The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

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

3:1 Randomization within each Regimen

- Zilucoplan
  - Placebo
- Verdiperstat
  - Placebo
- CNM-Au8
  - Placebo
- Pridopidine
  - Placebo
- Trehalose
  - Placebo
- Shared Placebo

Open Label Extension

2022
Regimens A, B, C and D completed enrollment!

- 162 individuals were randomized within Regimen A
- 167 individuals were randomized within Regimen B
- 161 individuals were randomized within Regimen C
- 163 individuals were randomized within Regimen D

365 participants have entered the Open Label Extension (OLE)

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PRESS RELEASE • 6 MINUTE READ • JAN 6, 2022

The HEALEY ALS Platform Trial achieves enrollment goal for its fourth regimen (pridopidine)

Marcela Quintanilla-Dieck • 617-726-0954 • mquintanilladieck@mgh.harvard.edu

Thank You

This breakthrough trial would not be possible without your partnership
52 Sites
Activated for Regimens A-D

- Texas Neurology
- Mass General Hospital
- UTHSCSA
- Hospital for Special Care
- Holy Cross Hospital
- Thomas Jefferson
- Houston Methodist
- Henry Ford Health System
- Barrow Neurological Institute
- Ohio State University
- Northwestern University
- University of Chicago
- Wake Forest
- University of Nebraska
- Loma Linda University
- University of Washington
- University of Iowa
- Washington University
- University of Pennsylvania
- University of Michigan
- California Pacific Medical Center
- Penn State Hershey
- UMass Worcester
- University of Miami
- University of Colorado
- Cedars-Sinai
- University of Florida
- University of South Florida
- Columbia University
- University of Virginia
- Emory University
- University of Maryland
- SUNY Upstate
- Beth Israel Deaconess
- Temple University
- Dartmouth-Hitchcock
- Medical College of Wisconsin
- Spectrum Health
- University of Missouri
- University of Minnesota
- Johns Hopkins University
- University of CA Irvine
- University of Kansas
- Vanderbilt University
- University of Kentucky
- Mayo Rochester
- Duke University
- Neurology Associates
- Ochsner Health System
- Mayo Clinic Florida
- St. Louis University
- Providence Brain and Spine
2 Additional Sites Activated for Regimen D

- Texas Neurology
- Mass General Hospital
- UTHSCSA
- Hospital for Special Care
- Holy Cross Hospital
- Thomas Jefferson
- Houston Methodist
- Henry Ford Health System
- Barrow Neurological Institute
- Ohio State University
- Northwestern University
- University of Chicago
- Wake Forest
- University of Nebraska
- Loma Linda University
- University of Washington
- University of Iowa
- Washington University
- University of Pennsylvania
- University of Michigan
- California Pacific Medical Center
- Penn State Hershey
- UMass Worcester
- University of Miami
- University of Colorado
- Cedars-Sinai
- University of Florida
- University of South Florida
- Columbia University
- University of Virginia
- Emory University
- University of Maryland
- SUNY Upstate
- Beth Israel Deaconess
- Temple University
- Dartmouth-Hitchcock
- Medical College of Wisconsin
- Spectrum Health
- University of Missouri
- University of Minnesota
- Johns Hopkins University
- University of CA Irvine
- University of Kansas
- Vanderbilt University
- University of Kentucky
- Mayo Rochester
- Duke University
- Neurology Associates
- Ochsner Health System
- Mayo Clinic Florida
- St. Louis University
- Providence Brain and Spine
- Georgetown University
- University of Southern California
20 New Sites to be Activated for Regimen E
New site spotlight: Essentia Health

First ever ALS platform drug trial comes to Duluth
## 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>
Regimen E Site Activation Plans

- Plan to activate about 20 sites per month
- Target number: about 65 sites total

Sites on track for January activation:

- Lehigh Valley Health Network - activated!
- Mass General Hospital
- University of Massachusetts
- Northwestern University
- Hospital for Special Care
- University of Maryland
- Virginia Commonwealth University
- University of Nebraska
- University of Cincinnati
- Spectrum Health
- University of Colorado
- Georgetown University
- Barrow Neurological Institute
- Indiana University
- University of South Florida
- University of Kansas Medical Center
- Wake Forest Health Sciences
- SUNY Upstate
- Washington University
- Beth Israel Deaconess Medical Center
- California Pacific Medical Center

as of 1/27/22
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:
Feb 10th- Ximena Arcila-Londono, MD (Henry Ford, Detroit MI)
Feb 17th- Jim Caress, MD (Wake Forest, Winston-Salem NC)