HEALEY ALS Platform Trial

Updates – Sept 29, 2020

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AGENDA

• Updates on the HEALEY ALS Platform Trial
  - Enrollment
  - Study Treatments
  - Sites
• How to stay in touch and find a site near you
• What’s next and EAPs
BRINGING TOGETHER A COMMUNITY to launch the first platform trial for ALS
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COVID-19 IMPACT

- Most centers experienced major disruptions to normal workflow
- Limited to no ability to start new studies / enroll new research participants (prohibited at many sites)
- Staff redeployment and furloughs
- Substantial restrictions on staff access to the clinic and research areas with most staff working remotely
- Restrictions on certain procedures (respiratory function testing still prohibited at many sites)
- Regional variability means that some sites have experienced different barriers at different times which might affect their timelines
We remained committed to the trial through COVID and were able to start the trial despite the pandemic.

- Lots of activities happened remotely
  - Training, contracts, and administrative tasks to enable site activation

- Protocol amendments made the trial “COVID proof”

Enrollment Started in July 2020!
Enrollment Updates

• **96** individuals with ALS signed informed consent
• **48** are currently on study drug

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

Questions about enrollment? Please contact the Patient Navigator

Phone: 833-425-8257 (HALT ALS)
E-mail: healeyalsplatform@mgh.harvard.edu
HEALEY ALS Platform Trial offers several benefits over traditional trials.

1. **Shared infrastructure and common protocol, allowing sharing of placebo participants**
2. **Randomization**
3. **Enroll in Platform Trial**
4. **Cuts costs by 1/3**
5. **Cuts time in 1/2**
6. **Increases Active by 3X**

**Traditional Clinical Trial vs. HEALEY ALS Platform Trial**

- **Cuts costs by 1/3**
- **Cuts time in 1/2**

**Benefits of HEALEY ALS Platform Trial**

- **Active**
- **Placebo**
- **Active Placebo**

**Comparison:**
- Traditional clinical trials cut costs by 1/3 and cut time in 1/2, while HEALEY ALS Platform Trial increases active by 3X through shared infrastructure and common protocol.
Perpetual adaptive trial-
Open Label Extension offered

Screening | 1:1:1 Randomization | Regimen A | Regimen B | Regimen C | 3:1 Randomization for each Regimen
(n=160 for each regimen) | (n=120 for active drug; n=40 for placebo)

Zilucoplan | Placebo
Verdiperstat | Placebo
CNM-Au8 | Placebo

Open Label Extension

Treatments were selected by ALS scientists and experts based on a competitive process

24 weeks on study drug (active:placebo = 3:1)
We want to keep adding great therapies

• Submissions accepted on a rolling basis

• We opened with 3 regimens

• The 4\textsuperscript{th} regimen will be added in 2020 (pridopidine, Prilenia)

• We are in discussions with 3 additional companies for 2021!

https://www.massgeneral.org/neurology/als/research/platform-trial
How to Find a Center Near You

- 22 sites are actively enrolling!
- More will be added once the first 54 have been activated

Contact Info of Participating Sites by State

https://www.massgeneral.org/neurology/als/research/platform-trial
How to Receive Platform Trial Updates

Sign up for the ALS Link Newsletter (for info on this trial and other research opportunities)

https://www.massgeneral.org/neurology/als/services/clinical-trials-enrollment-coordination
For More Updates

• **Weekly informal Q&A (starting next week!) + monthly 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/news/webinars
EXPANDED ACCESS PROGRAM (EAP) PLANS

• **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 submitted one grant to start building the EAP program
  - We are actively fundraising to support this effort
  - Please visit our website for more information:
    - [https://www.massgeneral.org/neurology/als/research/expanded-access](https://www.massgeneral.org/neurology/als/research/expanded-access)
QUESTIONS

• 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/research/platform-trial