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

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

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**: Database Lock
- **Q2**: LPLV (Last Participant Last Visit in the Randomized, Placebo Controlled Period)
- **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 5/26/22
Enrollment Updates (as of May 26, 2022)

- 64 individuals have signed informed consent
- 42 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 5/26/22)

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 Pittsburgh
- Augusta University
- Vanderbilt University
- Indiana University
- Penn State Hershey

as of 5/26/22
# 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</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>(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](https://bit.ly/3g2NZr5)

as of 5/26/22
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:
June 2nd - Daniel Hevert (Lead Coordinator, Mass General Hospital)
June 9th - Honora Dalamagas and Natalie Cartwright (Hospital for Special Care)
June 16th - Bruce Rosenblum (EAP Patient Advisory Committee Member)
Send us webinar questions and ideas!

Travel around the US meeting Platform Trial Site Investigators

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

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

Guest speakers from partner foundations