The goal of the HEALEY ALS Platform Trial is to screen drugs rapidly and efficiently, get solid answers, and determine next steps.
Informational Webinars about Regimen E

Trehalose/SLS-005 Drug Science and Mechanism of Action Q&A Webinar
Hosted by Seelos Therapeutics on 10 March 2022

Recording Available!
Recording available under “science and mechanism of action series”

The ALS Association/Northeast ALS Consortium Educational Webinar
Update on Healey ALS Platform Trial Regimen E: Trehalose for ALS

Recording Available!
https://bit.ly/3X2u004
Recording available under “educational webinars” on neals.org
57 Sites Currently Activated for Regimen E

(as of 11/17/22)

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
University of Cincinnati
Thomas Jefferson University
UC San Francisco
Mayo Rochester
University of Washington
Vanderbilt University

UPMC
Indiana University
Augusta University
University of Utah
Holy Cross Hospital
Penn State Hershey
University of CA, Irvine
Cedars Sinai Medical Center
University of Pennsylvania
Nova Southeastern University
Johns Hopkins University
Columbia University
Stony Brook University
Kaiser, Los Angeles
Cleveland Clinic
Medical College of Wisconsin
University of Michigan
Las Vegas Clinic
George Washington University
Mayo Clinic Florida

https://bit.ly/3g2NZr5
Enrollment Updates (as of Nov 17, 2022)

• 164 individuals have signed informed consent

• 124 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.
Requirements for All EAPs

➢ Patient has a **serious** or **immediately life-threatening** disease or condition; no comparable or satisfactory alternative therapy

➢ Potential benefit justifies potential risk; potential risk not unreasonable in context of disease or condition

➢ Providing drug will **not interfere with clinical trials** that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use

Only 10% of people living with ALS participate in clinical trials

https://www.fda.gov/news-events/public-health-focus/expanded-access
ACT for ALS

Signed into law on Dec 23, 2021

Section 2: Grants for Research on Therapies for ALS via Intermediate-Size EAPs
Community Support to EAPs

EAP COMPANION PROGRAM SUPPORTERS

- Healey Center forALS
- Clearing Corporation Charitable Foundation
- Eddie and Jo Allison Smith Family Foundation
- Richard Stravitz Foundation
- I AM ALS
- Biohaven
- Clene
- Prilenia
- Elliott & Frantz, Inc.

Community Fundraisers
- Tackle ALS – Team Change ALS
- Ellen Corinidia’s Fundraiser
- 2019 Olson Cornhole Tournament
- 2019 Worthington Fore ALS
- 2020 and 2021 Fishing for ALS Warriors
- 2020 and 2021 sALSa For a Cure Pick Your Own Path Walk

- 2020 and 2021 Lori’s Shoes “Hope Is In the Bag”
- 2021 and 2022 MLB Lou Gehrig Day 4-ALS
- 2021 Russ Pallesen Fundraiser for EAP
- 2021 Voices for ALS Golf Tournament
- 2021 The Martha Olson-Fernandez Foundation Golf Tournament
- 2021 Gwendolyn Strong Walk

- TechVs ALS

- Big thanks to countless individual contributors
EAP Companion To HEALEY ALS PLATFORM TRIAL

2021- present
- 3 study drugs (Regimens B, C, D)
- 10 sites
- 85 participants
- Funded by philanthropy (study drug donated by manufacturers)
- Safety and clinical data; biomarker data
Intermediate Size EAP - Trehalose

- In start up now
- Expected to enroll first participants in Q1 2023
- 25 sites
- 70 participants
- NIH-funded (PIs: Babu, Berry, Paganoni)
- Happening in parallel to Regimen E of the HEALEY ALS Platform Trial

Outcomes:
- Long-term safety in a broad population
- Biological impact (as measured by neurofilament light levels)
- Clinical efficacy signal (as compared to natural history cohorts)
Patient Navigation
Central resource for people living with ALS

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 Webinars:
November 24th- Webinar canceled for holiday, Happy Thanksgiving!
December 1st- Catherine Douthwright, PhD, CCRP (University of Massachusetts)