HEALEY ALS Platform Trial

Weekly Q&A – June 10, 2021
Perpetual Adaptive Trial
Shared Placebo; Randomization Ratio 3:1
Open Label Extension (OLE) offered

Screening → Regimen Assignment → 3:1 Randomization within each Regimen

Regimen A
Regimen B
(n=160 for each regimen)
Regimen C
Regimen D
(n=120 for active; n=40 for placebo)

Regimen Assignment
Informed Consent → Assign to Regimen → Randomize within Regimen (active/placebo) → Open Label Extension

Up to 6 weeks → 24 weeks (about 6 months)
Enrollment Updates (as of June 10, 2021)

- 582 individuals with ALS signed informed consent
- 452 individuals were assigned to a regimen
- 403 individuals were randomized within a regimen (active or placebo)
- 112 have entered the Open Label Extension (OLE)
- 110 individuals were randomized within Regimen A
- 127 individuals were randomized within Regimen B
- 127 individuals were randomized within Regimen C
- 39 individuals were randomized within Regimen D

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
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  
  [view recording](https://bit.ly/3ad3qel)
- **Feb 4** - Clene/CNM-Au8  
  [view recording](https://bit.ly/3jB3WWt)
- **Feb 18**- Biohaven/Verdiperstat  
  [view recording](https://bit.ly/301qdDR)
- **Feb 25**- UCB/Zilucoplan  
  [view recording](https://bit.ly/3b3cpyS)
Drug Development NEALS Webinar Series

This series will include talks on the preclinical development of potential therapies, the regulatory pathway, the science of trials, the clinical trial site level overview, and the importance of patient engagement.

Part 1: Scientific Considerations (Recorded April 7)
Part 2: Regulatory Considerations (Recorded April 9)
Part 3: Ethical Considerations (Recorded May 5)
Part 4: Site Considerations (Recorded May 14)

Part 5: Preclinical Development of Potential Therapies
       June 23, 2021 @ 3:30-4:30pm ET

Part 6: Research Engagement by People Living with ALS
       Sept 15, 2021 @ 4:00-5:00pm ET

An Update on the CENTAUR Trial: a Trial of AMX0035 in ALS

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

Monday, May 17, 2021
2:00 - 3:00 PM EDT

ALS Clinical Trial Pipeline Webinar

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

Monday, June 7, 2021
5:00 - 6:00 PM EDT

Dr. Cudkowicz will review the ALS clinical trial pipeline and answers questions about the current state of clinical research.

View recordings here:

www.neals.org
Send us webinar ideas!

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

Next Week’s Guest: Dr. Lauren Elman of UPenn
To see whether you might qualify, view the list of eligibility criteria online: https://bit.ly/3p4PrLL