Guest Speaker

Nadia Sethi, DDS
Patient Advisory Committee Member
Introduction

• Innovative trial design
• Momentum created by Healey Platform Trial
• Incorporating community voice

Nadia Sethi
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Healey Platform Trial
Patient Advisory Committee

- Weekly Q & A with Dr. Cudkowicz and Dr. Paganoni
- Enrollment updates
- Expanded Access Program in parallel to active clinical trial
- Communications about milestones
- Model for other organizations
- Learning experience for advocates
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial.

**Screening**
- Regimen A (n=160 for each regimen)
- Regimen B
- Regimen C
- Regimen D
- Regimen E (n=120 for active drug; n=40 for placebo)

**Regimen Assignment**

**3:1 Randomization within each Regimen**
- Zilucoplan
  - Placebo
- Verdiperstat
  - Placebo
- CNM-Au8
  - Placebo
- Pridopidine
  - Placebo
- Trehalose
  - Placebo
- Shared Placebo

**Open Label Extension**
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 Database Lock: July-Oct 2022
Top Line Results: Aug-Nov 2022
Study Reports: Nov-Dec 2022

as of 5/19/22
Enrollment Updates (as of May 19, 2022)

• 59 individuals have signed informed consent

• 36 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.
35 Sites Currently Activated for Regimen E

(as of 5/19/22)

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

<table>
<thead>
<tr>
<th>Sites closest to activation:</th>
</tr>
</thead>
<tbody>
<tr>
<td>➢ University of Pittsburgh</td>
</tr>
<tr>
<td>➢ Augusta University</td>
</tr>
<tr>
<td>➢ University of Washington</td>
</tr>
<tr>
<td>➢ Vanderbilt University</td>
</tr>
<tr>
<td>➢ Indiana University</td>
</tr>
<tr>
<td>➢ Penn State Hershey</td>
</tr>
</tbody>
</table>

as of 5/19/22
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>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: TBD
Send us webinar questions and ideas!

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)