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
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

Screening
Regimen Assignment
(n=160 for each regimen)

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

Regimen B
Regimen C
Regimen D

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Shared Placebo

Open Label Extension starting Jan 2021 (A,B,C)

Information up to date as of 12/17/20
How to Find a Center Near You

All 54 sites are IRB approved!
33 sites are actively enrolling
Call for new sites! Suggestions?

Contact Info of Participating Sites by State

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

Information up to date as of 12/17/20
List of enrolling sites as of Dec 17, 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

Information up to date as of 12/17/20
Enrollment Updates (as of Dec 17, 2020)

• 239 individuals with ALS signed informed consent
• 186 individuals were assigned to a regimen
• 164 are currently receiving study drug (active or placebo)

We will continue to update the ALS community on enrollment (website, webinars)
ONGOING COVID-19 IMPACT

• Many centers are experiencing a second surge
  - staff redeployment
  - some institutions are limiting research activities

• Supply chain problems (central vendor)
• Home nursing shortages
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/

  Early 2021: webinars about each drug’s mechanism of action and science