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
• 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
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
3:1 Randomization within each Regimen

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

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Shared Placebo

Open Label Extension

Screening

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

➢ 4

as of 1/28/21
How to Find a Center Near You

38 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 28, 2021
38 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 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
Enrollment Updates (as of Jan 28, 2021)

- **292** individuals with ALS signed informed consent
- **234** individuals were assigned to a regimen
- **200** are receiving investigational product (active or placebo)

*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 (Recording available)
  https://www.youtube.com/watch?v=YscMkGaDOhg
- **Feb 4**: Clene/CNM-Au8
- **Feb 18**: Biohaven/Verdiperstat
- **Feb 25**: UCB Ra Pharma/Zilucoplan