

HEALEY ALS Platform Trial

Weekly Q&A – Nov 17, 2022



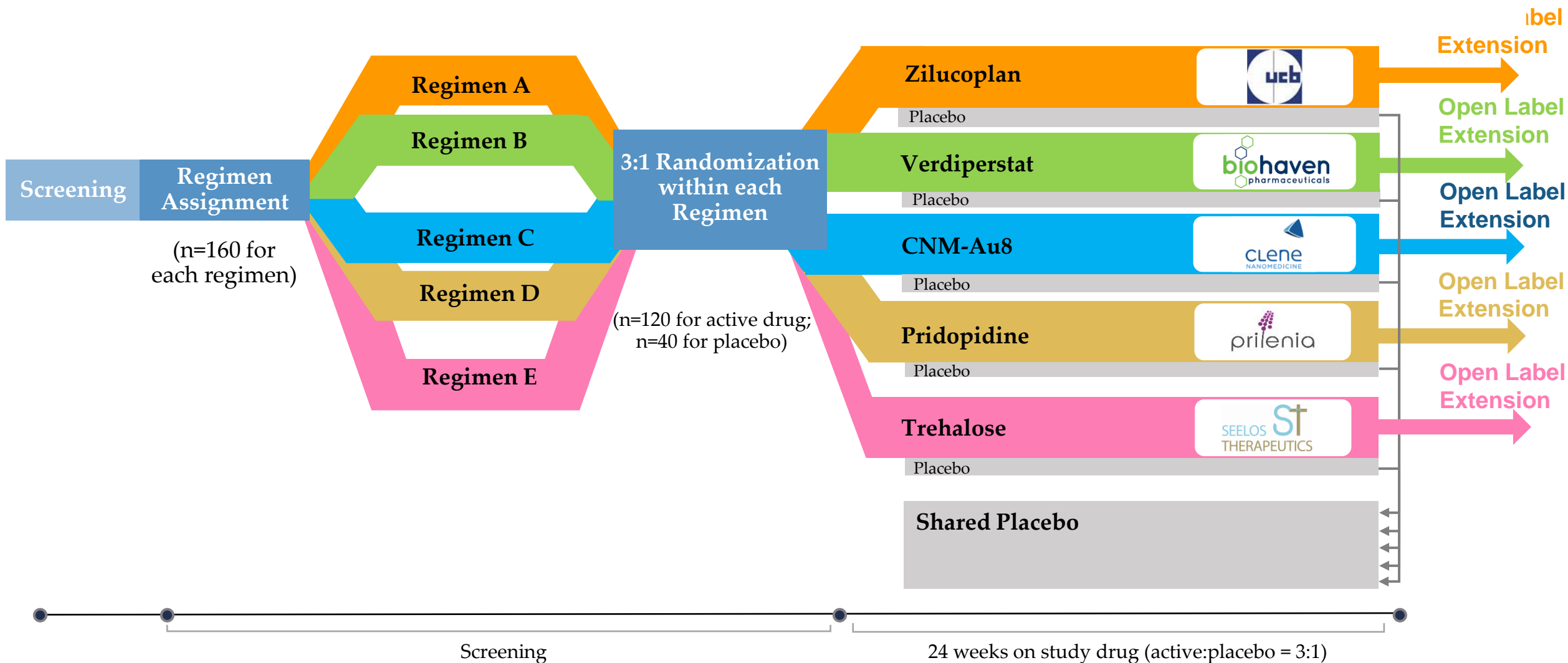
Healey Center

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



The AMG Foundation

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!



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

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

UPDATE ON REGIMEN E

TREHALOSE FOR ALS



Merit Cudkowicz, MD, MSc

SEAN M. HEALEY & AMG
CENTER FOR ALS AT MGH



Shafeeq Ladha, MD

BARROW NEUROLOGICAL
INSTITUTE

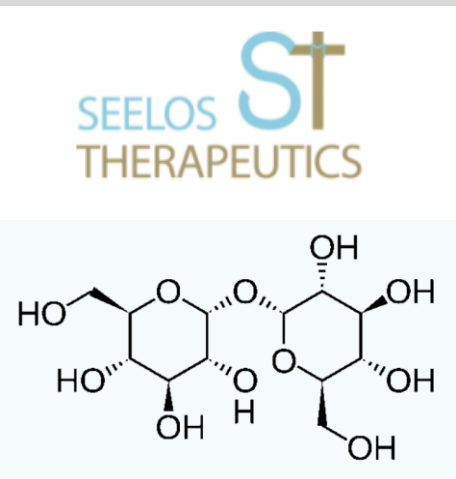
TUESDAY | 4 OCTOBER
3 PM EDT

Recording Available!

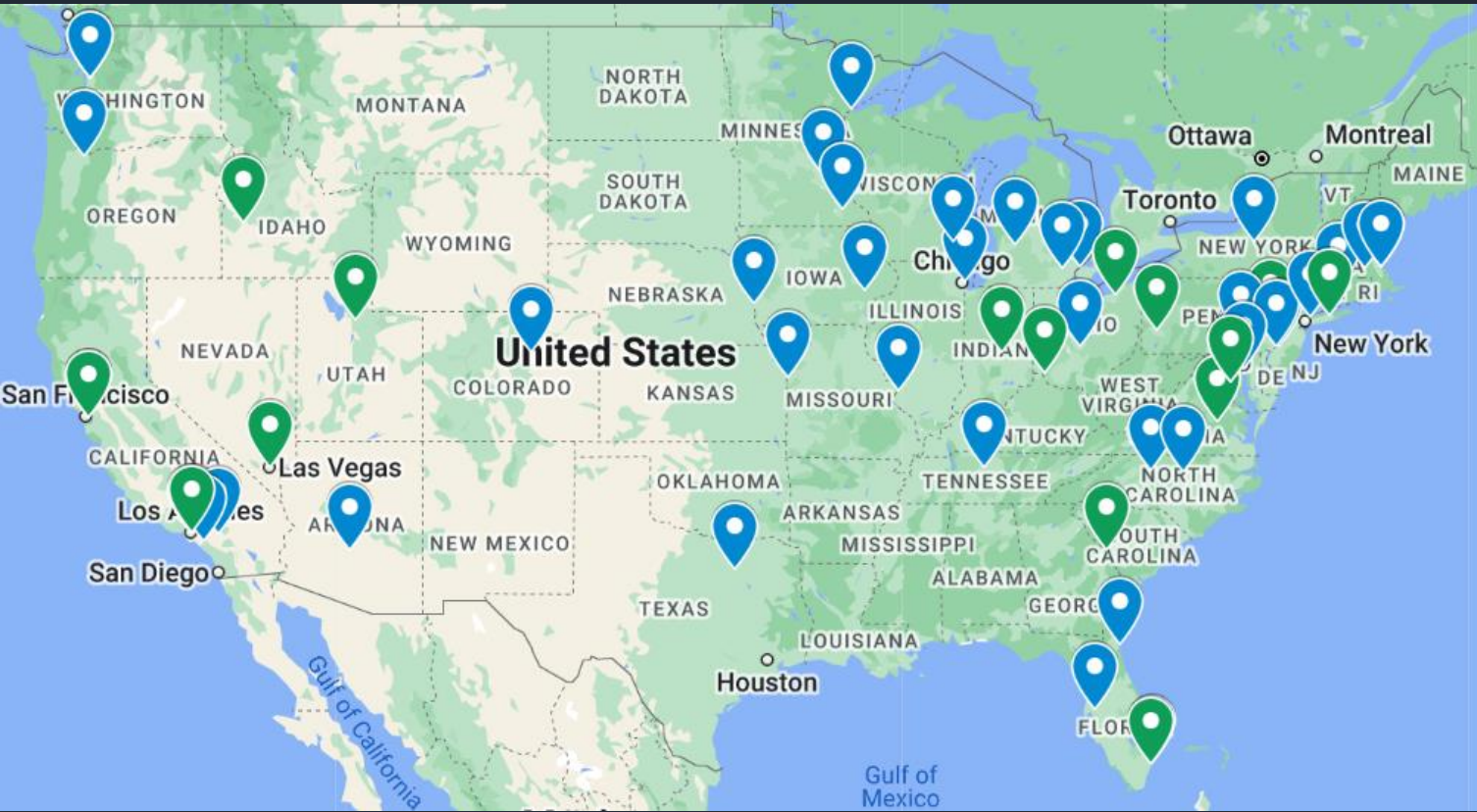


<https://bit.ly/3X2u004>

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 Alphonus 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

Site Map & Contacts:



<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

Expanded Access

FDA Definition:

- Sometimes called “compassionate use”
- Potential pathway for a patient with an immediately life-threatening or serious disease or condition
- Allows access to an investigational medical product outside of clinical trials
- Used when no comparable or satisfactory alternative therapy available

<https://www.fda.gov/news-events/public-health-focus/expanded-access>

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



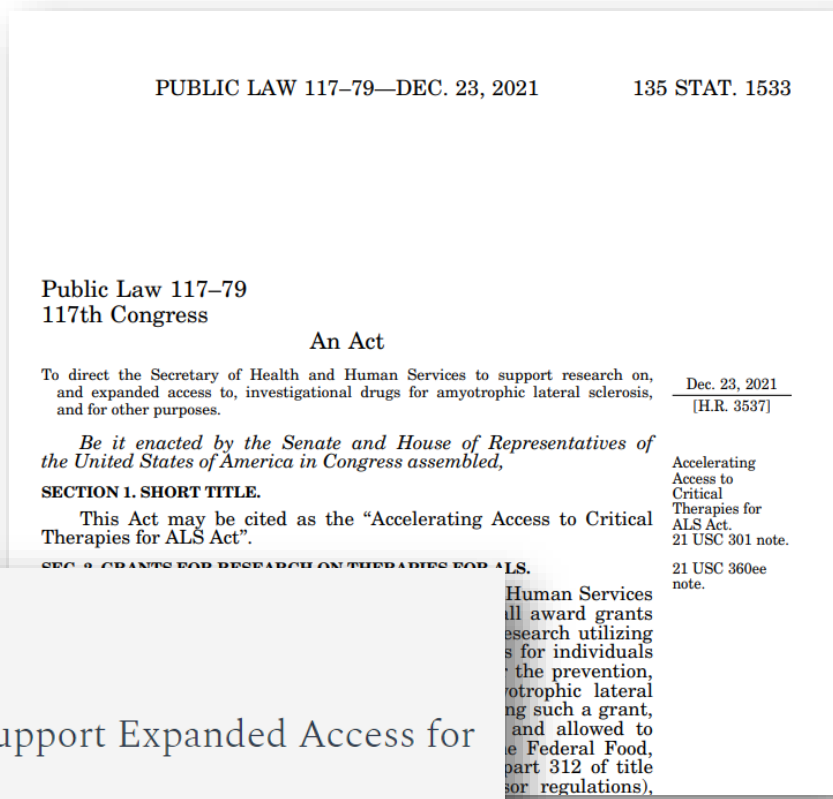
Requirements for All EAPs

- 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

ACT for ALS

Signed into law on Dec 23, 2021

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



ie - Neurology - ALS - News

NEWS · SEP | 30 | 2022

Sean M. Healey & AMG Center for ALS awarded NINDS UO1 Grant to support Expanded Access for Trehalose (SLS-005)

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The Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital has been awarded a grant from the National Institute of Neurological Disorders and Stroke (NINDS) to conduct an intermediate size Expanded Access Protocol (EAP) in Amyotrophic Lateral Sclerosis (ALS). The grant is supported by the ACT for ALS (Accelerating Access to Critical Therapies for ALS Act). This EAP will

Type

News

Centers and Depar

Community Support to EAPs

EAP COMPANION PROGRAM SUPPORTERS

- Healey Center for ALS
- 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 Corindia’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**



Healey Center

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

Never Surrender Inc.
Funding the Fight Against ALS



TECHVsALS

Benefiting the Sean M. Healey and AMG Center for ALS



TECH Vs ALS

Benefiting the Susan M. Shelby and AFQ Center for ALS

Link to Full Video:



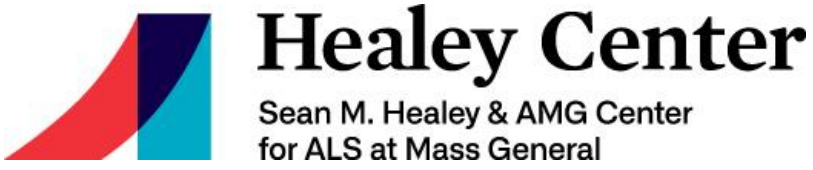
<https://bit.ly/3An9eye>

<https://youtu.be/A2tghxM2GyU?t=171>

EAP Companion To HEALEY ALS PLATFORM TRIAL

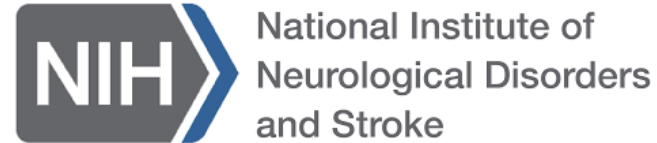
2021- present

- 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



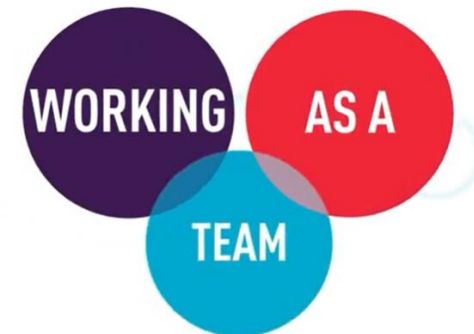
View Press Release:



<https://bit.ly/3S0a9eb>

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



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 Webinars:

November 24th- Webinar canceled for holiday, Happy Thanksgiving!

December 1st- Catherine Douthwright, PhD, CCRP (University of Massachusetts)



Allison Bulat