

HEALEY ALS Platform Trial

Weekly Q&A – May 5, 2022



Healey Center

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



The AMG Foundation



Guest Speaker

Peter Foss

President, Advisory Board
ALS Finding a Cure





Healey Center

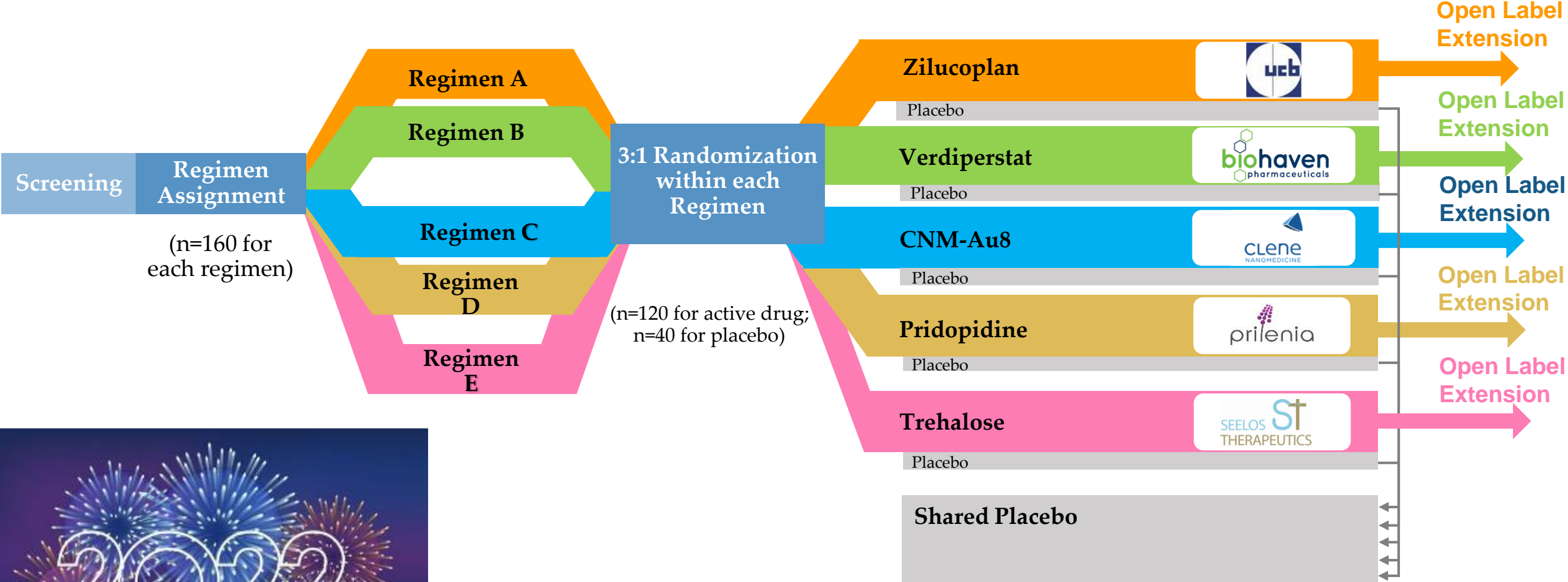
Sean M. Healey & AMG Center
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Speed

Leadership

Process

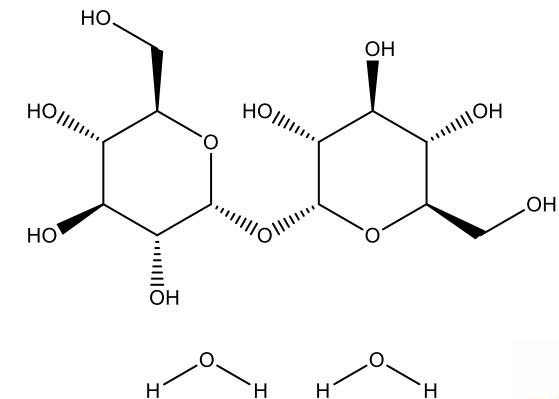
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



What is TREHALOSE ?

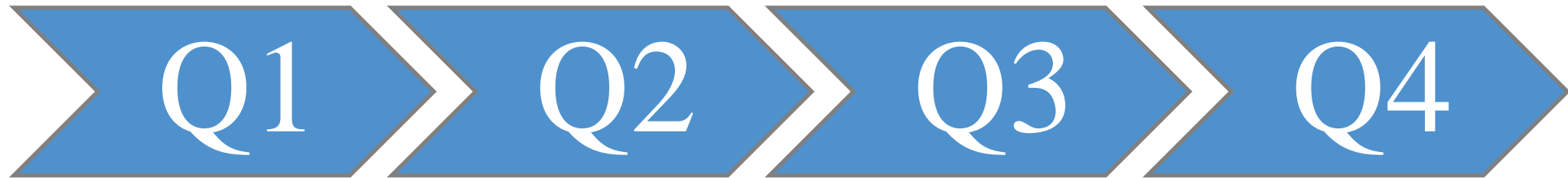
- Trehalose is a disaccharide composed of 2 glucose molecules linked together
- Found extensively in nature
- Humans do not make trehalose, but can metabolize it
- Oral trehalose is not absorbed due to breakdown by gut trehalase's (<0.5%)
- Trehalase's are found in the gut, kidney and liver and breakdown trehalose to the 2 glucose molecules
- In this study trehalose is administered IV to bypass the gut trehalase enzymes
- Trehalose penetrates muscle and brain

Link to watch recorded science webinar: <https://bit.ly/3L4hrdB>



Study Results Timelines – Regimens A-D

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



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: July-Oct 2022

Top Line Results: Aug-Nov 2022

Study Reports: Nov-Dec 2022

Enrollment Updates (as of May 5, 2022)

- **51** individuals have signed informed consent
- **33** individuals have been randomized within Regimen E

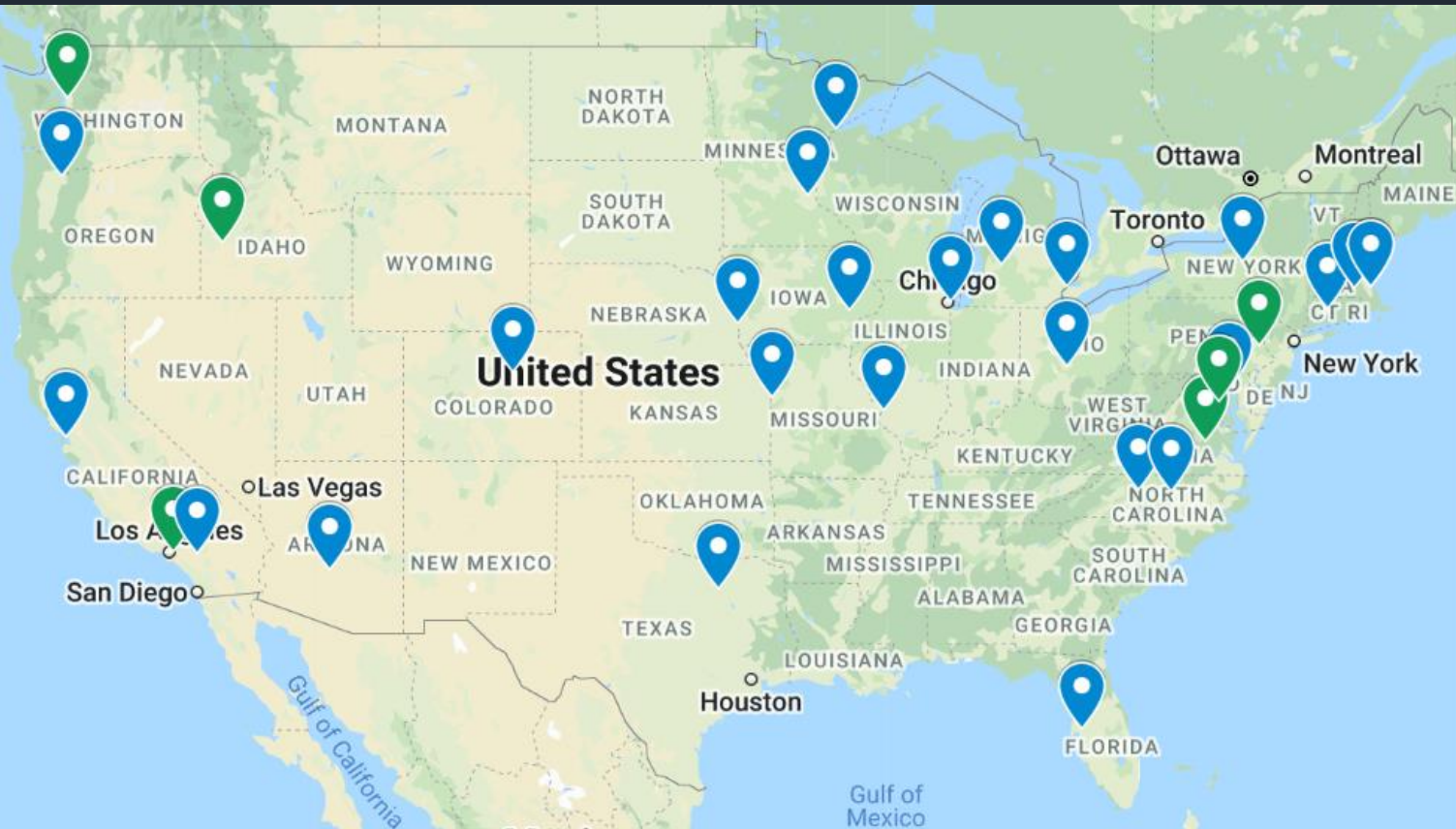
Thank You

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

31 Sites Currently Activated for Regimen E



(as of 5/5/22)

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
- ✓ Essentia Health
- ✓ University of Southern California
- ✓ University of South Florida
- ✓ University of Colorado
- ✓ Providence Brain and Spine
- ✓ University of Minnesota
- ✓ Loma Linda University
- ✓ University of Iowa
- ✓ Swedish Medical Center
- ✓ Ohio State University

Regimen E Site Activation

Sites closest to activation:

- Mayo Rochester
- University of Cincinnati
- University of Pittsburgh
- Thomas Jefferson
- University of CA, San Francisco
- Augusta University

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	Stephanie Hansen
Lehigh Valley Health Network	PA	Recruiting	Andrew Orzel (610-402-9543)
Penn State Hershey	PA	Active, Not recruiting	Heidi Runk



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

Patient Navigation

Central resource for people living with ALS



Catherine Small

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:

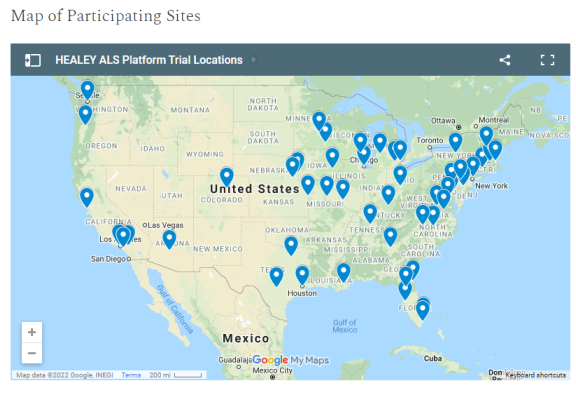
May 19th- Nadia Sethi (Platform Trial Patient Advisory Committee Member)



Allison Bulat

Send us webinar questions and ideas!

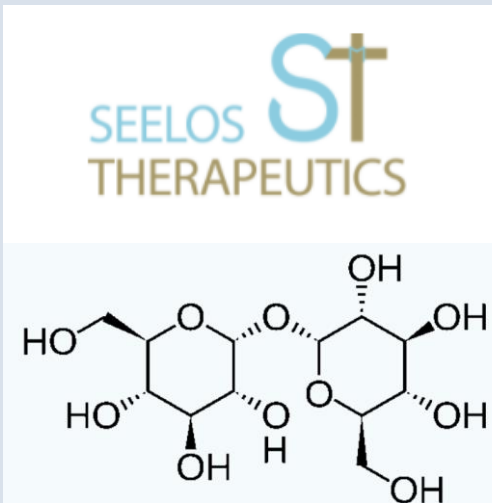
Travel around the
US meeting
Platform Trial Site
Investigators



Guest speakers
from partner
foundations

Trehalose/SLS-005
drug science and
mechanism of action
webinar with Seelos
Therapeutics

<https://bit.ly/3L4hrdB>



Special Topics:
Biomarkers, EAPs,
Biostats, Science
topics (epigenetics,
epidemiology)