HEALEY ALS Platform Trial

Weekly Q&A – April 14, 2022

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

Kuldeep Dave, PhD
Vice President of Research
The ALS Association
ALS Research Ecosystem

Drug Development

- Biology and Genetics of ALS
- Preclinical Drug Development
- Phase I
- Phase II
- Phase III
- Biomarker Development
- Drug Approval

Managing ALS

- Assistive Technology
- Telehealth / Telemedicine
- Phase IV Medication
- Access / Quality of Life
- Patient Surveys
- Family / Caregiver Burden
- Natural History

Infrastructure, Specimens, Data, Tools, Training

149 active projects, across 10 countries, $53M commitment
Holistic Strategy to Increase # of Clinical Trials in ALS

- Assistive Technology
- Telehealth / Telemedicine
- Phase IV Medication
- Access / Quality of Life
- Patient Surveys
- Family / Caregiver Burden
- Natural History

Infrastructure, Specimens, Data, Tools, Training

- Barnett program
- Preclinical Drug Development
- Clinical Trial Awards
- Healey Trial Funding ($3M)
- NEALS, capacity grant, trial match, ThinkALS, ALS Focus, advocacy

Biology and Genetics of ALS

Risks and Causes of ALS

Drug Approval
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

- **Screening**: 
  - Regimen Assignment
  - (n=160 for each regimen)

- **Regimen A**
  - Placebo

- **Regimen B**
  - CNM-Au8
  - Placebo

- **Regimen C**
  - Verdiperstat
  - Placebo

- **Regimen D**
  - Zilucoplan
  - Placebo

- **Regimen E**
  - Pridopidine
    - Placebo
  - Trehalose
    - Placebo

**3:1 Randomization within each Regimen**

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

**Open Label Extension**
**Study Results Timelines – Regimens A-D**

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

**Q1**
- LPLV
  - (Last Participant Last Visit in the Randomized, Placebo Controlled Period)
- Database Lock

**Q2**
- Top Line Results

**Q3**
- Study Reports

**Q4**

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

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as of 4/14/22
Enrollment Updates (as of April 14, 2022)

• 32 individuals have signed informed consent

• 23 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
24 Sites Currently Activated for Regimen E

(as of 4/14/22)

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

Sites closest to activation:

- University of Colorado
- Providence Brain and Spine
- Mayo Rochester
- University of Cincinnati
- Swedish Medical Center
- Thomas Jefferson
- Loma Linda University
- Ohio State University

as of 4/14/22
# 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:
https://bit.ly/3r6Nd2L

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

Upcoming Guest Speakers:
April 28th - Kelly Gwathmey, MD (Site PI at Virginia Commonwealth University)
May 5th - Peter Foss (President, ALS Finding a Cure)
Travel around the US meeting Platform Trial Site Investigators

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

Send us webinar questions and ideas!

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

Guest speakers from partner foundations