Guest Speaker

Jaimin Shah, MD
Platform Trial Site Principal Investigator
Mayo Clinic, Jacksonville FL
MULTIDISCIPLINARY CLINIC

Bjorn Oskarsson, MD  
Jaimin Shah, MD  
Jamie Zernicke, RN  
Jennifer Reidell, MSW, LCSW  
Melissa Fehrenkamp, RRT  
Angelica Gicalone, OT  
Gleydiane DeOliviera, PT  
Amanda Lazo, OT  
Sarah Reising, CCC-SLP  
Amanda Vignola, CCC-SLP  
Melissa Stewart, RDN, LD/N
RESEARCH PROGRAMS

• Clinical trials
• Mayo Clinic Florida ALS Registry
• Biospecimen collection
• ALS brain bank and autopsy program

Clinical research coordinator team

Megan Donahue
Alex Burch
Collette McHugh Strong
Lisa Thuro
Jany Paulett
THANK YOU!
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

Regimen A
- CNM-Au8 Placebo
- Verdiperstat Placebo
- Zilucoplan Placebo

Regimen B
- Zilucoplan
- Verdiperstat
- CNM-Au8

Regimen C
- Pridopidine Placebo

Regimen D
- Trehalose Placebo

Regimen E
- Shared Placebo

3:1 Randomization within each Regimen
(n=160 for each regimen)
(n=120 for active drug; n=40 for placebo)

Open Label Extension
Study Results Timelines – Regimens A-D

*** Note: COVID-19 might impact these estimates

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

LPLV
(Last Participant Last Visit in the Randomized, Placebo Controlled Period)

Database Lock

Top Line Results
Study Reports

as of 3/24/22
Enrollment Updates (as of March 24, 2022)

• 21 individuals have signed informed consent

• 12 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
20 Sites Currently Activated for Regimen E

(as of 3/24/22)

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
Regimen E Site Activation

**Sites closest to activation:**

- University of Colorado
- Henry Ford Hospital
- University of South Florida
- Providence Brain and Spine
- Ohio State University
- Essentia Health
- University of Cincinnati

as of 3/24/22
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,</td>
<td>PA</td>
<td>Active, Not recruiting</td>
<td>Stephanie Hansen</td>
</tr>
<tr>
<td>Thomas Jefferson University</td>
<td></td>
<td></td>
<td></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

as of 3/24/22
Patient Navigation
Central resource for people living with ALS

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

Weekly webinar registration: [QR code]
https://bit.ly/3r6Nd2L

ALS Link sign-up: [QR code]
https://bit.ly/3o2Ds3m

Upcoming Guest Speaker:
April 14th- Kuldip Dave, PhD (VP of Research, The ALS Association)
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)
The ALS Association/Northeast ALS Consortium Educational Webinar

**The Latest on Expanded Access Protocols in ALS**

Moderator: Jinsy A. Andrews, MD, MSc, Columbia University Medical Center
Panelists: Merit Cudkowicz, MD, MSc, Sean M. Healey & AMG Center for ALS at Mass General Hospital, Sabrina Paganoni, MD, PhD, Sean M. Healey & AMG Center for ALS at Mass General Hospital, Philip Green, and Sandy Morris

**Recording Available:**