• To submit text questions to today’s presenters, please type your questions into the ‘Q&A’ box

• Today’s presentation is being recorded and will be posted on the Healey & AMG Center for ALS website

https://www.massgeneral.org/neurology/als/news/webinars
HEALEY ALS Platform Trial

Weekly Q&A – Jan 14, 2021

Healey Center

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

• Updates on the HEALEY ALS Platform Trial
  - Study Treatments
  - Sites
  - Enrollment

• How to stay in touch and find a site near you

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

Screening

Regimen A
(\(n=160\) for each regimen)

Regimen B

Regimen C
(\(n=120\) for active drug; \(n=40\) for placebo)

Regimen D

3:1 Randomization within each Regimen

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Shared Placebo

Screening

24 weeks on study drug (active:placebo = 3:1)

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as of 1/14/21
How to Find a Center Near You

37 sites are actively enrolling

Contact Info of Participating Sites by State

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

as of 1/14/21
List of enrolling sites as of Jan 14, 2021
37 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 Nebraska
- 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
Enrollment Updates (as of Jan 14, 2021)

- **272** individuals with ALS signed informed consent
- **211** individuals were assigned to a regimen
- **186** are currently receiving study drug (active or placebo)
- First participant assigned to Regimen D Pridopidine


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

Catherine Small
Allison Bulat

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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/

  **Coming up: Drug mechanism of action and science webinars**
  
  Jan 21 - Prilenia/Pridopidine
  Feb 4  - Clene/CNM-Au8
  Feb 18- Biohaven/Verdiperstat
  Feb 25- UCB Ra Pharma/Zilucoplan