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 – Dec 10, 2020

Healey Center
Sean M. Healey & AMG Center for ALS at Mass General
• Updates on the HEALEY ALS Platform Trial
  - Study Treatments
  - Sites
  - Enrollment

• How to stay in touch and find a site near you
Perpetual Adaptive Trial
Randomization Ratio 3:1; Shared Placebo
Open Label Extension offered

Screening

Regimen Assignment
(n=160 for each regimen)

Regimen A

Regimen B

Regimen C

Regimen D

3:1 Randomization within each Regimen
(n=120 for active drug; n=40 for placebo)

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Shared Placebo

Open Label Extension

Screening

24 weeks on study drug (active:placebo = 3:1)
How to Find a Center Near You

33 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 Dec 10, 2020
33 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
Enrollment Updates (as of Dec 10, 2020)

- 227 individuals with ALS signed informed consent
- 177 individuals were assigned to a regimen
- 153 are currently receiving study drug (active or placebo)

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

  **Coming up: webinars about each drug’s mechanism of action and science**