HEALEY ALS Platform Trial

Weekly Q&A – Sept 30, 2021

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

[Logos and images of various organizations]
Guest Speaker

Irfan Qureshi, MD
Biohaven Pharmaceuticals
Vice President, Neurology
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

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 September 30, 2021)

• 773 individuals with ALS signed informed consent
• 615 individuals were assigned to a regimen
• 554 individuals were randomized within a regimen (active or placebo)
• 220 have entered the Open Label Extension (OLE)
• 150 individuals were randomized within Regimen A  = 161
• 167 individuals were randomized within Regimen B  = 168
• 160 individuals were randomized within Regimen C  = 161
• 77 individuals were randomized within Regimen D  = 91

Thank You
This breakthrough trial would not be possible without your partnership
52 sites are actively enrolling

Contact Info of Participating Sites by State

https://www.massgeneral.org/neurology/als/research/platform-trial-sites
Patient Navigator

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:**

Oct 7th- No webinar this week

Oct 14th- David Walk, MD FAAN (University of Minnesota, MN)

Oct 21st- TBD

Oct 28th- Megan Miller, PhD (IAMALS Director of Science and Policy)
An Update on AMX0035 and Design of the Phase 3 PHOENIX Study

Thursday, September 23, 2021
1:00 - 2:00 PM EDT

Merit Cudkowicz, MD, MSc
Sean M. Healey & AMG Center for ALS at Mass General Hospital

Sabrina Paganoni, MD, PhD
Sean M. Healey & AMG Center for ALS at Mass General Hospital

ALS Drug Development Webinar Series Part 6: Research Engagement by People Living With ALS

Wednesday, September 15, 2021
4:00 - 5:00 PM EDT

Richard Bedlack, MD, PhD
Duke ALS Clinic

Merit Cudkowicz, MD, MSc
Sean M. Healey & AMG Center for ALS at Mass General Hospital
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**

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