HEALEY ALS Platform Trial

Weekly Q&A – Aug 11, 2022

Healey Center
Sean M. Healey & AMG Center for ALS at Mass General
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

- **Regimen A (n=160 for each regimen)**
  - Placebo
  - Verdiperstat
  - CNM-Au8

- **Regimen B (n=160 for each regimen)**
  - Placebo
  - Verdiperstat
  - Pridopidine

- **Regimen C (n=160 for each regimen)**
  - Placebo
  - Verdiperstat
  - Trehalose

- **Regimen D (n=120 for active drug; n=40 for placebo)**
  - Placebo
  - Verdiperstat
  - Zilucoplan

- **Regimen E (n=120 for active drug; n=40 for placebo)**
  - Placebo
  - Verdiperstat
  - Zilucoplan

- **Shared Placebo**
Enrollment Updates (as of Aug 11, 2022)

• **108** individuals have signed informed consent

• **74** 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.
46 Sites Currently Activated for Regimen E

(as of 8/11/22)

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 Guest Speakers:

August 18th - Amanda Peltier, MD, MS (Site Investigator at Vanderbilt University in TN)
August 25th - Hani A. Kushlaf, MD, FAAN (Site Investigator at UCincinnati in OH)
September 1st - Hosted by James Berry, MD, MPH