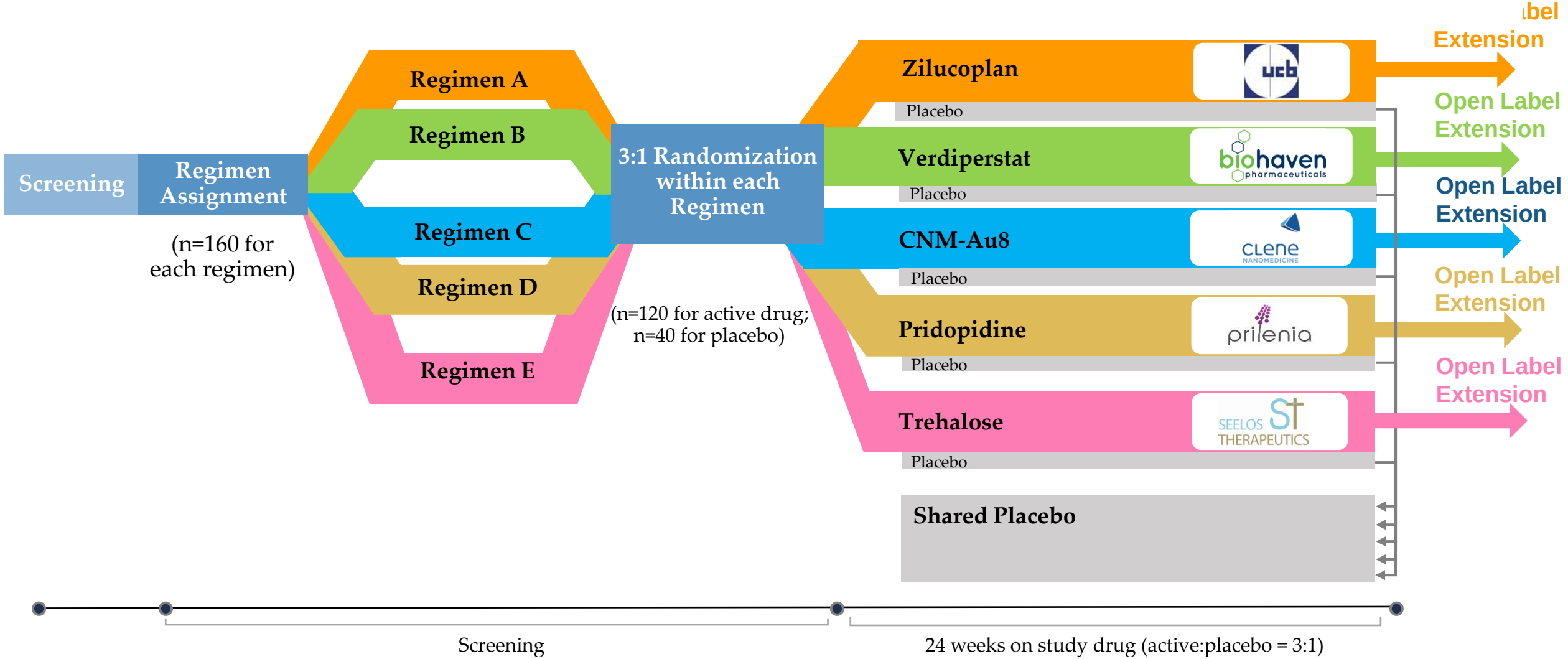
HEALEY ALS Platform Trial

Weekly Q&A – Dec 15, 2022

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

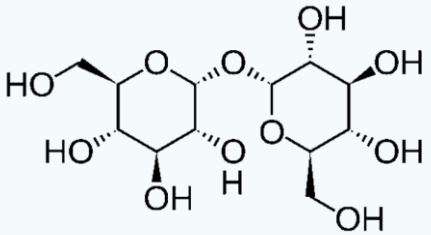


Informational Webinars about Regimen E

Trehalose/SLS-005 Drug Science and Mechanism of Action Q&A Webinar

Hosted by Seelos Therapeutics on 10 March 2022

SEELOS THERAPEUTICS



Recording Available!

https://bit.ly/3L4hrdB

Recording available under "science and mechanism of action series"

The ALS Association/Northeast ALS Consortium Educational Webinar

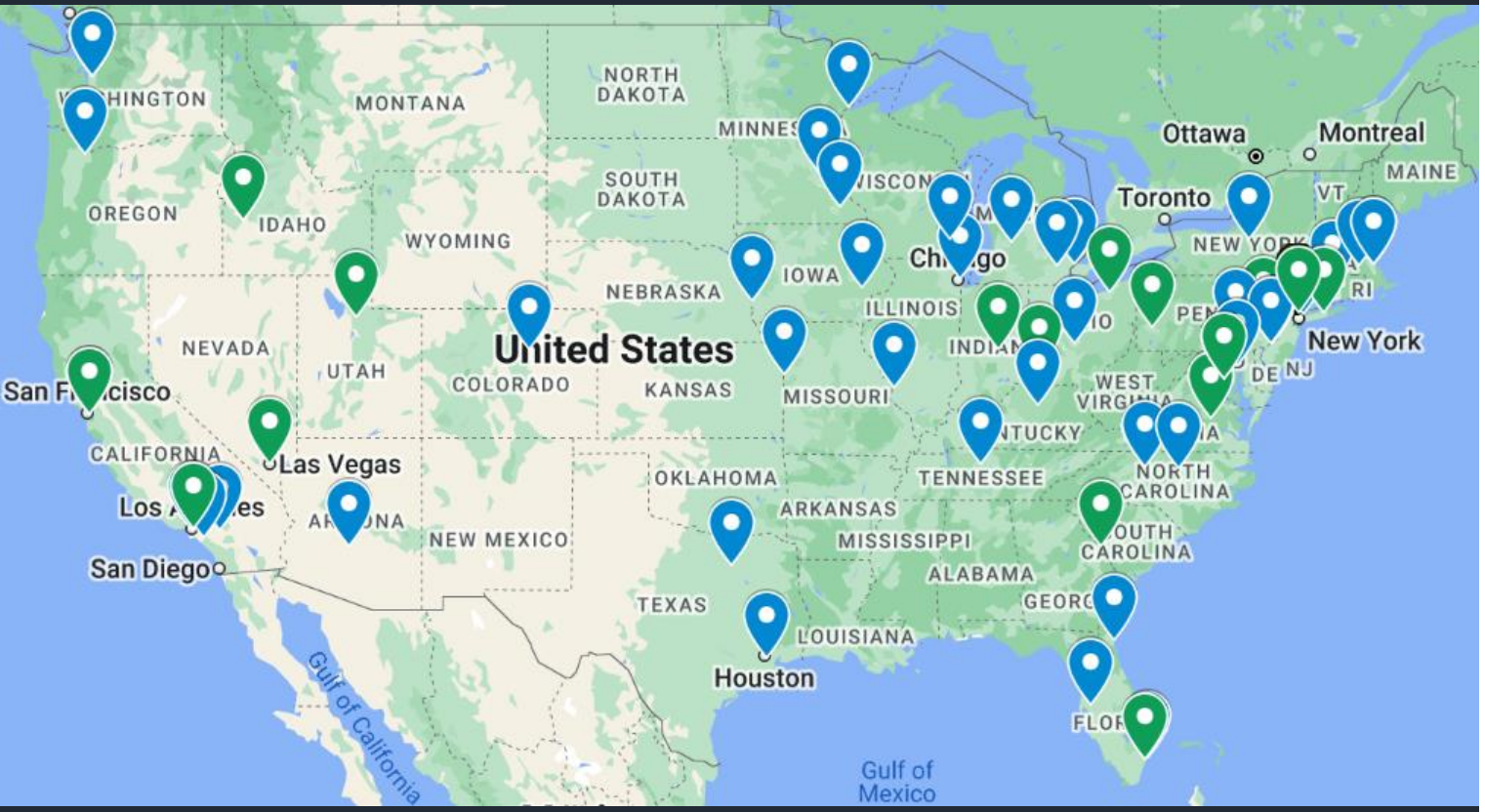
Update on Healey ALS Platform Trial Regimen E: Trehalose for ALS

Recording Available!

https://bit.ly/3X2u004

Recording available under "educational webinars" on neals.org

60 Sites Currently Activated for Regimen E



(as of 12/15/22)

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
- University of Kentucky
- Houston Methodist
- Hackensack University

Site Map & Contacts:

https://bit.ly/3g2NZr5

Enrollment Updates (as of Dec 15, 2022)

- **185 individuals have signed informed consent**

- **132 individuals have been randomized within Regimen E**

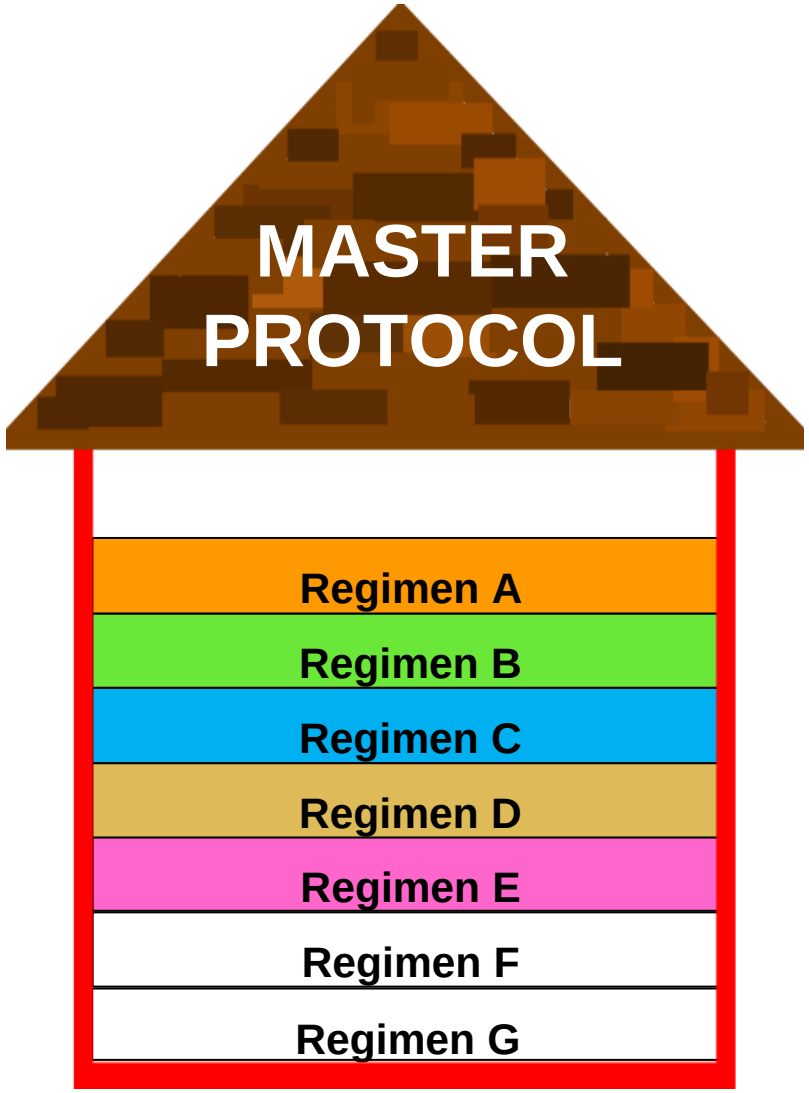
Thank You

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


2023


MASTER PROTOCOL
Regimen A
Regimen B
Regimen C
Regimen D
Regimen E
Regimen F
Regimen G

Regimen F

PRESS RELEASE · 5 MINUTE READ · JUL | 12 | 2022

Investigational drug ABBV-CLS-7262 initiates design phase for entry into the HEALEY ALS Platform Trial

ABBV-CLS-7262 targets eIF2B, a key regulator of the integrated stress response (ISR). In neurons exposed to cellular stressors, inhibition of the ISR by ABBV-CLS-7262 restores protein synthesis and dissolves pre-formed TDP-43 containing stress granules. This effect of ABBV-CLS-7262 is of clinical interest because TDP-43 containing stress granules are thought to lead to TDP-43 inclusions, a hallmark of ALS pathology.

Calico

Regimen G

PRESS RELEASE · DEC | 5 | 2022

Healey & AMG Center for ALS announces new drug regimen for testing DNL343 in HEALEY ALS Platform Trial

- **DNL343 is being developed by Denali Therapeutics.**
- It targets eIF2B, a key regulator of the integrated stress response, to restore protein synthesis and dissolve pre-formed TDP-43 containing stress granules which are thought to lead to TDP-43 inclusions, a hallmark of ALS pathology.

> *"By adding one more drug to the platform, **we continue to push research forward** in hopes of soon finding many more effective treatments for ALS."*
>
> Merit Cudkowicz, MD, MSc
>
> Director, Sean M. Healey & AMG Center for ALS, Massachusetts General Hospital

https://bit.ly/3uwypuU

Patient Navigation
Central resource for people living with ALS

Catherine Small

Allison Bulat

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:

December 22nd- Canceled for holidays

December 29th- Canceled for holidays

See you in the New Year!

New Expanded Access Video

Sean M. Healey & AMG Center for ALS Expanded Access Program, with TechVsALS

Watch Video:

https://bit.ly/3HCwGw3

- **Direct YouTube Link:** https://www.youtube.com/watch?v=25-EeCwpEnU
- **Mass General's EAP Webpage:** https://www.massgeneral.org/neurology/als/research/expanded-access