Guest Speaker

Jeffrey Rosenfeld, PhD, MD, FAAN
Loma Linda University, CA
Platform Trial Site Investigator

- Professor and Associate Chairman Department of Neurology
- Director, Neuromuscular & ALS/MND Programs
- Medical Director, Center for Restorative Neurology
CRN at LLUH Platform Trial Research Team

Sedano, Josie

Qasim, Syed

Borg, David

Baroya, Sheba S.
Perpetual Adaptive Trial
Shared Placebo; Randomization Ratio 3:1
Open Label Extension (OLE) offered

- **Regimen A**
  - (n=160 for each regimen)
  - Placebo

- **Regimen B**
  - 3:1 Randomization within each Regimen
  - Placebo

- **Regimen C**
  - Placebo

- **Regimen D**
  - Placebo

- **Zilucoplan**
  - Placebo
  - (n=120 for active; n=40 for placebo)

- **Verdiperstat**
  - Placebo

- **CNM-Au8**
  - Placebo

- **Pridopidine**
  - Placebo

- **Shared Placebo**

**Screening**

**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 July 1, 2021)

• 627 individuals with ALS signed informed consent
• 492 individuals were assigned to a regimen
• 444 individuals were randomized within a regimen (active or placebo)
• 135 have entered the Open Label Extension (OLE)
• 120 individuals were randomized within Regimen A
• 138 individuals were randomized within Regimen B
• 139 individuals were randomized within Regimen C
• 47 individuals were randomized within Regimen D

Thank You
This breakthrough trial would not be possible without your partnership
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 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/

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

- **Jan 21** - Prilenia/Pridopidine  
- **Feb 4** - Clene/CNM-Au8  
  (view recording- https://bit.ly/3jB3WWt)
- **Feb 18** - Biohaven/Verdiperstat  
- **Feb 25** - UCB/Zilucoplan  