

# HEALEY ALS Platform Trial

Weekly Q&A – March 3, 2022



## Healey Center

Sean M. Healey & AMG Center  
for ALS at Mass General



The AMG Foundation

# Guest Speaker

**Andrea Swenson, MD**

Platform Trial Site Principal Investigator  
University of Iowa, Iowa City IA





Celebrating 150 years of advancing medical research, education, and patient care to help people live longer, healthier lives



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# University of Iowa ALS Center

**Medical Director: Andrea Swenson, MD**

➤ CHANGING MEDICINE.  
➤ CHANGING LIVES.®

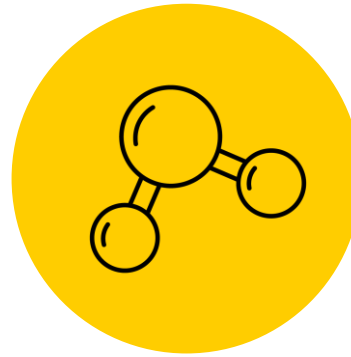
# Our three-part mission

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## 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

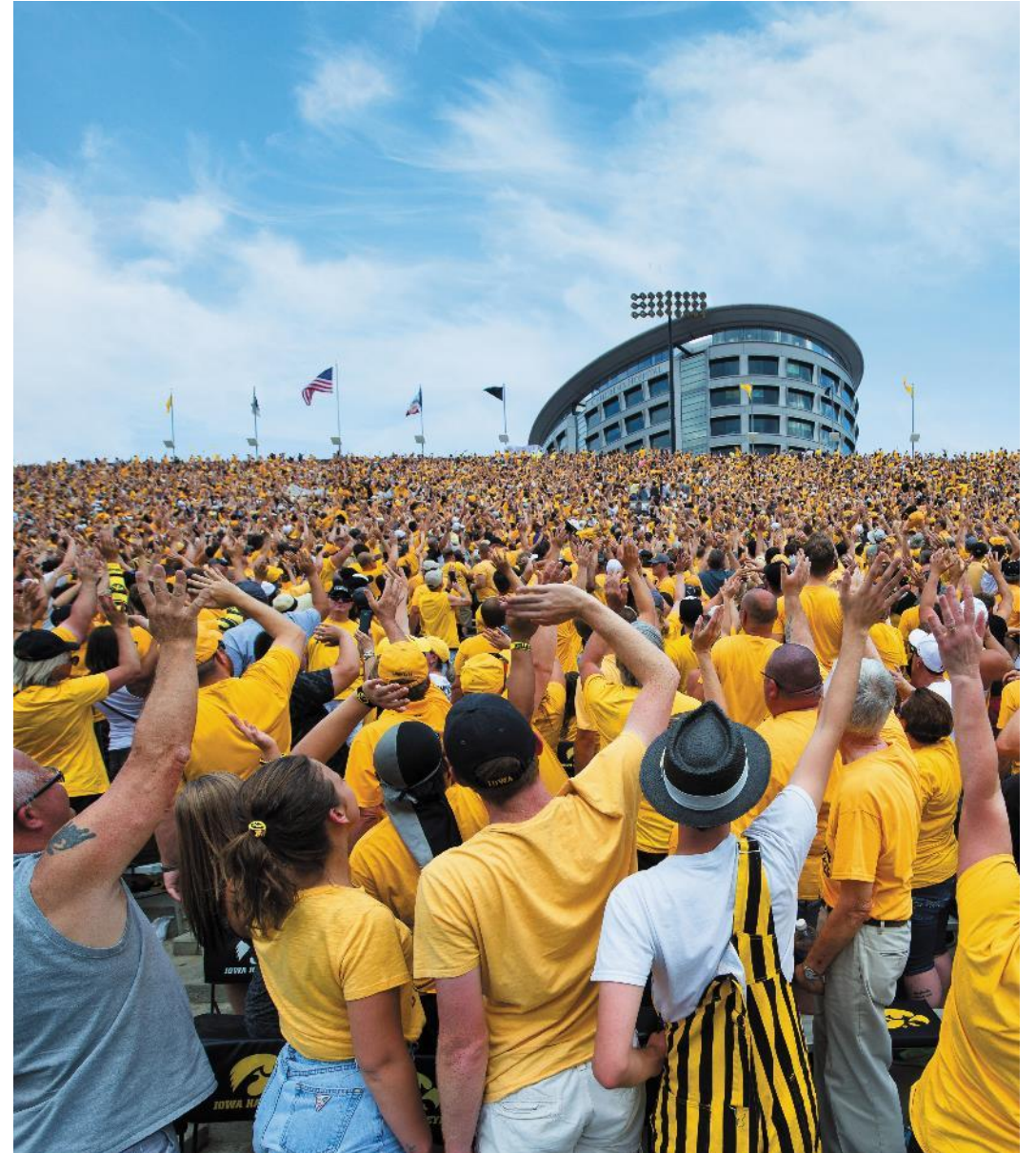
- Multidisciplinary Clinics
  - UIHC Main Campus– Iowa City
  - Outreach Clinic– Des Moines



# Education

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- Clinical rotations for
  - Neurology residents and fellows
  - Medical students
  - PT, OT, speech pathology, nursing students



# Research

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- Clinical trials
  - Platform Trial
  - Cytokinetics
  - AI Therapeutics (C9orf72)
- Opportunities for patients who do not qualify for trials
  - Focus group (open to all)
  - EAP



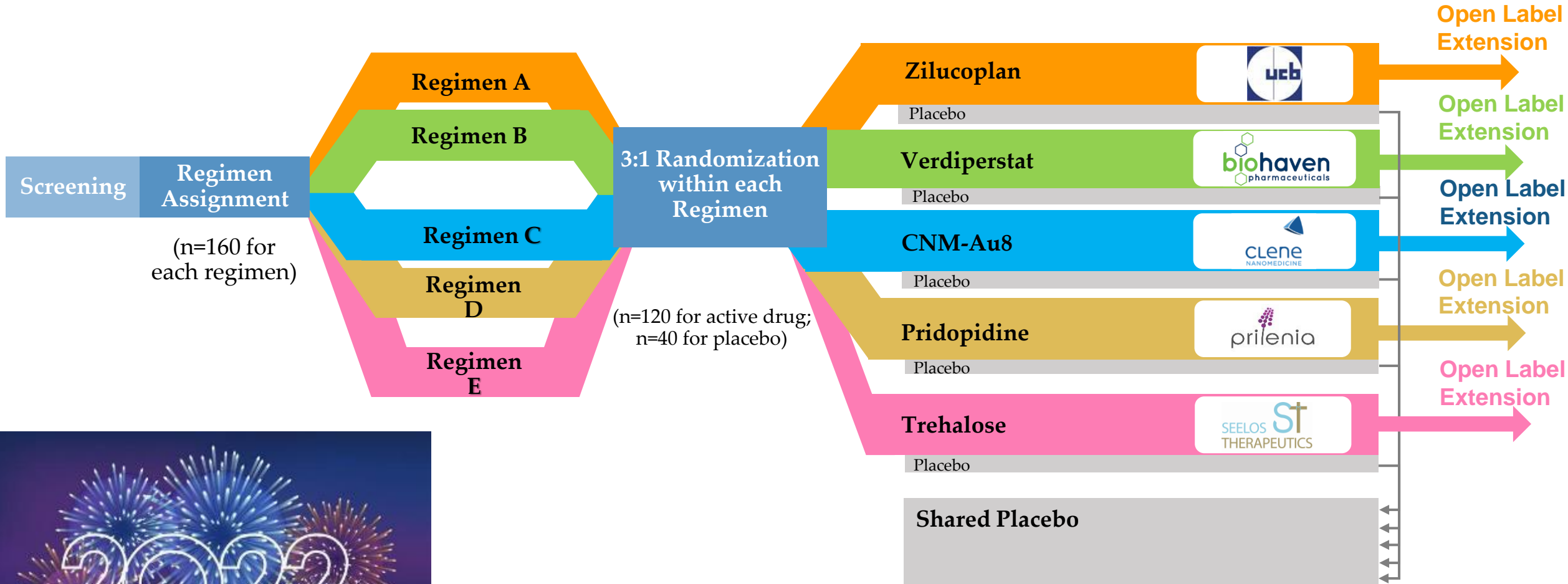


# Contacts

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- For clinical trials
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    - [heena-olalde@uiowa.edu](mailto:heena-olalde@uiowa.edu)
    - 319-356-8326
  - Emily McDonnell, RN, MSN
    - [emily-mcdonnell@uiowa.edu](mailto:emily-mcdonnell@uiowa.edu)
    - 319-356-8744
- For ALS multidisciplinary clinic
  - 319-356-8958

# The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



PRESS RELEASE · 5 MINUTE READ · FEB | 28 | 2022

## 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](mailto:mquintanilladieck@mgh.harvard.edu)

PRESS RELEASE · 5 MINUTE READ · MAR | 1 | 2022

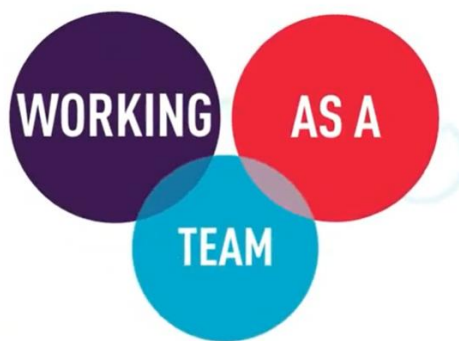
## HEALEY ALS Platform Trial Update: Zilucoplan arm stopped early for futility

Marcela Quintanilla-Dieck · 617-724-0954  
· [mquintanilladieck@mgh.harvard.edu](mailto:mquintanilladieck@mgh.harvard.edu)

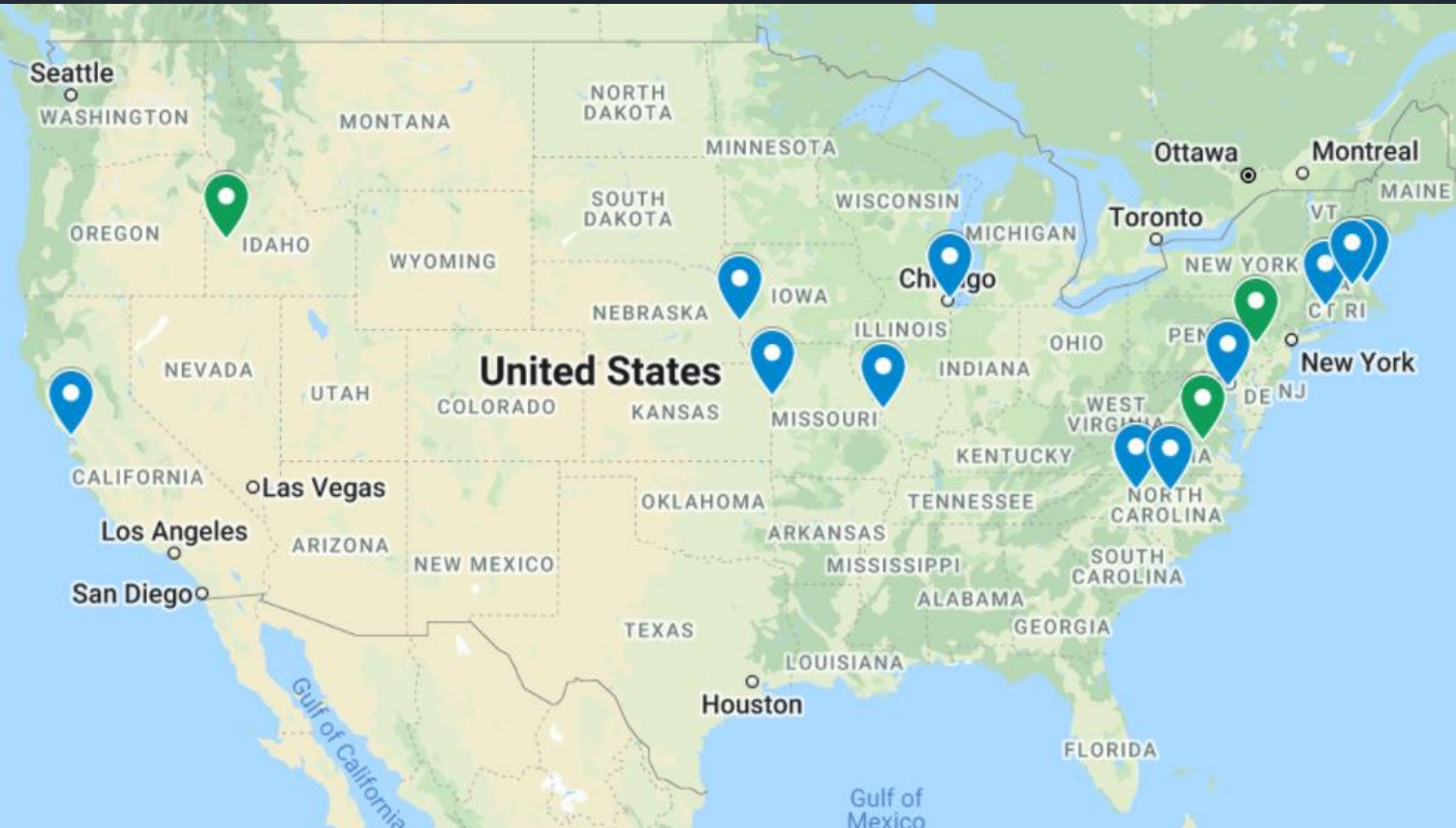
*“We are extremely grateful to the many participants in the trial and the members of our patient advisory group for their contributions to ALS science and therapy development.”*

Merit Cudkowicz, MD, MSc

Director, Healey & AMG Center for ALS, Massachusetts General Hospital



# 14 Sites Currently Activated for Regimen E



(as of 3/3/22)

- ✓ 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 Alphonus 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!*

# Regimen E Site Activation

## 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

# 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.*

Site	State	Enrollment Status	Contact Information
Jefferson Weinberg ALS Center, Thomas Jefferson University	PA	Active, Not recruiting	<a href="#">Stephanie Hansen</a>
<b>Lehigh Valley Health Network</b>	<b>PA</b>	<b>Recruiting</b>	<b><a href="#">Andrew Orzel</a></b> <b><a href="#">(610-402-9543)</a></b>
Penn State Hershey	PA	Active, Not recruiting	<a href="#">Heidi Runk</a>



<https://bit.ly/3g2NZr5>

# Regimen E- Brochure Available Online

Now Enrolling

## 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](#)



<https://bit.ly/3CIYHn3>



**Healey Center**  
Sean M. Healey & AMG Center  
for ALS at Mass General

### HEALEY ALS 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.


**Visit our website to learn more about what to expect in the trial process:**  
<https://bit.ly/3EXRdR8>



### About Regimen E:

- Regimen E is enrolling approximately 160 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 have a 3 in 4 chance of being assigned to the active treatment group and a 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 may qualify, please review the list of eligibility criteria:**  
<https://bit.ly/3Qctvnm>



For general questions about the HEALEY ALS Platform Trial, **Contact the Patient Navigator**  
[healeyalsplatform@mgh.harvard.edu](mailto:healeyalsplatform@mgh.harvard.edu)  
833-425-8257 (HALT ALS)

### Q&A for Regimen E:

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

**Q: Has this drug been studied before?**  
**A:** Yes. In previous studies using cell and mouse models of ALS, trehalose was shown to increase the clearance of TDP-43 (helping cells remove toxic proteins), decrease SOD1 and SQSM1/p62 aggregation (helping cells prevent the accumulation of toxic proteins), delay disease progression, and preserve motor neurons in the spinal cord. In prior Phase II trials involving patients with Oculopharyngeal Muscular Dystrophy and Spinocerebellar Ataxia Type 3, trehalose showed a favorable safety profile. The HEALEY ALS Platform Trial is the first trial studying trehalose in patients with ALS.

**Q: 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 materials from motor neurons.

### Additional questions?

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




<https://bit.ly/3DvkJTa>

### 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 fundraising initiatives.

**Visit our website to learn more about current and future regimens:**




<https://bit.ly/31EKT98>

**View map and contact info for participating research centers:**



<https://bit.ly/3IICv9t>

**Sign up for the ALS Link to hear about ALS news and research:**



<https://bit.ly/3EH2eMT>

# Patient Navigation

## Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: [healeyalsplatform@mgh.harvard.edu](mailto:healeyalsplatform@mgh.harvard.edu)

Weekly webinar  
registration:



<https://bit.ly/3r6Nd2L>

ALS Link sign-up:



<https://bit.ly/3o2Ds3m>

### Upcoming Guest Speakers:

**March 10<sup>th</sup>- Trehalose Science Webinar with Seelos Therapeutics**

**March 17<sup>th</sup>- Edward Kasarskis, MD PhD (University of Kentucky, Lexington KY)**

**March 24<sup>th</sup>- Jaimin Shah, MD (Mayo Clinic, Jacksonville FL)**

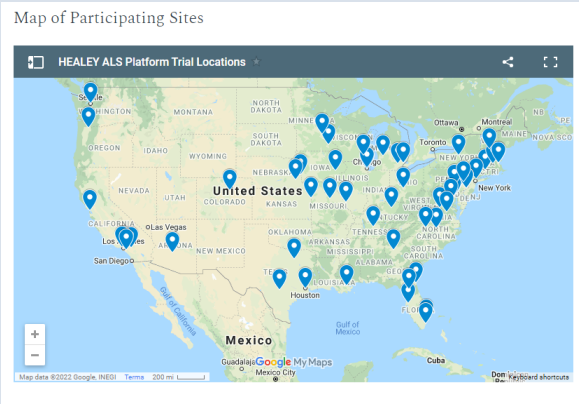


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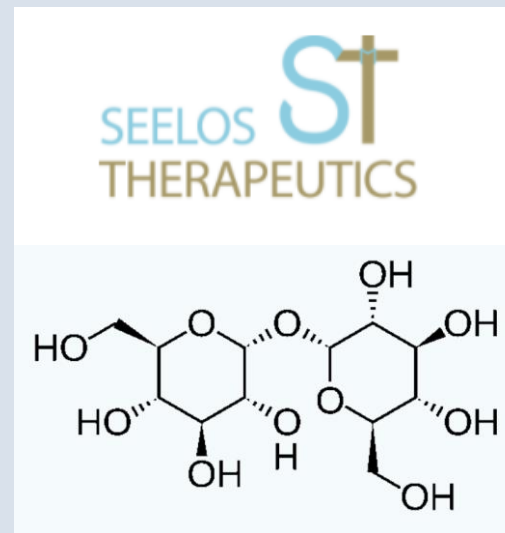
# Send us webinar questions and ideas!

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)