HEALEY ALS Platform Trial

Weekly Q&A – Aug 19, 2021

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

Laura Foster, MD
University of Colorado Anschutz, CO
Platform Trial Site Investigator
Laura Foster, MD
University of Colorado – Healey ALS Platform Trial Research Team

Laura Foster, MD  
Assistant Professor

Stacy Dixon, MD, PhD  
Assistant Professor

Brianna Blume, MS, CCRP  
Neuromuscular Research Development Program Manager

Brenna Baines, BS  
Clinical Research Coordinator

Haley Simpson, BS  
Clinical Research Recruitment Specialist & Coordinator

Emily Hyslop, BA  
Clinical Research Coordinator

Diana Santacruz, BS  
Clinical Research Coordinator
CU's participation in the Healey ALS Platform Trial

- Well-established research team
- First participant enrolled October 2020
- 14 participants (as of Aug 18, 2021)
CU study visit sites
How to help screening process

• Tell us about every medication or supplement, including over-the-counter products
• Know where prior medical records are
Travel tips

• Bring a travel buddy
• Bring snacks
• Stay at a hotel nearby
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)

Zilucoplan

Verdiperstat

CNM-Au8

Pridopidine

Shared Placebo

Open Label Extension

Open Label Extension

Open Label Extension

Open Label Extension

Up to 6 weeks

24 weeks (about 6 months)

Informed Consent

Assign to Regimen

Randomize within Regimen (active/placebo)

Open Label Extension
Enrollment Updates (as of August 19, 2021)

- **705** individuals with ALS signed informed consent
- **558** individuals were assigned to a regimen
- **510** individuals were randomized within a regimen (active or placebo)
- **180** have entered the Open Label Extension (OLE)
- **139** individuals were randomized within Regimen A = **150**
- **154** individuals were randomized within Regimen B = **157**
- **155** individuals were randomized within Regimen C = **158**
- **62** individuals were randomized within Regimen D = **67**

**Total randomized + Participants in screening**

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**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
Catherine Small
Allison Bulat
Phone: 833-425-8257 (HALT ALS)
E-mail: healeyalsplatform@mgh.harvard.edu

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:
Aug 26th- Niraja Suresh, MD (University of South Florida, FL)
Sept 2nd- Terry Heiman-Patterson, MD (Temple University, PA)
Sept 9th- Jennifer DiMartino (Executive Director, ALS ONE)
Sept 16th- No webinar this week in observance of Yom Kippur
Sept 23rd- Namita Goyal, MD (University of CA Irvine, CA)
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/](https://www.massgeneral.org/neurology/als/research/platform-trial-news/)

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

Feb 4  - Clene/CNM-Au8  (recorded- [https://bit.ly/3jB3WWt](https://bit.ly/3jB3WWt))