HEALEY ALS Platform Trial

Weekly Q&A – Aug 26, 2021

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

Northeast Amyotrophic Lateral Sclerosis Consortium

Healey ALS
TACKLE ALS
MIDA
alsFINDINGaCURE
Jam ALS
ALS ONE
TAMBOURINE
The Arthur M. Blank Family Foundation

AMG
The AMG Foundation
Guest Speaker

Niraja Suresh, MD
University of South Florida, FL
Platform Trial Site Investigator
ALS Clinic at the University of South Florida (USF) is located at the Carol & Frank Morsani Center for Advanced Healthcare.

Established in 2010, the USF ALS Clinic provides multidisciplinary care for patients with ALS in the Tampa Bay and surrounding regions.

We serve average 250 to 350 patients per year, we are the only academic, tertiary referral center on the west coast of Florida.
University of South Florida

• Dr. Tuan Vu, Dr. Niraja Suresh, and Jerrica Farias

• Dr. Mark Armanious, Dr. Anthony Bradshaw, Dr. Rebecca Hurst, Dr. Cliff Gooch, Dr. Hera Stephens, Dr. Rossitza Chichkova, and Dr. Katie Murray

• Coordinators: Lucy Lam, Laura Alfonso
Perpetual Adaptive Trial
Shared Placebo; Randomization Ratio 3:1
Open Label Extension (OLE) offered

Screening → Regimen Assignment
(n=160 for each regimen)

Regimen A -> Regimen B -> Regimen C -> Regimen D
3:1 Randomization within each Regimen
(n=120 for active; n=40 for placebo)

Informed Consent → Assign to Regimen
Randomize within Regimen (active/placebo)

Zilucoplan
Placebo
Open Label Extension

Verdiperstat
Placebo
Open Label Extension

CNM-Au8
Placebo
Open Label Extension

Pridopidine
Placebo
Open Label Extension

Shared Placebo
Open Label Extension

Up to 6 weeks → 24 weeks (about 6 months)
Enrollment Updates (as of August 19, 2021)

• 717 individuals with ALS signed informed consent
• 565 individuals were assigned to a regimen
• 518 individuals were randomized within a regimen (active or placebo)
• 187 have entered the Open Label Extension (OLE)

• 140 individuals were randomized within Regimen A = 150
• 158 individuals were randomized within Regimen B = 160
• 156 individuals were randomized within Regimen C = 160
• 64 individuals were randomized within Regimen D = 67

Thank You

This breakthrough trial would not be possible without your partnership
How to Find a Center Near You

52 sites are actively enrolling

Contact Info of Participating Sites by State

https://www.massgeneral.org/neurology/als/research/platform-trial-sites
To see whether you might qualify, view the list of eligibility criteria:
Send us webinar ideas!

- Biomarkers
- Biostatistics / Trial Design
- Get to know our sites

Upcoming Guest Speakers:
Sept 2\textsuperscript{nd}- Terry Heiman-Patterson, MD (Temple University, PA)
Sept 9\textsuperscript{th}- Jennifer DiMartino (Executive Director, ALS ONE)
Sept 16\textsuperscript{th}- No webinar this week in observance of Yom Kippur
Sept 23\textsuperscript{rd}- Namita Goyal, MD (University of CA Irvine, CA)
Oct 7\textsuperscript{th}- No webinar this week
For More Updates

• **Weekly webinars**
  
The idea of came from our Patient Advisory Committee: we are excited to be talking with you on a weekly basis and take any questions you might have

• **Find the schedule and registration links on our website**
  
https://www.massgeneral.org/neurology/als/research/platform-trial-news/

**Previously: Drug mechanism of action and science webinars**

- **Jan 21** - Prilenia/Pridopidine  
  (recorded- https://bit.ly/3ad3qel)
- **Feb 4**  - Clene/CNM-Au8  
  (recorded- https://bit.ly/3jB3WWt)
- **Feb 18** - Biohaven/Verdiperstat  
- **Feb 25** - UCB/Zilucoplan  