HEALEY ALS Platform Trial

Weekly Q&A – June 2, 2022
Guest Speaker

Daniel Hevert, BS
Lead Clinical Research Coordinator
Mass General Hospital
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

**Screening**

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

**Regimen A**
- Placebo

**Regimen B**
- **3:1 Randomization** within each Regimen
  - (n=120 for active drug; n=40 for placebo)

**Regimen C**
- Placebo

**Regimen D**
- Verdiperstat
  - Placebo

**Regimen E**
- CNM-Au8
  - Placebo

- Pridopidine
  - Placebo

- Trehalose
  - Placebo

- **Zilucoplan**
  - Placebo

- **Open Label Extension**

- **Shared Placebo**
Enrollment Updates (as of June 2, 2022)

• 68 individuals have signed informed consent

• 44 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
36 Sites Currently Activated for Regimen E

(as of 6/2/22)

Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!
# 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>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:
June 9th: Honora Dalamagas and Natalie Cartwright (Hospital for Special Care, CT)
June 16th: Bruce Rosenblum (EAP Patient Advisory Committee Member)
June 23rd: Jackie Whitesell, MD (Investigator at Saint Alphonsus Medical Center, Idaho)
The ALS Association/Northeast ALS Consortium Educational Webinar

**Healey ALS Platform Trial Regimen E:**
A Participant's Guide

**Friday, June 17, 2022**
5:00 - 6:00 PM EDT

The purpose of this webinar is to provide an overview of how SLS-005 (trehalose) may help to slow ALS and illustrate what potential participants can expect when enrolling in Regimen E of the Healey ALS Platform Trial. The presenters will review some of the unique aspects of the regimen related to the intravenous administration.


Recording will later be available under “educational webinars” on neals.org