HEALEY ALS Platform Trial

Weekly Q&A – Dec 8, 2022
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!
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

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

Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!

(as of 12/8/22)

Sites 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

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!
Recent News

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