

HEALEY ALS Platform Trial

Weekly Q&A – Dec 8, 2022



Healey Center

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



The AMG Foundation



Guest Speaker

Mia Resendes, BS, MBA
Platform Trial Primary Coordinator
Massachusetts General Hospital, Boston MA



COORDINATING PLATFORM TRIAL

Mia Resendes

OUR COORDINATING TEAM



MIA RESENDES

Platform Trial



KATHERINE DARROW

Platform Trial



SUHINA PATEL

Platform Trial



VISHNI ARULANANDAM

Platform EAP

REGIMEN BREAKDOWN

Regimens A, B, C, D

- Visits every 4-8 weeks
- Visiting the Healey Center
- Independently dosing



Regimen E

- In-person once every week for 4-5 weeks
- Visiting the TCRC
- A nurse visiting for infusion





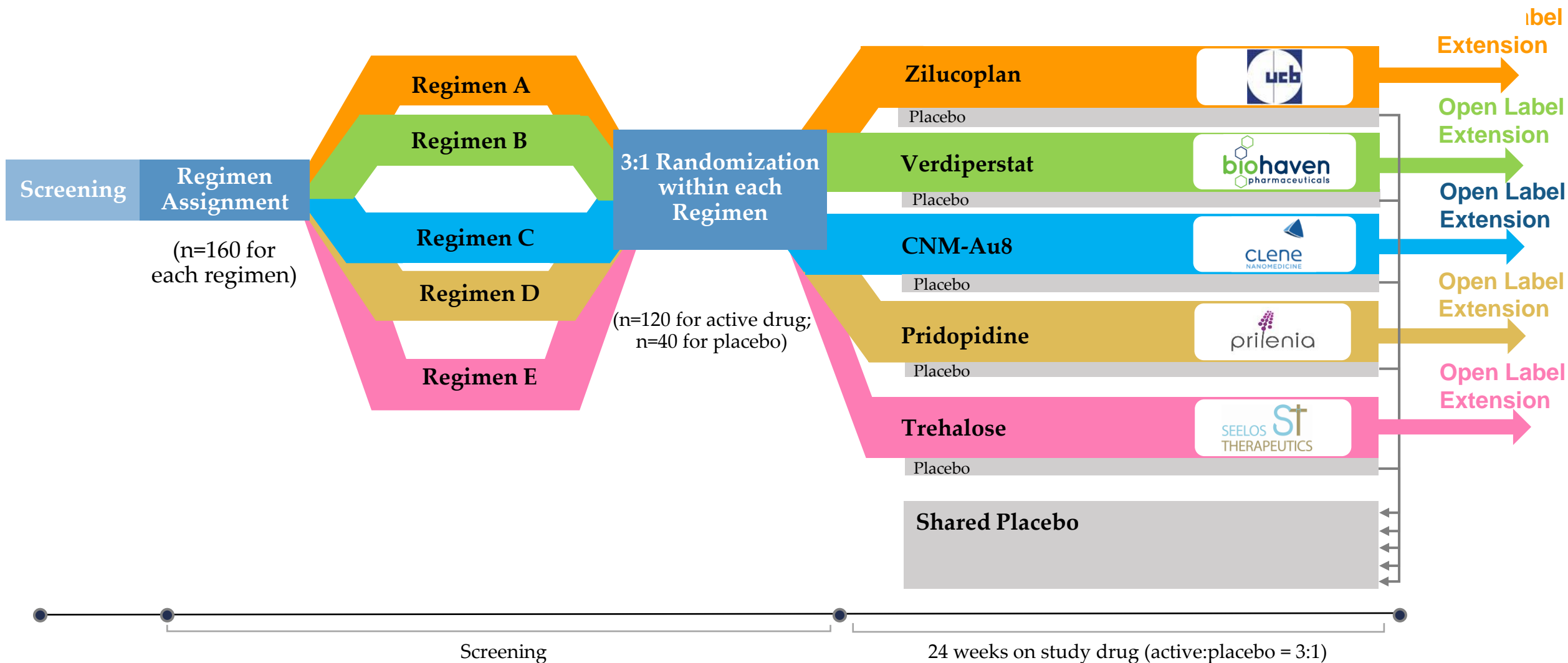
HOW IS RGE DIFFERENT FOR CRC'S?

- We see participants ALL the time!
- Working with the TCRC
- Home infusions & working with an outside organization
- Data, data, data

PROUD PARTNERS IN RESEARCH

- Regardless of the extra work, we love working with this regimen!
- Getting to know our participant on a deeper level
- Working towards new effective treatments for ALS

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!

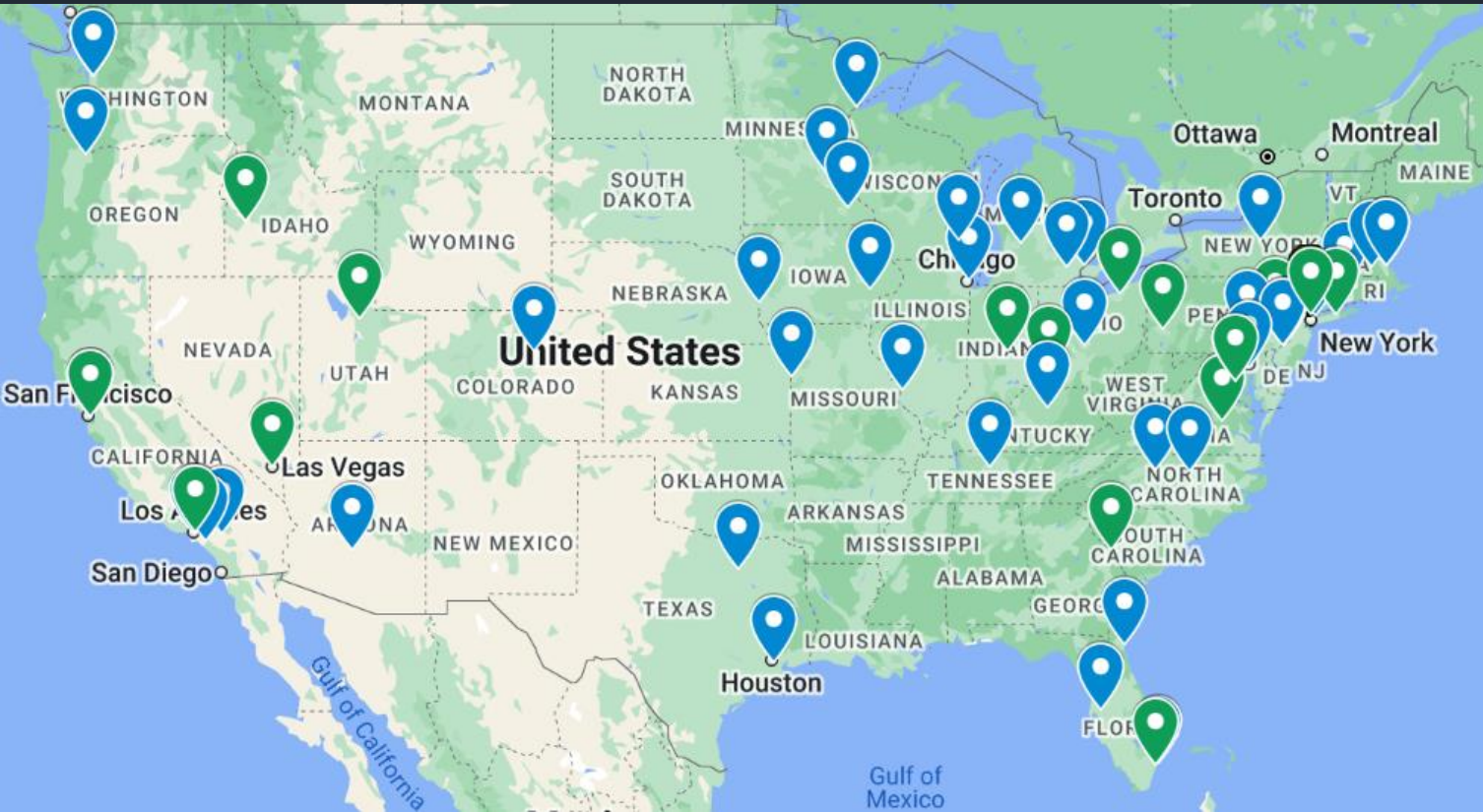


<https://bit.ly/3X2u004>

Recording available under “educational webinars” on neals.org



60 Sites Currently Activated for Regimen E



(as of 12/8/22)

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
- University of Kentucky
- Houston Methodist
- Hackensack University

Site Map & Contacts:



<https://bit.ly/3g2NZr5>

Enrollment Updates (as of Dec 8, 2022)

- **179** individuals have signed informed consent
- **129** 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 Webinars:

December 15th- No guest speaker

December 22nd- Canceled for holidays

December 29th- Canceled for holidays

See you in the New Year!



Allison Bulat

Recent News

PRESS RELEASE · DEC | 5 | 2022

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

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- **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>