Guest Speaker

Andrea Swenson, MD
Platform Trial Site Principal Investigator
University of Iowa, Iowa City IA
University of Iowa ALS Center

Medical Director: Andrea Swenson, MD
Our three-part mission

Education
Teaching and training tomorrow’s health care providers

Research
Bringing new discoveries and new treatments

Patient Care
Providing high-quality clinical care services
Patient Care

- Rural population
- ALS patients travel a total of 180 miles for an in person clinic visit (on average)

310 miles
Patient Care

- Multidisciplinary Clinics
  - UIHC Main Campus – Iowa City
  - Outreach Clinic – Des Moines
Education

• Clinical rotations for
  – Neurology residents and fellows
  – Medical students
  – PT, OT, speech pathology, nursing students
Research

- Clinical trials
  - Platform Trial
  - Cytokinetics
  - AI Therapeutics (C9orf72)

- Opportunities for patients who do not qualify for trials
  - Focus group (open to all)
  - EAP
Contacts

• For clinical trials
  – Heena Olalde, RN, MSN
    • heena-olalde@uiowa.edu
    • 319-356-8326
  – Emily McDonnell, RN, MSN
    • emily-mcdonnell@uiowa.edu
    • 319-356-8744

• For ALS multidisciplinary clinic
  – 319-356-8958
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

Zilucoplan

Placebo

Verdiperstat

Placebo

CNM-Au8

Placebo

Pridopidine

Placebo

Trehalose

Placebo

Shared Placebo

(n=120 for active drug; n=40 for placebo)
The HEALEY ALS Platform Trial announces first participants enrolled for investigational product SLS-005 (Trehalose)

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

HEALEY ALS Platform Trial Update: Zilucoplan arm stopped early for futility

Marcela Quintanilla-Dieck - 617-724-0954
mquintanilladieck@mgh.harvard.edu

“We are extremely grateful to the many participants in the trial and the members of our patient advisory group for their contributions to ALS science and therapy development.”

Merit Cudkowicz, MD, MSc
Director, Healey & AMG Center for ALS, Massachusetts General Hospital
14 Sites Currently Activated for Regimen E

- 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

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

(as of 3/3/22)
### Sites closest to activation:

- Spectrum Health
- University of Colorado
- University of South Florida
- Barrow Neurological Institute
- SUNY Upstate
- Henry Ford
- Beth Israel Deaconess
- Providence Brain and Spine
- Ohio State University
- Essentia Health

As of 3/3/22
# 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>
Regimen E: Trehalose (SLS-005), by Seelos Therapeutics – Active, Recruiting

Trehalose is a low molecular weight disaccharide (0.342 kDa) that crosses the blood-brain barrier, stabilizes proteins and, importantly, activates autophagy, which is the process that clears pathologic material from cells. In animal models of ALS, treatment with trehalose delayed onset of disease, prolonged survival and preserved motor function and motor neurons in the spinal cord. Trehalose activates autophagy through the activation of Transcription Factor EB (TfEB), a key factor in lysosomal and autophagy gene expression. Activation of TfEB is an emerging therapeutic target for a myriad of diseases with pathologic accumulation of storage material. The drug is manufactured by Seelos Therapeutics, Inc., a clinical-stage biopharmaceutical company focused on the development of therapies for central nervous system disorders and rare diseases.

We will host a webinar about the science behind Trehalose on March 10 at 5:00 pm Eastern Time. Register here.

Download brochure


Regimen E Platform Trial

Regimen E
SLS-005 (trehalose)
Developed by Seelos Therapeutics

Regimen E is testing the investigational product SLS-005 (trehalose). The investigational products included in the HEALEY ALS Platform Trial were selected by a team of experts after careful review of the science supporting the potential of the product for ALS.

Please discuss the potential benefits and risks of this investigational product with your study physician.

About Regimen E:

- Regimen E is enrolling approximately 150 participants to evaluate the safety and efficacy of trehalose as a potential treatment for ALS.
- 3:1 Active Drug to Placebo Ratio: Participants who enroll in the trial are assigned to the active treatment group and 1 in 4 chance of being assigned to the placebo group.
- Open Label Extension (OLE): Upon completion of the 24-week trial, participants will have the option to enroll in the OLE for trehalose. During OLE, all participants will receive active study drug.

To see if you qualify, please review the list of eligibility criteria:
https://bit.ly/3ClYHn3

For general questions about the HEALEY ALS Platform Trial, contact the Patient Navigator:
healeyalsplatform@mgh.harvard.edu
833-476-8297 (HAL ALS)

Visit our website to learn more about current and future regimens:
http://www.healys.org

Q&A for Regimen E:

- How is this drug administered?
  A. Trehalose is administered as weekly intravenous (IV) infusions. The first four infusions must be completed in patients in the study center, but thereafter the option to transition to at-home infusions starting after week 4 of the study.

- What does this drug do?
  A. Trehalose is a naturally occurring low molecular weight disaccharide (sugar). When administered through IV, trehalose crosses the blood-brain barrier, stabilizes proteins and, importantly, activates autophagy, which is the process that clears material from cells. Trehalose may delay the progression of ALS by reducing mutant protein aggregation and improving the clearance of toxic material from motor neuron.

Additional questions?
Register to attend the Weekly Platform Trial Q&A Webinars:

Stay Connected to the Platform Trial

More investigational products are anticipated to be added to the HEALEY ALS Platform Trial through support by pharma, foundation partners, philanthropy, federal, and other funding initiatives.

Visit our website to learn more about current and future regimens:
http://www.healys.org

View map and contact info for participating research centers:

Sign up for the ALS Link to hear about ALS news and research:
http://bit.ly/3JZx5Wf

Now Enrolling

Regimen E: Brochure Available Online
Patient Navigation
Central resource for people living with ALS

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

Upcoming Guest Speakers:

**March 10th** - Trehalose Science Webinar with Seelos Therapeutics
**March 17th** - Edward Kasarskis, MD PhD (University of Kentucky, Lexington KY)
**March 24th** - Jaimin Shah, MD (Mayo Clinic, Jacksonville FL)

ALS Link sign-up: [https://bit.ly/3o2Ds3m](https://bit.ly/3o2Ds3m)
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)