The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

Regimen A
Regimen B
Regimen C
Regimen D
Regimen E

3:1 Randomization within each Regimen

- Regimen A: Zilucoplan, Verdiperstat, CNM-Au8
- Regimen B: Placebo
- Regimen C: Placebo
- Regimen D: Pridopidine
- Regimen E: Trehalose

Shared Placebo

(n=160 for each regimen)
(n=120 for active drug; n=40 for placebo)
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 LPLV: March-July 2022** (depending on the regimen and OLE choice)

**Expected Database Lock: May-Sept 2022**

**Top Line Results: July-Sept 2022**

**Study Reports: Oct-Dec 2022**

as of 3/31/22
Enrollment Updates (as of March 31, 2022)

• 21 individuals have signed informed consent

• 15 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
21 Sites Currently Activated for Regimen E

(as of 3/31/22)

Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!

- Lehigh Valley Health Network
- Mass General Hospital
- University of Kansas
- University of Maryland
- California Pacific Medical Center
- Northwestern University
- Virginia Commonwealth University
- University of Nebraska
- Washington University
- Wake Forest University
- Hospital for Special Care
- Saint Alphonsus Regional
- University of Massachusetts
- Duke University
- Barrow Neurological Institute
- Georgetown University
- Texas Neurology
- Beth Israel Deaconess Medical Center
- SUNY Upstate
- Spectrum Health
- Henry Ford Hospital
### Sites closest to activation:

- University of Colorado
- University of South Florida
- Providence Brain and Spine
- Ohio State University
- Essentia Health
- University of Cincinnati
- Loma Linda University
- Swedish Medical Center

**as of 3/31/22**
# 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>Jefferson Weinberg ALS Center, Thomas Jefferson University</td>
<td>PA</td>
<td>Active, Not recruiting</td>
<td>Stephanie Hansen</td>
</tr>
<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](https://bit.ly/3g2NZr5)

As of 3/31/22
New & Updated Webpages on Expanded Access

Expanding Access to Investigational Products

HEALEY ALS Platform Trial Multicenter Companion

Expanded Access

CNM-Au8, by Clene Nanomedicine

Pridopidine, by Prilenia Therapeutics: Multiple Locations

Verdiperstat, by Biohaven: Multiple Locations

Expanded Access Information for Patients

Frequently asked questions and helpful online resources for people interested in participating in an EAP.

Learn about EAPs →

https://bit.ly/3qK2INq

as of 3/31/22
New & Updated Webpages on Expanded Access

Common Questions about Expanded Access Protocols

What is Expanded Access?

How is Expanded Access different from clinical trials?

How do I learn more about participating in an Expanded Access Protocol?

Why would I participate in an Expanded Access Protocol?

How are Expanded Access Protocols funded?

- Watch an educational webinar about EAPs hosted by the Northeast ALS Consortium (NEALS)
- Visit the NEALS website
- Visit the US FDA website
- Read the scientific journal article Accessing Investigational Products Outside of a Trial: Considerations for Neuromuscular Providers

https://bit.ly/3tPNDMj
Patient Navigation
Central resource for people living with ALS

Phone: 833-425-8257 (HALT ALS)
E-mail: healeyalsplatform@mgh.harvard.edu

Catherine Small

Weekly webinar registration: https://bit.ly/3r6Nd2L

ALS Link sign-up: https://bit.ly/3o2Ds3m

Allison Bulat

Upcoming Guest Speakers:
April 14th - Kuldip Dave, PhD (VP of Research, The ALS Association)
May 5th - Peter Foss (President, ALS Finding a Cure)
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)