HEALEY ALS Platform Trial

Weekly Q&A – May 12, 2022

Healey Center
Sean M. Healey & AMG Center for ALS at Mass General
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

- **Regimen A** (n=160 for each regimen)
  - Zilucoplan
  - Placebo

- **Regimen B**
  - Verdiperstat
  - Placebo

- **Regimen C**
  - CNM-Au8
  - Placebo

- **Regimen D**
  - Pridopidine
  - Placebo

- **Regimen E**
  - Trehalose
  - Placebo

Shared Placebo

3:1 Randomization within each Regimen

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

Open Label Extension

(n=160 for each regimen)
**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)
- **Q2**: Database Lock
- **Q3**: Top Line Results
- **Q4**: Study Reports

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

(as of 5/12/22)
Enrollment Updates (as of May 12, 2022)

• 55 individuals have signed informed consent

• 34 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.
33 Sites Currently Activated for Regimen E

(as of 5/12/22)

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:

- Mayo Rochester
- University of Pittsburgh
- University of CA, San Francisco
- Augusta University
- University of Washington
- Vanderbilt University

*as of 5/12/22*
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>

https://bit.ly/3g2NZr5

as of 5/12/22
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:
May 19th- Nadia Sethi (Platform Trial Patient Advisory Committee Member)
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)