HEALEY ALS Platform Trial

Weekly Q&A – June 9, 2022
Guest Speaker

Honora Dalamagas, RN, MSN, CNS
Research Manager/Clinical Research Coordinator

Natalie Cartwright, MS
Clinical Research Coordinator

Hospital for Special Care, New Britain CT
Participating in a Clinical Trial at HFSC

**Recruitment**
- Clinic sees ≈ 250 people with ALS every year
- Neurologist introduces research
- Meet with Coordinator during Clinic Visit, if interested

**Research Visits**
- Followed by one Coordinator
- All assessments in one location
- Efficient and Comfortable

**Between Visits**
- Communication with Research team
- Continuity of care
Keys to Success as a High Enrolling Site

- Top priority = Safety
- Excellent Care ➔ Retention
- Communication
- Documentation
- Organization
- Consistency
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

Zilucoplan

- Placebo

Verdiperstat

- Placebo

CNM-Au8

- Placebo

Pridopidine

- Placebo

Trehalose

- Placebo

Shared Placebo

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

Open Label Extension
Enrollment Updates (as of June 9, 2022)

• 71 individuals have signed informed consent

• 46 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
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

Upcoming Guest Speakers:
June 16th - Bruce Rosenblum (EAP Patient Advisory Committee Member)
June 23rd - Jackie Whitesell, MD (Investigator at Saint Alphonsus Medical Center, Idaho)
June 30th - Gale Kittle, RN, MPH (Director, Outcomes Measurement Training)

https://bit.ly/3r6Nd2L
https://bit.ly/3o2Ds3m
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.

https://bit.ly/3aeT5kc

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