HEALEY ALS Platform Trial

Weekly Q&A – May 13, 2021

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

Northeast Amyotrophic Lateral Sclerotic Consortium

Healey Center

biohaven pharmaceuticals
clene NANOMEDICINE
prilenia
SEELOS THERAPEUTICS

The AMG Foundation
Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) 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

Open Label Extension

Open Label Extension

Open Label Extension

Informed Consent

Assign to Regimen

Randomize within Regimen (active/placebo)

Up to 6 weeks

24 weeks (about 6 months)
Enrollment Updates (as of May 13, 2021)

• 512 individuals with ALS signed informed consent
• 391 individuals were assigned to a regimen
• 338 individuals were randomized within a regimen (active or placebo)
• 89 have entered the Open Label Extension (OLE)

• 93 individuals were randomized within Regimen A
• 111 individuals were randomized within Regimen B
• 109 individuals were randomized within Regimen C
• 25 individuals were randomized within Regimen D

Thank You

This breakthrough trial would not be possible without your participation
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
List of enrolling sites as of May 13, 2021
52 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
- University of Maryland
- SUNY Upstate
- Beth Israel Deaconess
- Temple University
- Dartmouth-Hitchcock
- Medical College of Wisconsin
- Spectrum Health
- University of Missouri
- University of Minnesota
- Johns Hopkins University
- University of CA Irvine
- University of Kansas
- Vanderbilt University
- University of Kentucky
- Mayo Rochester
- Duke University
- Neurology Associates
- Ochsner Health System
- Mayo Clinic Florida
- St. Louis University
- Providence Brain and Spine
New Online Resources!

Online Eligibility Checking Tool  https://bit.ly/3ezu4Qx

Find Out If You're Eligible

We’ve prepared a short list of questions to help you find out if you might be eligible to participate in the HEALEY ALS Platform Trial.

Please note that the result of this survey does not serve as official confirmation of your eligibility, as many factors are considered prior to study participation (view a full list of inclusion / exclusion criteria here). Determining eligibility for the Platform Trial will depend on a thorough assessment of your clinical symptoms, review of past medical history, and lab work that can only be performed by an investigator (doctor or nurse practitioner) on the trial.
New Online Resources!

Download the Digital Drug Brochure from our website:
https://bit.ly/2Q9N3aZ
What to Expect in the Trial Process

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FAQ: Which supplements/interventions are exclusionary?

Guiding Principles:
- Any supplements/medications that are in a trial are considered exclusionary (as they might affect the efficacy of the treatment under evaluation)
- Additional supplements/medications might be exclusionary based on safety and interactions with the treatment under evaluation
- Examples of exclusionary agents: TUDCA, sodium phenylbutyrate, curcumin, very high doses of methylcobalamin

→ Most trials have a list of exclusionary supplements/medications, but each trial has a different list
→ The list might change as new trials open and others end
→ Note: a washout period prior to Master Protocol Screening is required (30 days or 5 half-lives if known, whichever is longer)
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/](https://www.massgeneral.org/neurology/als/research/platform-trial-news/)

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

- **Jan 21-** Prilenia/Pridopidine  
  [view recording](https://bit.ly/3ad3qel)

- **Feb 4 -** Clene/CNM-Au8  
  [view recording](https://bit.ly/3jB3WWt)

- **Feb 18-** Biohaven/Verdiperstat  
  [view recording](https://bit.ly/301qdDR)

- **Feb 25-** UCB/Zilucoplan  
  [view recording](https://bit.ly/3b3cpyS)
Drug Development NEALS Webinar Series

This series will include talks on the preclinical development of potential therapies, the regulatory pathway, the science of trials, the clinical trial site level overview, and the importance of patient engagement.

Part 1: Scientific Considerations (Recorded April 7)
Part 2: Regulatory Considerations (Recorded April 9)
Part 3: Ethical Considerations (Recorded May 5)
Part 4: Site Considerations –Friday, May 14th 3:30-4:30pm EDT

Register to attend and view recordings at https://bit.ly/3frQsfB
An Update on the CENTAUR Trial: a Trial of AMX0035 in ALS

Sabrina Paganoni, MD, PhD
Sean M. Healey & AMG Center for ALS at Mass General Hospital

Merit Cudkowicz, MD, MSc
Sean M. Healey & AMG Center for ALS at Mass General Hospital

Monday, May 17, 2021
2:00 - 3:00 PM EDT

Register to attend and view recordings here:

www.neals.org

ALS Clinical Trial Pipeline Webinar

Merit Cudkowicz, MD, MSc
Sean M. Healey & AMG Center for ALS at Mass General Hospital
Monday, June 7, 2021
5:00 - 6:00 PM EDT

Dr. Cudkowicz will review the ALS clinical trial pipeline and answers questions about the current state of clinical research.
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 online: