WE WILL BEGIN SHORTLY

• 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 7, 2021
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

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)

➢ as of 1/7/21
How to Find a Center Near You

34 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 Jan 7, 2021
34 sites are enrolling

- Texas Neurology
- MGH
- 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 Medical Center
- Loma Linda
- University of Washington
- University of Iowa
- Washington University
- University of Pennsylvania
- University of Michigan
- Forbes Norris MDA/ALS Research Center, CPMC
- Penn State Hershey
- UMass
- University of Miami
- University of Colorado
- Cedars-Sinai
- University of Florida
- University of South Florida
- Columbia University
- University of Virginia
- Emory
- University of Maryland
- SUNY Upstate
- Beth Israel Deaconess Medical Center
Enrollment Updates (as of Jan 7, 2021)

• **257** individuals with ALS signed informed consent
• **203** individuals were assigned to a regimen
• **181** are currently receiving study drug (active or placebo)

*We will continue to update the ALS community on enrollment (website, webinars)*
Expanded Access Program (EAP)

• Great interest in EAPs from the patient community

• Long term goal is to build an EAP in parallel to the Platform Trial
  - The idea came from the Patient Advisory Committee
  - The EAP would allow access to patients who are not eligible for the trial

• We are working to achieve this goal
  - We are actively fundraising to support this effort
  - One grant has been received from I AM ALS to start building the EAP

https://www.massgeneral.org/neurology/als/news/2020-12-18-iamals-eap-grant

as of 1/7/21
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**

  [website link]

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