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
  - Investigational Products
  - Activated 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; n=40 for placebo)

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Shared Placebo

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

as of 2/11/21
How to Find a Center Near You

42 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 Feb 11, 2021
42 sites are enrolling

<table>
<thead>
<tr>
<th>Texas Neurology</th>
<th>Loma Linda University</th>
<th>Columbia University</th>
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<tr>
<td>Mass General Hospital</td>
<td>University of Washington</td>
<td>University of Virginia</td>
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<td>UTHSCSA</td>
<td>University of Iowa</td>
<td>Emory</td>
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<td>Hospital for Special Care</td>
<td>Washington University</td>
<td>University of Maryland</td>
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<td>Holy Cross Hospital</td>
<td>University of Pennsylvania</td>
<td>SUNY Upstate</td>
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<td>Thomas Jefferson</td>
<td>University of Michigan</td>
<td>Beth Israel Deaconess</td>
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<td>California Pacific Medical Center</td>
<td>Temple University</td>
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<td>Penn State Hershey</td>
<td>Dartmouth-Hitchcock</td>
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<td>University of Nebraska</td>
<td>University of South Florida</td>
<td>University of CA Irvine</td>
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Enrollment Updates (as of Feb 11, 2021)

- 323 individuals with ALS signed informed consent
- 257 individuals were assigned to a regimen
- 217 are receiving investigational product (active or placebo)

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

To see whether you might qualify, view the list of eligibility criteria online: https://bit.ly/3p4PrLL
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: Drug mechanism of action and science webinars**

- **Jan 21** - Prilenia/Pridopidine (Recording available)
  https://bit.ly/3ad3qel
- **Feb 4** - Clene/CNM-Au8 (Recording available)
  https://bit.ly/3jB3WWt
- **Feb 18** - Biohaven/Verdiperstat
- **Feb 25** - UCB Ra Pharma/Zilucoplan