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

- **Screening**
- **Regimen Assignment**
  - Regimen A: (n=160 for each regimen)
- **3:1 Randomization within each Regimen**
  - Regimen B
  - Regimen C
  - Regimen D: (n=120 for active; n=40 for placebo)

- **Open Label Extension**
  - Zilucoplan
  - Verdiprestat
  - CNM-Au8
  - Pridopidine
  - Shared Placebo

**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 24, 2021)

• 609 individuals with ALS signed informed consent
• 477 individuals were assigned to a regimen
• 429 individuals were randomized within a regimen (active or placebo)
• 128 have entered the Open Label Extension (OLE)

• 116 individuals were randomized within Regimen A
• 135 individuals were randomized within Regimen B
• 136 individuals were randomized within Regimen C
• 42 individuals were randomized within Regimen D

Thank You
This breakthrough trial would not be possible without your partnership
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 1\(^{st}\)- Jeffrey Rosenfeld, MD (Loma Linda University, CA)
July 15\(^{th}\)- Shafeeq Ladha, MD (Barrow Neurological Institute, AZ)
July 22\(^{nd}\)- Richard Bedlack, MD, PhD, MS (Duke University, NC)
Aug 12\(^{th}\)- Michael Weiss, MD, FAAN (University of Washington, WA)
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 18**- Biohaven/Verdiperstat (view recording- https://bit.ly/301qdDR)
An Update on the CENTAUR Trial: a Trial of AMX0035 in ALS

Recording Available

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

Merit Cudkowicz, MD, MSc
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

Recording Available

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