HEALEY ALS Platform Trial

Weekly Q&A – July 29, 2021

Healey Center
Sean M. Healey & AMG Center for ALS at Mass General
Guest Speaker

Hristelina Ilieva, MD, PhD
Thomas Jefferson, PA
Platform Trial Site Investigator
Piera Pasinelli, PhD -
  • Director

Hristelina Ilieva, MD, PhD -
  • Clinical Director and PI of Healey Platform Trial

Davide Trotti, PhD
  • Research Director

Jessica Rinaldi -
  • Associate Director of Clinical Research

Stephanie Hansen, Madeleine Singson, Sofia Nastri, Christopher Hague, Melissa Warren
  • Clinical Research Team
Jefferson Weinberg ALS Center

4 RESEARCH LABORATORIES
(BASIC-TRANSLATIONAL)

INTER-DISCIPLINARY CLINIC
(Standard of care + Pulmonology; Physiatry; Palliative Care; Research Education ALSA Staff)

CLINICAL RESEARCH

MENTAL HEALTH
SOCIAL WORK
ALSIA
Healey Platform Trial

- Our site started enrolling patients in August of 2020, and enrollment is still ongoing.

- Our site currently has 11 patients participating in this trial.
  - 3 on Regimen A, 3 on Regimen B, 4 on Regimen C, 1 on Regimen D.
  - 5 patients have moved on to the open label extension, 6 remain on double-blind.
  - 2 patients have completed DB phase but unable to continue with OLE due to disease progression.
Perpetual Adaptive Trial
Shared Placebo; Randomization Ratio 3:1
Open Label Extension (OLE) offered

Screening
Regimen Assignment
Regimen A (n=160 for each regimen)
Regimen B
Regimen C
Regimen D

3:1 Randomization within each Regimen

Regimen A: CNM-Au8 Placebo
Regimen B: Verdiperstat Placebo
Regimen C: CNM-Au8 Placebo
Regimen D: Pridopidine Placebo

Up to 6 weeks
24 weeks (about 6 months)

Informed Consent
Assign to Regimen
Randomize within Regimen (active/placebo)
Open Label Extension
Enrollment Updates (as of July 29, 2021)

- 676 individuals with ALS signed informed consent
- 534 individuals were assigned to a regimen
- 482 individuals were randomized within a regimen (active or placebo)
- 155 have entered the Open Label Extension (OLE)

- 130 individuals were randomized within Regimen A
- 149 individuals were randomized within Regimen B
- 148 individuals were randomized within Regimen C
- 55 individuals were randomized within Regimen D

Thank You

This breakthrough trial would not be possible without your partnership
How to Find a Center Near You

52 sites are actively enrolling

Contact Info of Participating Sites by State

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

Catherine Small
Allison Bulat
Phone: 833-425-8257 (HALT ALS)
E-mail: healeyalsplatform@mgh.harvard.edu

To see whether you might qualify, view the list of eligibility criteria:
Send us webinar ideas!

- Biomarkers
- Biostatistics / Trial Design
- Get to know our sites

**Upcoming Guest Speakers:**

Aug 5th- TBD

Aug 12th- Michael Weiss, MD, FAAN (University of Washington, WA)

Aug 19th- Laura Foster, MD (University of Colorado, CO)

Aug 26th- TBD
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/

**Previously: Drug mechanism of action and science webinars**

Jan 21- Prilenia/Pridopidine (recorded- https://bit.ly/3ad3qel)
Feb 4  - Clene/CNM-Au8 (recorded- https://bit.ly/3jB3WWt)