HEALEY ALS Platform Trial

Weekly Q&A - March 4, 2021













BARROW

Neurological Institute*



Healey Center

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





































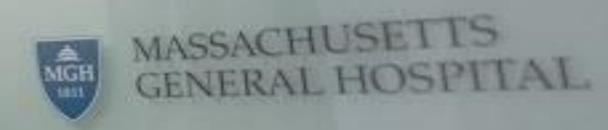
AGENDA

- Updates on the HEALEY ALS Platform Trial
 - Investigational Products
 - Activated Sites
 - Enrollment
- How to stay in touch and find a site near you

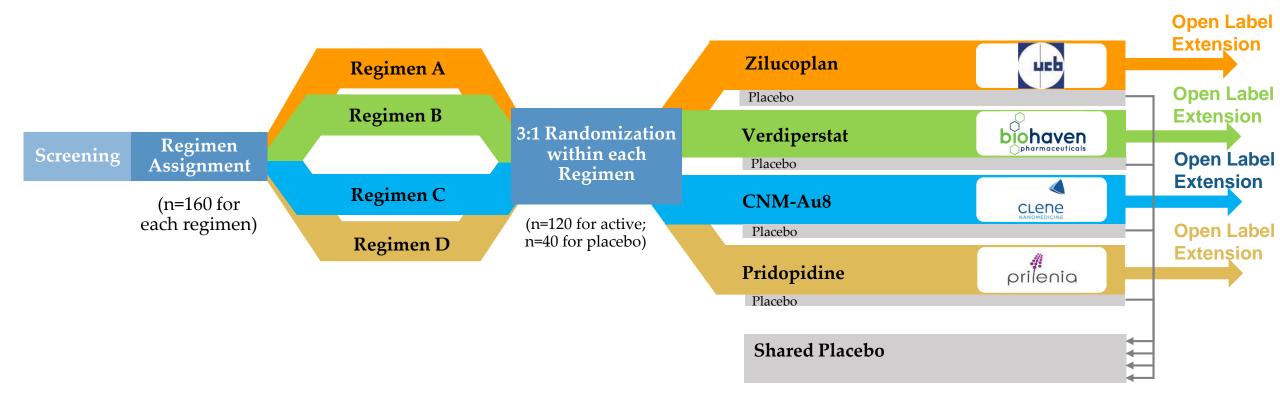
YOUR QUESTIONS

Healey Center Sean M. Healey & AMG Center for Al S at Maco

for ALS at Mass General



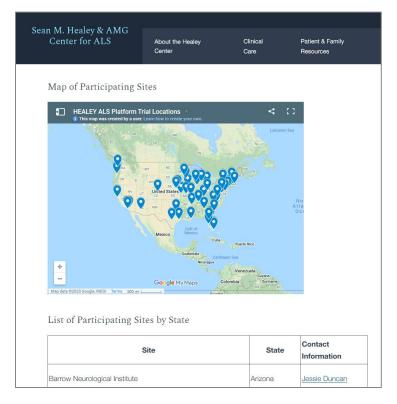
Perpetual Adaptive Trial Randomization Ratio 3:1; Shared Placebo Open Label Extension offered



Screening

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

How to Find a Center Near You



44 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 4, 2021 44 sites are enrolling

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

- **™** Thomas Jefferson
- **™** Houston Methodist
- **☑** Henry Ford Health System
- **☑** Barrow Neurological Institute
- **™** Ohio State University
- **✓** Northwestern University
- **™** 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**
- **☑** University of Florida
- **✓ University of South Florida**
- **☑** Columbia University
- **™** University of Virginia

- **Emory**
- **☑** 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**

Enrollment Updates (as of March 4, 2021)

- 358 individuals with ALS signed informed consent
- 285 individuals were assigned to a regimen
- 248 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

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

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

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

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