HEALEY ALS Platform Trial

Weekly Q&A - July 8, 2021

















Healey Center

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





























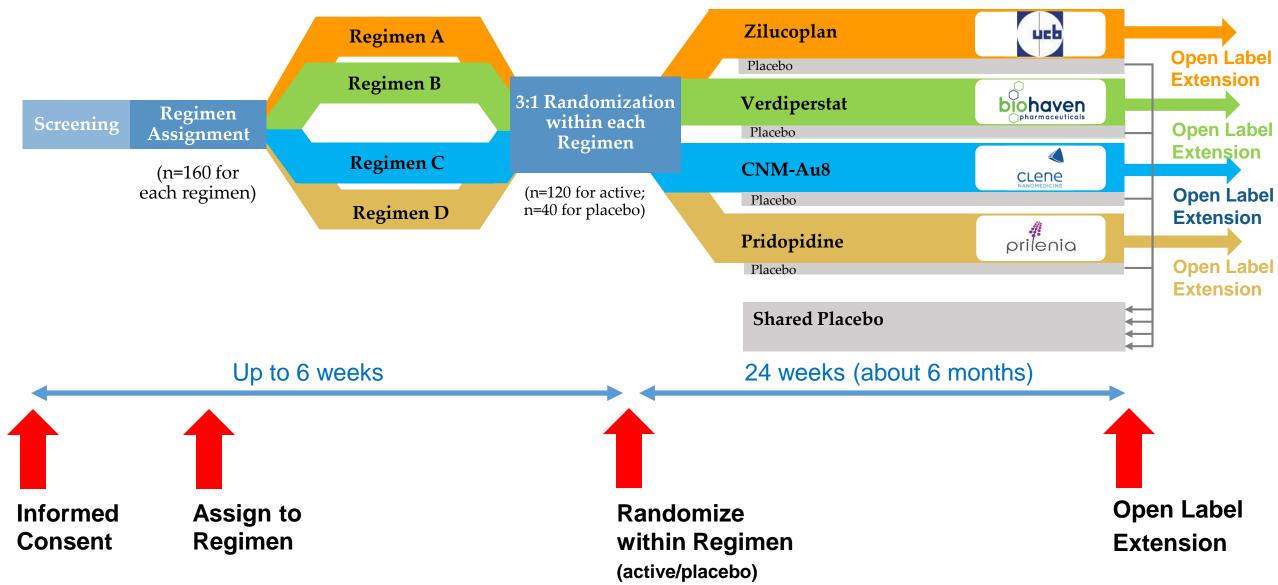








Perpetual Adaptive Trial Shared Placebo; Randomization Ratio 3:1 Open Label Extension (OLE) offered

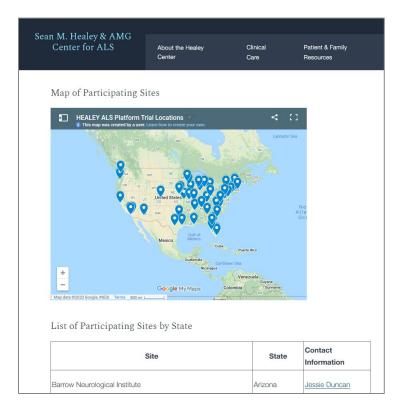


Enrollment Updates (as of July 8, 2021)

- 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

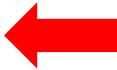


How to Find a Center Near You



52 sites are actively enrolling

Contact Info of Participating Sites by State



https://www.massgeneral.org/neurology/als/research/platform-trial-sites

Patient Navigator



Catherine Small Allison Bulat

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E-mail:healeyalsplatform@mgh.harvard.edu



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 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 (view recording- https://bit.ly/3ad3qel)

Feb 4 - Clene/CNM-Au8 (<u>view recording</u>- https://bit.ly/3jB3WWt)

Feb 18- Biohaven/Verdiperstat (view recording- https://bit.ly/301qdDR)

Feb 25- UCB/Zilucoplan (view recording- https://bit.ly/3b3cpyS)