HEALEY ALS Platform Trial

Weekly Q&A - March 25, 2021













BARROW

Neurological Institute*



Healey Center

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































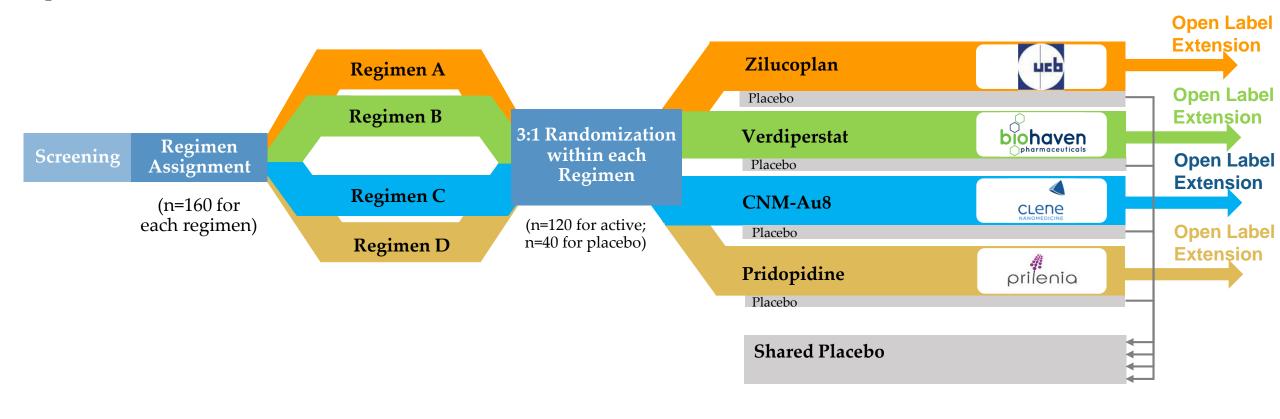






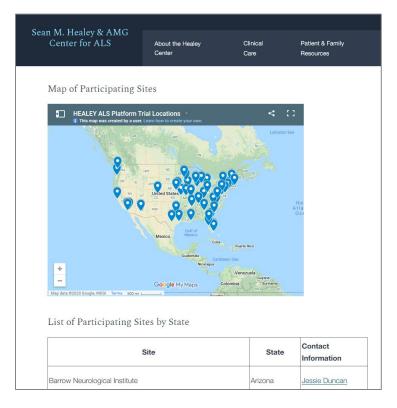
Perpetual Adaptive Trial Randomization Ratio 3:1; Shared Placebo Open Label Extension offered

Screening



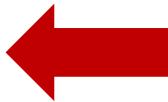
24 weeks on investigational product (active:placebo = 3:1)

How to Find a Center Near You



48 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 March 25, 2021 48 sites are enrolling

- **™** Texas Neurology
- **™** Mass General Hospital
- **™ UTHSCSA**

- **™** Thomas Jefferson
- **™** Houston Methodist
- **☑** 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
- **™** University of CA Irvine
- **✓ University of Kansas**
- **✓ Vanderbilt University**
- **☑** University of Kentucky
- **™** Mayo Rochester
- **☑** Duke University
- **™** Neurology Associates

Enrollment Updates



Enrollment Updates (as of March 25, 2021)

- 372 individuals with ALS signed informed consent
- 293 individuals were assigned to a regimen
- 262 are receiving investigational product (active or placebo)

We will continue to update the ALS community on enrollment (website, webinars)

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

https://bit.ly/3p4PrLL

Patient Navigator

Catherine Small Allison Bulat

Phone: 833-425-8257 (HALT ALS)

E-mail:healeyalsplatform@mgh.harvard.edu

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https://bit.ly/3p4PrLL

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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Send us webinar ideas!

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



