HEALEY ALS Platform Trial

Weekly Q&A – May 20, 2021

Healey Center
Sean M. Healey & AMG Center for ALS at Mass General
Perpetual Adaptive Trial
Shared Placebo; Randomization Ratio 3:1
Open Label Extension (OLE) offered

**Regimen A** (n=160 for each regimen)
- Regimen A
- CNM-Au8
- Placebo
- Open Label Extension

**Regimen B**
- Regimen B
- Verdiperstat
- Placebo
- Open Label Extension

**Regimen C**
- Regimen C
- CNM-Au8
- Placebo
- Open Label Extension

**Regimen D**
- Regimen D
- Pridopidine
- Placebo
- Open Label Extension
- Zilucoplan
- Placebo
- Open Label Extension

**Shared Placebo**

- Open Label Extension

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**Screening**

**Informed Consent**

**Assign to Regimen**

**Randomize within Regimen (active/placebo)**

**Up to 6 weeks**

**24 weeks (about 6 months)**

**Open Label Extension**
Enrollment Updates (as of May 20, 2021)

• 531 individuals with ALS signed informed consent
• 404 individuals were assigned to a regimen
• 358 individuals were randomized within a regimen (active or placebo)
• 94 have entered the Open Label Extension (OLE)

• 99 individuals were randomized within Regimen A
• 115 individuals were randomized within Regimen B
• 114 individuals were randomized within Regimen C
• 30 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

as of 5/20/21
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

- Expressing Interest in Participation
- The Pre-Screening Process
- Master Protocol Screening Visit and Informed Consent
- Assignment to Regimen and Screening
- Open Label Extension and Re-Assignment to Regimen
- Trial Data and Results
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**

- Feb 4  - Clene/CNM-Au8         (view recording- [https://bit.ly/3jB3WWt](https://bit.ly/3jB3WWt))
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 (Recorded May 14)

An Update on the CENTAUR Trial: a Trial of AMX0035 in ALS

Recording Available

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

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

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

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.

Register to attend and view recordings here:

www.neals.org
Patient Navigator

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To see whether you might qualify, view the list of eligibility criteria online: