Guest Speaker

Amanda Peltier, MD, MS
Platform Trial Site Investigator
Vanderbilt University Medical Center
Nashville, TN
ALS at Vanderbilt

• We have a multidisciplinary clinic monthly which currently serves ~125 patients from Tennessee, Kentucky and Alabama.

• We are supported by MDA and ALSA.

• Our clinic includes 4 neuromuscular specialists, 2 neuromuscular NP, pulmonology (1 MD, 1 NP), respiratory therapy, social work, palliative care, speech therapy, physical therapy, occupational therapy, assistive technology, nutrition

• We have 2 full time research coordinators.

• We have 10 patients who have been randomized in Healey
Thank You!!

• Your participation makes it possible for advances in ALS.
• We could not do it without you!
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

Screening

Regimen A
(n=160 for each regimen)

Regimen B

Regimen C

Regimen D

Regimen E

3:1 Randomization within each Regimen

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Trehalose
Placebo

Shared Placebo

Open Label Extension

(n=120 for active drug; n=40 for placebo)
Enrollment Updates (as of Oct 20, 2022)

- 153 individuals have signed informed consent
- 112 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.
51 Sites Currently Activated for Regimen E

(as of 10/20/22)

Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!
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 Cancelations:
November 3rd- Webinar canceled due to annual research conference
November 24th- Webinar canceled for holiday, Happy Thanksgiving!
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