

- Lehigh Valley Health Network
- Mass General Hospital
- University of Kansas
- University of Maryland
- California Pacific Medical Center
- Northwestern University
- Virginia Commonwealth University
- University of Nebraska
- Washington University
- Wake Forest University
- Hospital for Special Care
- Saint Alphonsus Regional
- University of Massachusetts
- Duke University
- Barrow Neurological Institute
- Georgetown University
- Texas Neurology
- Beth Israel Deaconess Medical Center
- SUNY Upstate
- Spectrum Health
- Henry Ford Hospital
- Essentia Health
- University of Southern California
- University of South Florida
- University of Colorado
- Providence Brain and Spine
- University of Minnesota
- Loma Linda University
- University of Iowa
- Swedish Medical Center
- Ohio State University
Regimen E Site Activation

Sites closest to activation:

- Mayo Rochester
- University of Cincinnati
- University of Pittsburgh
- Thomas Jefferson
- University of CA, San Francisco
- Augusta University

as of 5/5/22
# Checking Site Status Online

**List of Participating Sites**

*Sites marked "Recruiting" are currently enrolling participants in regimen E.*

*Sites marked "Active, Not recruiting" are active in the platform (for example, they are following participants in regimens A-D) but are not enrolling new participants at this time. Most active sites are expected to start enrolling in regimen E soon.*

<table>
<thead>
<tr>
<th>Site</th>
<th>State</th>
<th>Enrollment Status</th>
<th>Contact Information</th>
</tr>
</thead>
<tbody>
<tr>
<td>Jefferson Weinberg ALS Center, Thomas Jefferson University</td>
<td>PA</td>
<td>Active, Not recruiting</td>
<td>Stephanie Hansen</td>
</tr>
<tr>
<td>Lehigh Valley Health Network</td>
<td>PA</td>
<td>Recruiting</td>
<td>Andrew Orzel (610-402-9543)</td>
</tr>
<tr>
<td>Penn State Hershey</td>
<td>PA</td>
<td>Active, Not recruiting</td>
<td>Heidi Runk</td>
</tr>
</tbody>
</table>
Patient Navigation
Central resource for people living with ALS

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

Weekly webinar registration: [QR Code]
https://bit.ly/3r6Nd2L

ALS Link sign-up: [QR Code]
https://bit.ly/3o2Ds3m

Upcoming Guest Speakers:
May 19th- Nadia Sethi (Platform Trial Patient Advisory Committee Member)
Travel around the US meeting Platform Trial Site Investigators

Guest speakers from partner foundations

Trehalose/SLS-005 drug science and mechanism of action webinar with Seelos Therapeutics

Special Topics:
Biomarkers, EAPs, Biostats, Science topics (epigenetics, epidemiology)