

HEALEY ALS Platform Trial

Weekly Q&A – March 31, 2022



Healey Center

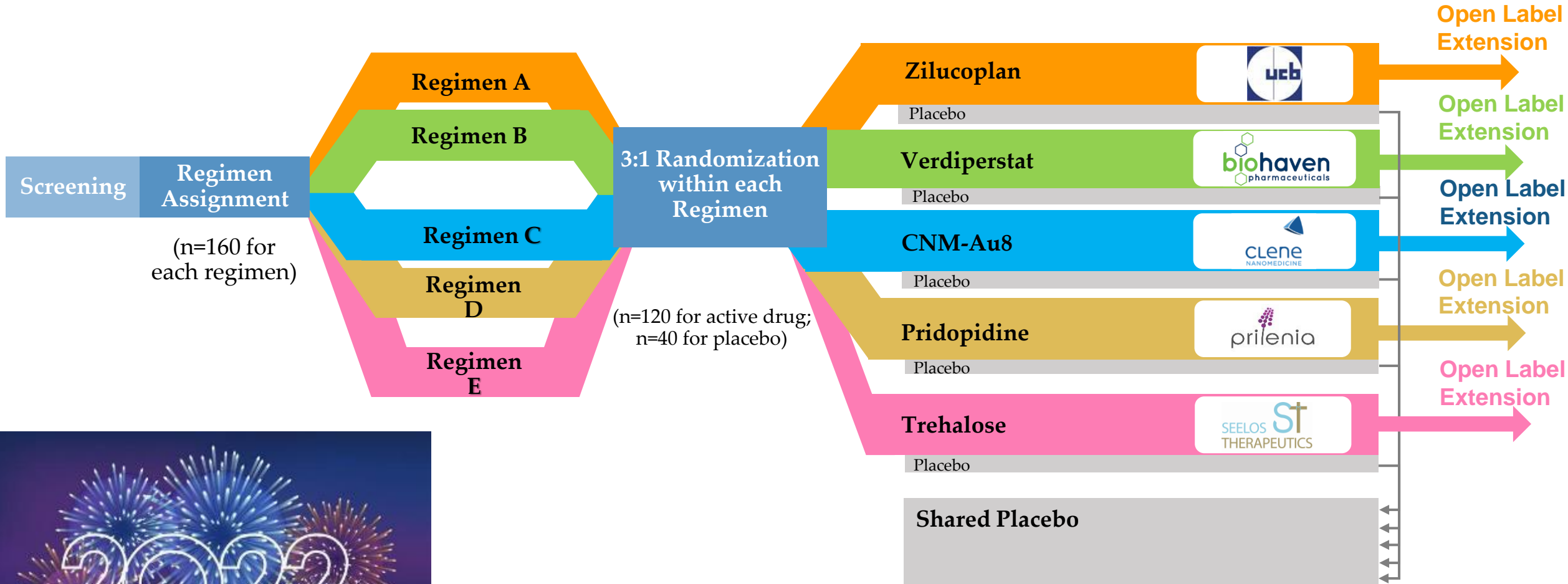
Sean M. Healey & AMG Center
for ALS at Mass General



The AMG Foundation

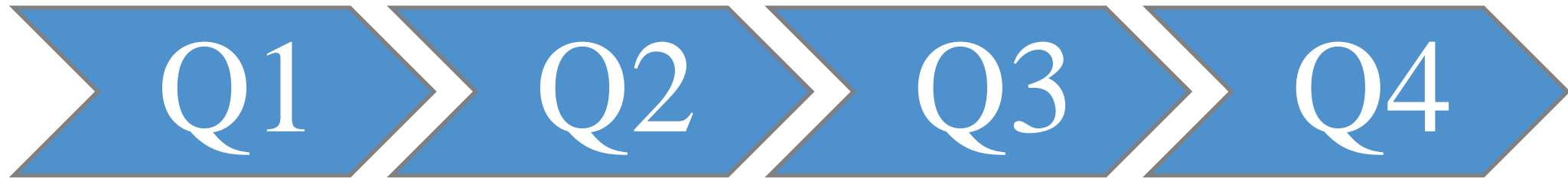


The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



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: May-Sept 2022

Top Line Results: July-Sept 2022

Study Reports: Oct-Dec 2022

Enrollment Updates (as of March 31, 2022)

- **21** individuals have signed informed consent
- **15** individuals have been randomized within Regimen E

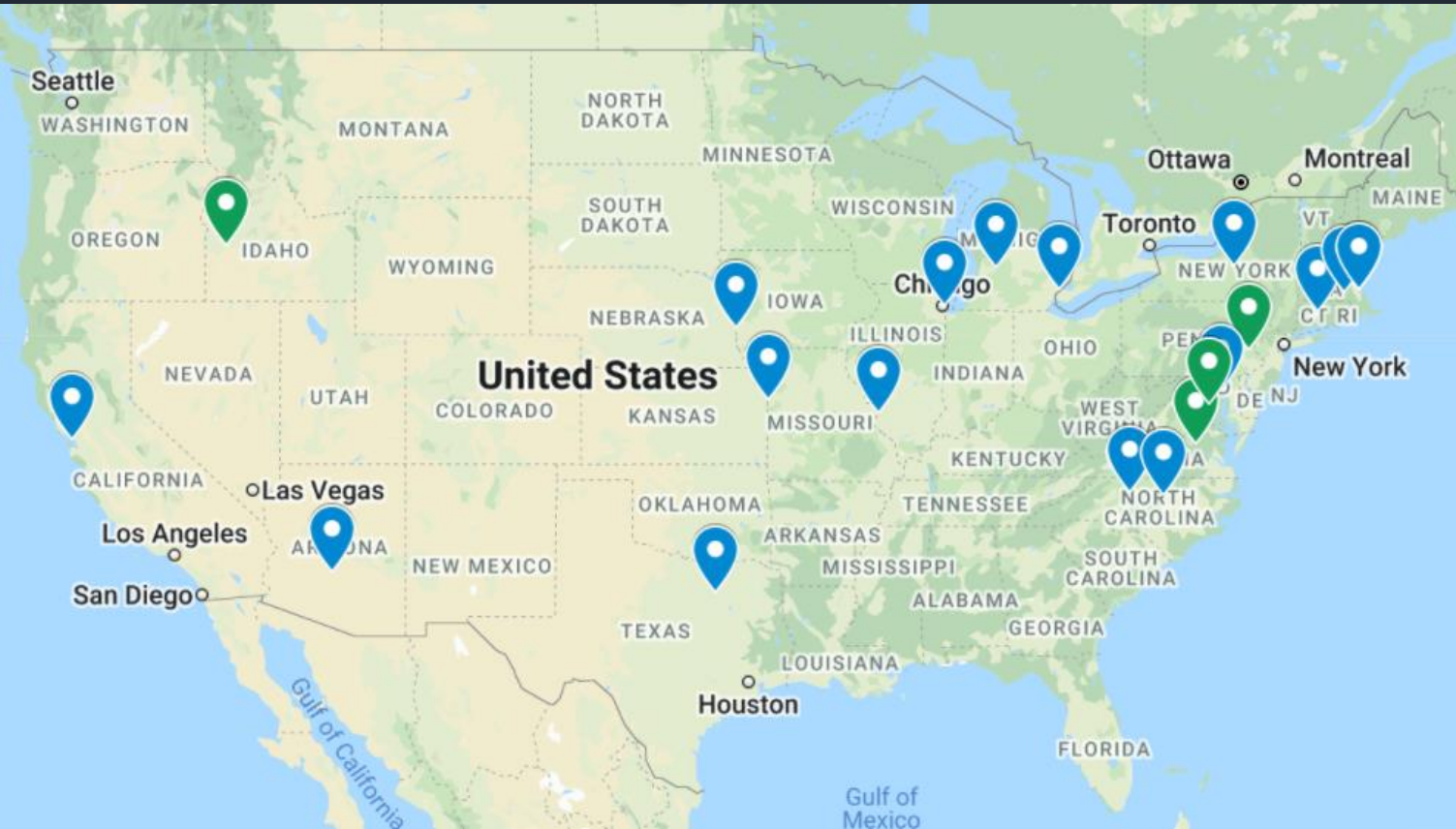
Thank You

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

21 Sites Currently Activated for Regimen E



(as of 3/31/22)

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

Regimen E Site Activation

Sites closest to activation:

- University of Colorado
- University of South Florida
- Providence Brain and Spine
- Ohio State University
- Essentia Health
- University of Cincinnati
- Loma Linda University
- Swedish Medical Center

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>

New & Updated Webpages on Expanded Access

Expanding Access to Investigational Products

HEALEY ALS Platform Trial Multicenter Companion Expanded Access

CNM-Au8, by Clene Nanomedicine



Pridopidine, by Prilenia Therapeutics: Multiple Locations



Verdiperstat, by Biohaven: Multiple Locations



Expanded Access Information for
Patients

Frequently asked questions and helpful online
resources for people interested in participating in an
EAP.

[Learn about EAPs >](#)



<https://bit.ly/3qK2INq>

New & Updated Webpages on Expanded Access

Common Questions about Expanded Access Protocols

What is Expanded Access?



How is Expanded Access different from clinical trials?



How do I learn more about participating in an Expanded Access Protocol?



Why would I participate in an Expanded Access Protocol?



How are Expanded Access Protocols funded?



- [Watch an educational webinar about EAPs](#) hosted by the Northeast ALS Consortium (NEALS)
- [Visit the NEALS website](#)
- [Visit the US FDA website](#)
- Read the scientific journal article [Accessing Investigational Products Outside of a Trial: Considerations for Neuromuscular Providers](#)



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

Patient Navigation

Central resource for people living with ALS



Catherine Small

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Weekly webinar
registration:



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

ALS Link sign-up:



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



Allison Bulat

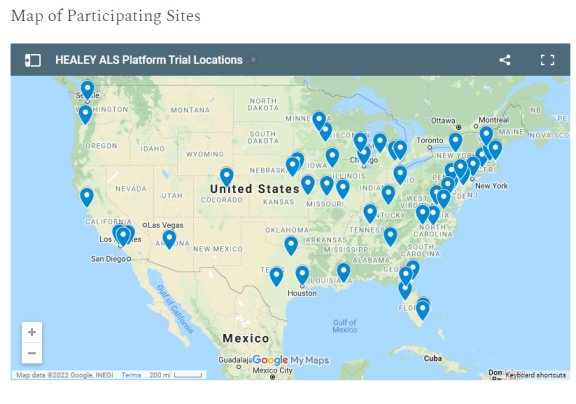
Upcoming Guest Speakers:

April 14th- Kuldip Dave, PhD (VP of Research, The ALS Association)

May 5th- Peter Foss (President, ALS Finding a Cure)

Send us webinar questions and ideas!

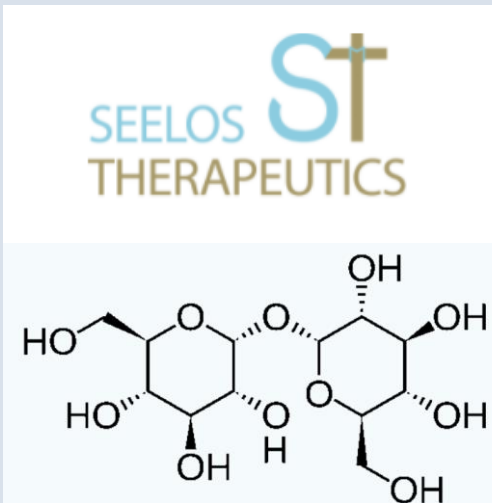
Travel around the
US meeting
Platform Trial Site
Investigators



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)