HEALEY ALS Platform Trial

Weekly Q&A - May 26, 2022









Berry Consultants









Healey Center

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































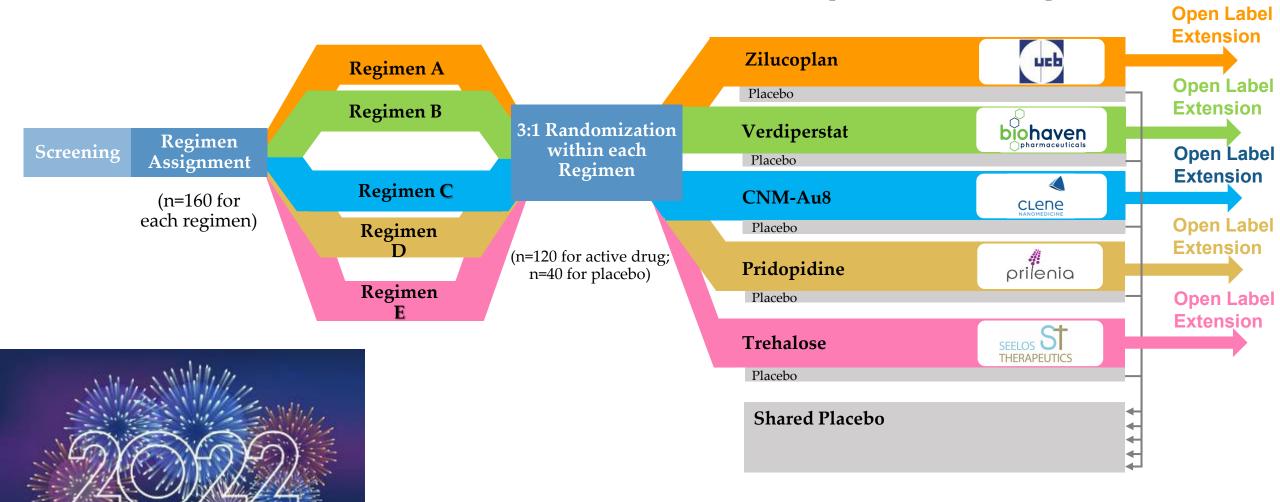








The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



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 Database Lock: July-Oct 2022

Top Line Results: Aug-Nov 2022

Study Reports: Nov-Dec 2022

Enrollment Updates (as of May 26, 2022)

• 64 individuals have signed informed consent

42 individuals have been randomized within Regimen E

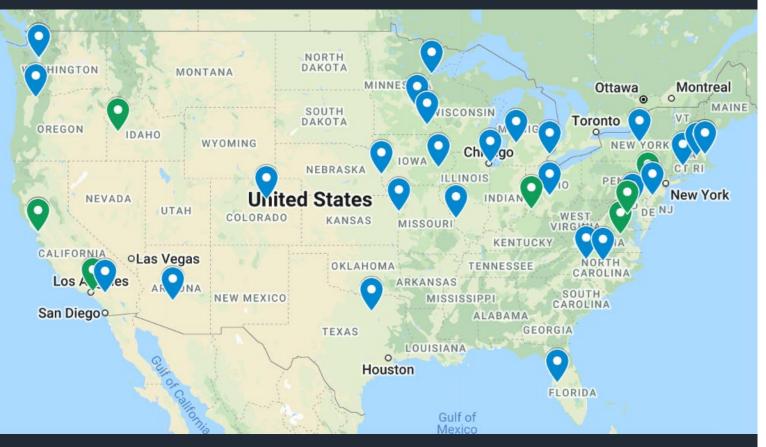


This breakthrough trial would not be possible without your participation

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

36 Sites Currently Activated for Regimen E



(as of 5/26/22)

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

- Mass General Hospital
- ☑ University of Kansas
- University of Maryland
- California Pacific Medical Center
- Morthwestern 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
- **B**eth 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
- University of Cincinnati
- Thomas Jefferson University

UC San Francisco

Mayo Rochester

University of Washington

Regimen E Site Activation

Sites closest to activation:

➤ University of Pittsburgh

➤ Indiana University

➤ Augusta University

➤ Penn State Hershey

➤ Vanderbilt 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
Lehigh Valley Health Network	PA	Recruiting	Andrew Orzel (610-402-9543)
Penn State Hershey	PA	Active, Not recruiting	<u>Heidi Runk</u>



 $\underline{https://bit.ly/3g2NZr5}$

Patient Navigation Central resource for people living with ALS



Catherine Small



Allison Bulat

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Weekly webinar registration:



https://bit.ly/3r6Nd2L

ALS Link sign-up:



https://bit.ly/3o2Ds3m

Upcoming Guest Speakers:

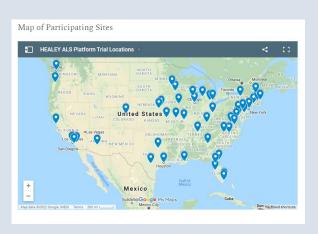
June 2nd- Daniel Hevert (Lead Coordinator, Mass General Hospital)

June 9th- Honora Dalamagas and Natalie Cartwright (Hospital for Special Care)

June 16th- Bruce Rosenblum (EAP Patient Advisory Committee Member)

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)