HEALEY ALS Platform Trial

Weekly Q&A – May 6, 2021
Perpetual Adaptive Trial
Randomization Ratio 3:1; Shared Placebo
Open Label Extension (OLE) offered

Screening ➢ Regimen Assignment

Regimen A
(n=160 for each regimen)

Regimen B

Regimen C

Regimen D

3:1 Randomization within each Regimen

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Zilucoplan

Verdiperstat

CNM-Au8

Pridopidine

Shared Placebo

Screening ➢ 24 weeks on investigational product (active:placebo = 3:1)

➢ as of 5/6/21
Enrollment Updates (as of May 6, 2021)

• 485 individuals with ALS signed informed consent
• 369 individuals were assigned to a regimen
• 323 individuals were randomized within a regimen (active or placebo)
• 82 have entered the Open Label Extension (OLE)

• 90 individuals were randomized within Regimen A
• 104 individuals were randomized within Regimen B
• 106 individuals were randomized within Regimen C
• 23 individuals were randomized within Regimen D

Thank You

This breakthrough trial would not be possible without your participation
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
List of enrolling sites as of May 6, 2021
52 sites are enrolling

- Texas Neurology
- Mass General Hospital
- UTHSCSA
- Hospital for Special Care
- Holy Cross Hospital
- Thomas Jefferson
- Houston Methodist
- Henry Ford Health System
- Barrow Neurological Institute
- Ohio State University
- Northwestern University
- University of Chicago
- Wake Forest
- University of Nebraska
- Loma Linda University
- University of Washington
- University of Iowa
- Washington University
- University of Pennsylvania
- University of Michigan
- California Pacific Medical Center
- Penn State Hershey
- UMass Worcester
- University of Miami
- University of Colorado
- Cedars-Sinai
- University of Florida
- University of South Florida
- Columbia University
- University of Virginia
- Emory University
- University of Maryland
- SUNY Upstate
- Beth Israel Deaconess
- Temple University
- Dartmouth-Hitchcock
- Medical College of Wisconsin
- Spectrum Health
- University of Missouri
- University of Minnesota
- Johns Hopkins University
- University of CA Irvine
- University of Kansas
- Vanderbilt University
- University of Kentucky
- Mayo Rochester
- Duke University
- Neurology Associates
- Ochsner Health System
- Mayo Clinic Florida
- St. Louis University
- Providence Brain and Spine
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)
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 –Friday, May 14th 3:30-4:30pm EDT

Register to attend and view recordings at https://bit.ly/3frQsfB
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.

Register to attend and view recordings here:

www.neals.org
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

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

Register to attend and view recordings here:
www.neals.org
Send us webinar ideas!

- Biomarkers
- Biostatistics / Trial Design
- Get to know our 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 online: