

HEALEY ALS Platform Trial

Weekly Q&A – Nov 10, 2022



Healey Center

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



The AMG Foundation

SEAN M. HEALEY & AMG CENTER FOR ALS

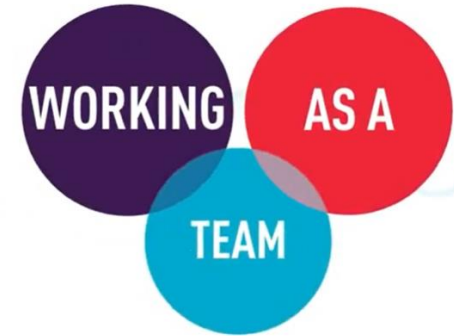
HEALEY ALS Platform Trial Patient Information

An adaptive platform trial designed to accelerate the development of effective and breakthrough treatments for people in ALS.

Participating sites >

Trial news & webinars >

Treatments being tested >



Video Link:



<https://bit.ly/3N9eTx6>

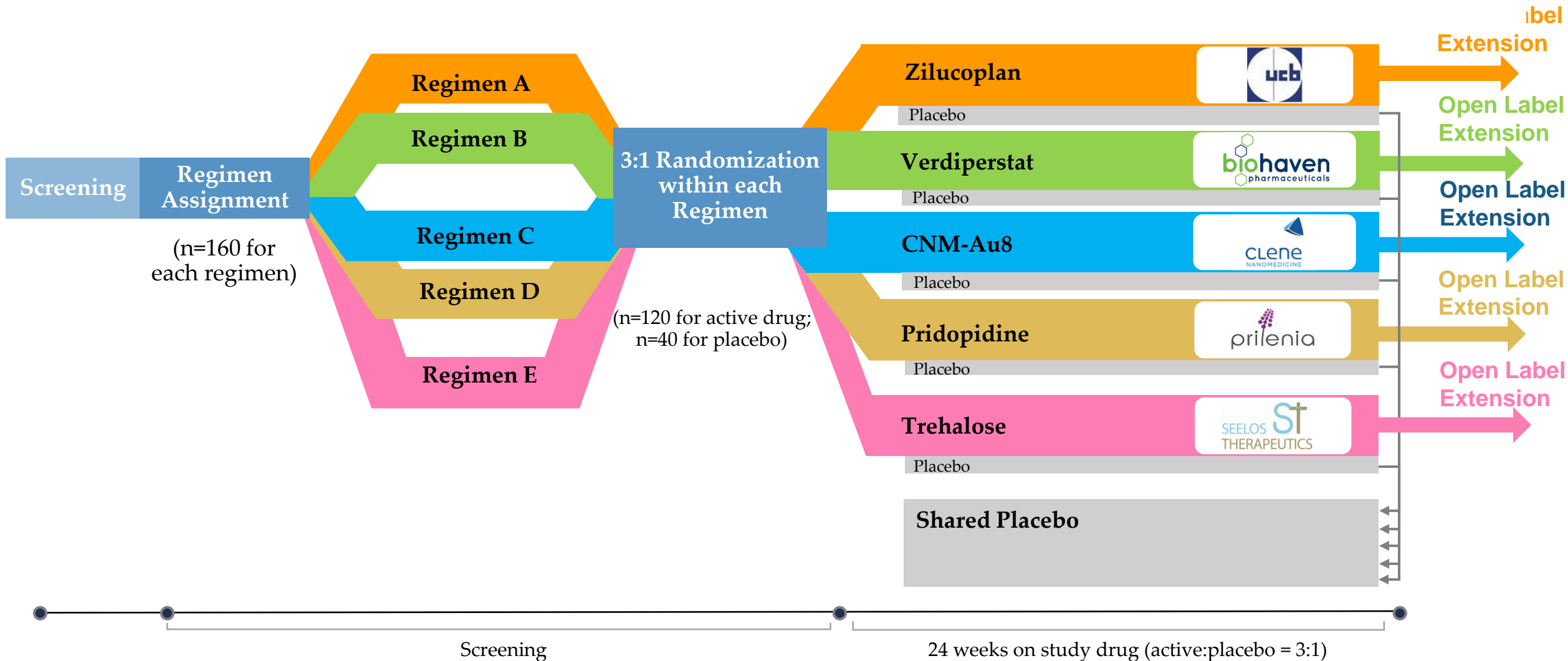
**'For Patients' page
on MGH website:
<https://bit.ly/3UFzgV7>**

The trial operates
across an expanding network of



coordinated sites nationwide

The goal of the HEALEY ALS Platform Trial is to screen drugs rapidly and efficiently, get solid answers, and determine next steps



Recap of Sept. and Oct. Research News

– **September 29, 2022: FDA Approval of AMX0035**

- <https://www.fda.gov/news-events/press-announcements/fda-approves-new-treatment-option-patients-als>

– **September 29, 2022: Verdiperstat/Regimen B Topline Results Released**

- <https://www.massgeneral.org/news/press-release/results-from-healy-als-platform-trial-with-verdiperstat>

– **September 30, 2022: Grant Funding for Trehalose EAP (Regimen E)**

- <https://www.massgeneral.org/neurology/als/news/healeyamg-awarded-ninds-u01-grant>

– **October 3, 2022: CNM-Au8/Regimen C Topline Results Released**

- <https://www.massgeneral.org/news/press-release/healey-amg-center-announces-top-line-results-in-als-platform-trial-with-cmn-au-eight>

HEALEY ALS Platform Trial

Initial Regimen Updates – trial gave clear answers and direction, efficient execution (time, resources, less placebo)

- February 2022: **Regimen A** stopped early for futility
- September 2022: **Regimen B** top line results announced
 - * Did not meet the prespecified primary endpoint and there were no statistically significant benefits on key secondary measures; full study results, including data on biomarkers and exploratory measures, are expected in the coming months
- October 2022: **Regimen C** top line results announced
 - * While the primary endpoint was not met, a secondary endpoint analysis of survival demonstrated a significant reduction in risk of death or permanently assisted ventilation when adjusting for baseline risk imbalances in the CNM-Au8 regimen for the 30 mg dose; full study results, including data on biomarkers and exploratory measures, are expected in the coming months

Weekly Recordings Available on Website

Webinar Recordings

Science & Mechanism of Action Series +

Weekly & Monthly Updates -

October 6, 2022: Weekly Q&A featuring discussion of **Regimen C/CNM-Au8 results**

Merit Cudkowicz, MD, MSc and Sabrina Paganoni, MD, PhD presented this week's updates on the HEALEY ALS Platform Trial and answered questions from the audience. We were joined by the co-lead investigators for Regimen C and representatives from Clene Nanomedicine, Inc. to discuss the trial results for CNM-Au8.

[Watch recording.](#)

September 29, 2022: Weekly Q&A featuring discussion of **Regimen B/Verdiperstat results**

Merit Cudkowicz, MD, MSc and Sabrina Paganoni, MD, PhD presented this week's updates on the HEALEY ALS Platform Trial and answered questions from the audience. We were joined by the co-lead investigators for Regimen B and representatives from Biohaven Pharmaceuticals to discuss the trial results for Verdiperstat.

[Watch recording.](#)

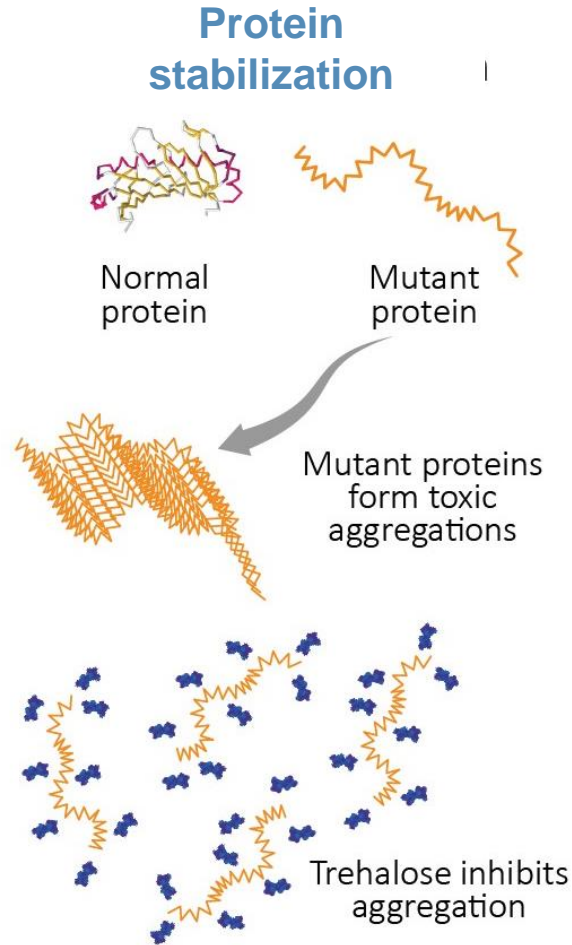
Visit the Website:



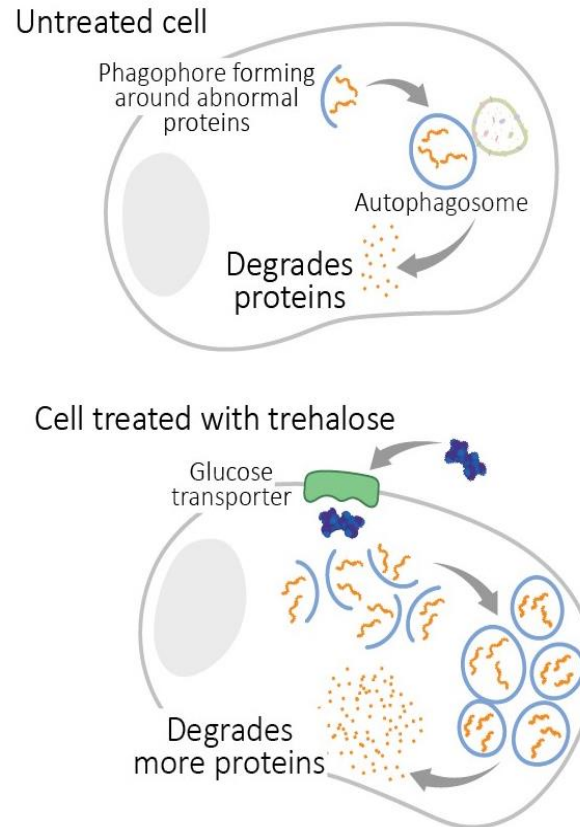
<https://bit.ly/3g4kzfv>

Regimen E - Trehalose

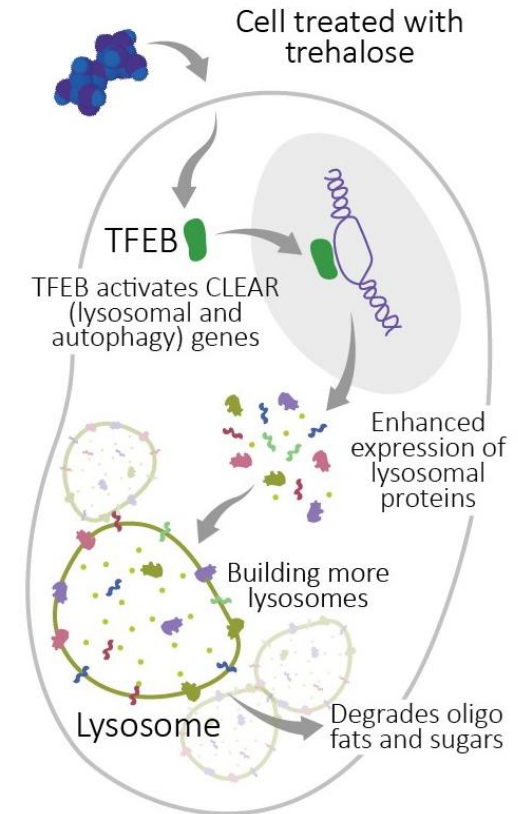
MECHANISM OF ACTION



Immediate enhancement of autophagy



Prolonged enhancement of lysosomal pathways thus autophagy



1. Mayer AL, Higgins CB, Heitmeier MR, et al. SLC2A8 (GLUT8) is a mammalian trehalose transporter required for trehalose-induced autophagy. *Scientific Reports*. 2016;6(38586).

Available at: <https://www.nature.com/articles/srep38586#f1> Accessed February 2019.

2. Uchida K, Unuma K, Funakoshi T, et al. Activation of Master Autophagy Regulator TFEB During Systemic LPS Administration in the Cornea. *Journal Toxicol Pathol*. 2014;27(2):153-8.

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

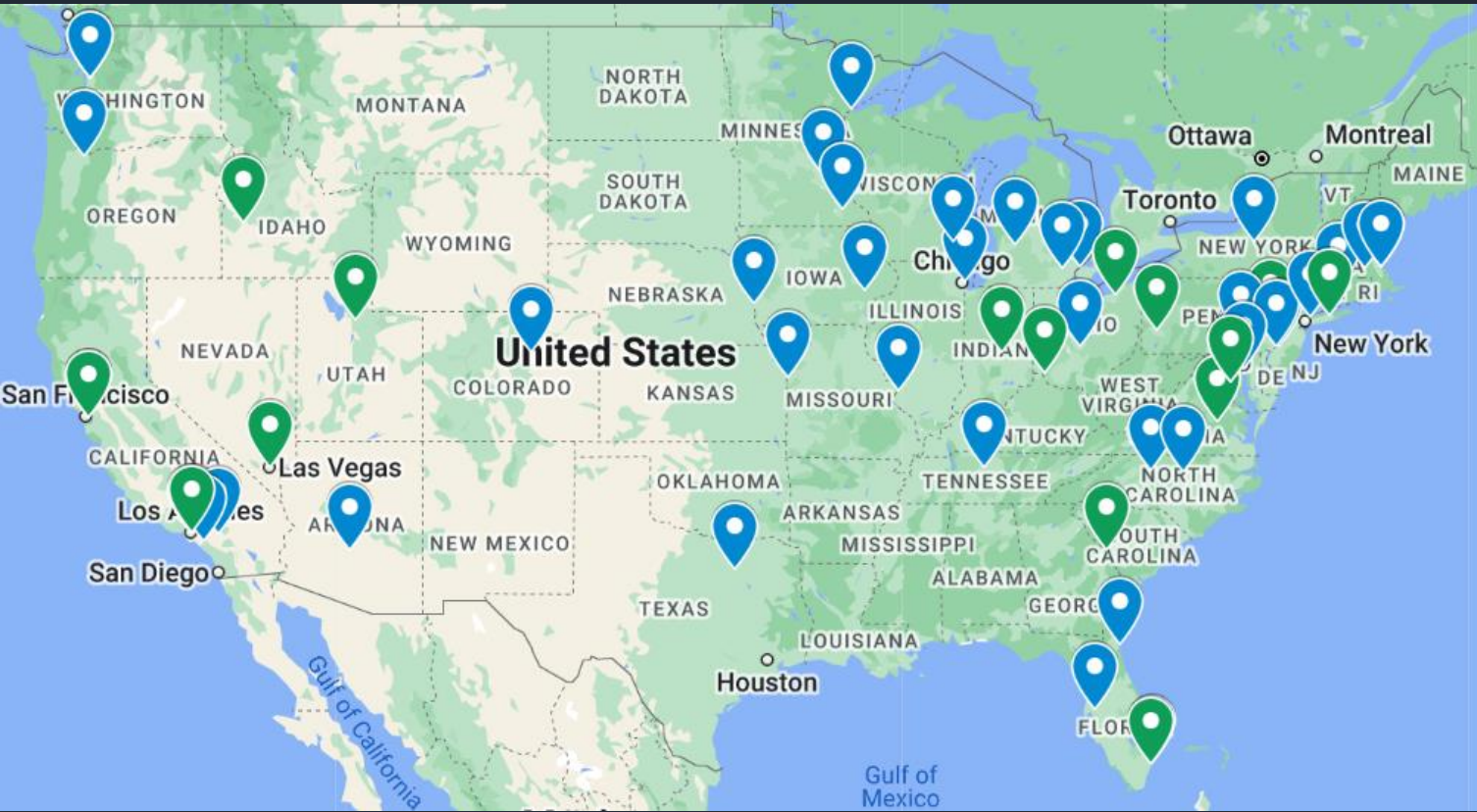
Recording Available!



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

Recording available under “educational webinars” on neals.org

57 Sites Currently Activated for Regimen E



(as of 11/10/22)

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 10, 2022)

- **164** individuals have signed informed consent
- **121** 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

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 Cancellation:

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



Allison Bulat