Perpetual Adaptive Trial
Shared Placebo; Randomization Ratio 3:1
Open Label Extension (OLE) offered

Screening
Regimen Assignment
(n=160 for each regimen)

Regimen A
(n=160 for each regimen)

Regimen B
(n=120 for active; n=40 for placebo)

Regimen C

Regimen D

3:1 Randomization within each Regimen

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

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 July 8, 2021)

- 637 individuals with ALS signed informed consent
- 499 individuals were assigned to a regimen
- 454 individuals were randomized within a regimen (active or placebo)
- 138 have entered the Open Label Extension (OLE)

- 123 individuals were randomized within Regimen A
- 141 individuals were randomized within Regimen B
- 141 individuals were randomized within Regimen C
- 49 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

as of 7/8/21
Catherine Small
Allison Bulat
Phone: 833-425-8257 (HALT ALS)
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To see whether you might qualify, view the list of eligibility criteria online:
Send us webinar ideas!

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

Upcoming Guest Speakers:
July 15th- Shafeeq Ladha, MD (Barrow Neurological Institute, AZ)
July 22nd- Richard Bedlack, MD, PhD, MS (Duke University, NC)
Aug 12th- Michael Weiss, MD, FAAN (University of Washington, WA)
Aug 19th- Laura Foster, MD (University of Colorado, CO)
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**

  **Jan 21**- Prilenia/Pridopidine  

  **Feb 4** - Clene/CNM-Au8  

  **Feb 18**- Biohaven/Verdiperstat  

  **Feb 25**- UCB/Zilucoplan  