HEALEY ALS Platform Trial

Weekly Q&A - June 24, 2021

















Healey Center

Sean M. Healey & AMG Center for ALS at Mass General































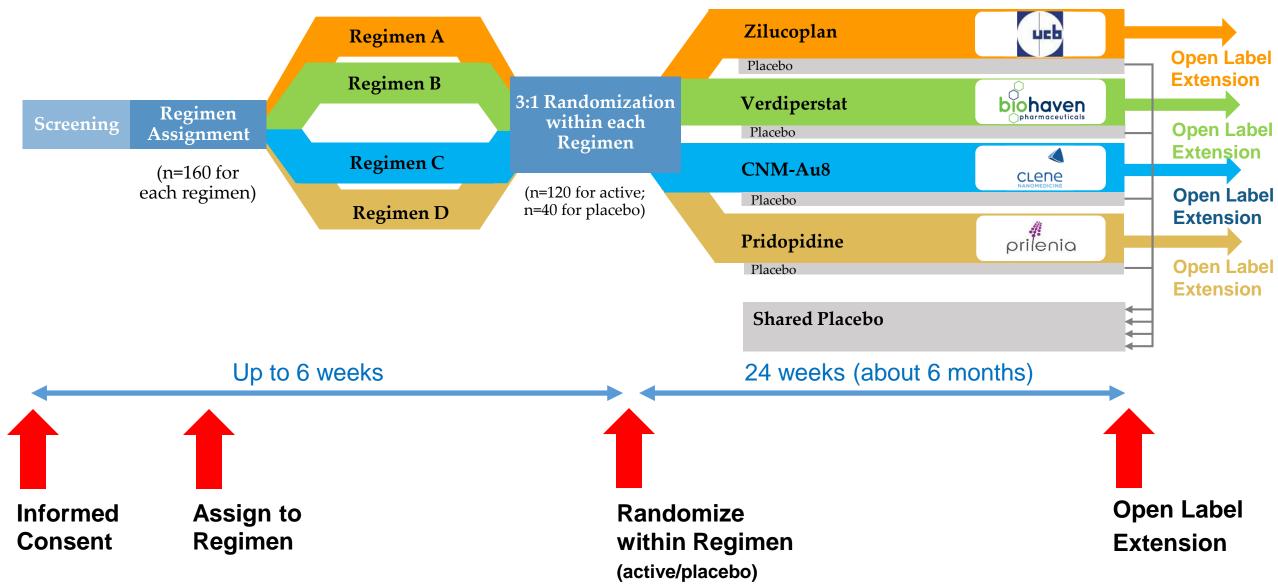








Perpetual Adaptive Trial Shared Placebo; Randomization Ratio 3:1 Open Label Extension (OLE) offered



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



Patient Navigator



Catherine Small Allison Bulat

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To see whether you might qualify, view the list of eligibility criteria online:

https://bit.ly/3p4PrLL

Send us webinar ideas!

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



Upcoming Guest Speakers:

July 1st- Jeffrey Rosenfeld, MD (Loma Linda University, CA)
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)

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

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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)
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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