HEALEY ALS Platform Trial

Weekly Q&A – June 17, 2021

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

Tackle ALS
ALS Association
MDA
Muscular Dystrophy Association
ALS ONE
FINDING A CURE
FINDING A CURE
ALS
FINDING A CURE
ALS ONE
MEDICAL MEETINGS
THE ARTHUR M. BLANK FAMILY FOUNDATION
AMG
The AMG Foundation
Guest Speaker

Lauren Elman, MD
University of Pennsylvania, Philadelphia
Platform Trial Site Investigator
Perpetual Adaptive Trial
Shared Placebo; Randomization Ratio 3:1
Open Label Extension (OLE) offered

Screening

Regimen A
(n=160 for each regimen)

Regimen B

Regimen C

Regimen D
(n=120 for active; n=40 for placebo)

3:1 Randomization within each Regimen

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Shared Placebo

Open Label Extension

Informed Consent
Assign to Regimen
Randomize within Regimen (active/placebo)
Open Label Extension

Up to 6 weeks
24 weeks (about 6 months)
Enrollment Updates (as of June 17, 2021)

- **591** individuals with ALS signed informed consent
- **469** individuals were assigned to a regimen
- **410** individuals were randomized within a regimen (active or placebo)
- **116** have entered the Open Label Extension (OLE)
- **111** individuals were randomized within Regimen A
- **130** individuals were randomized within Regimen B
- **130** individuals were randomized within Regimen C
- **39** individuals were randomized within Regimen D

Thank You

This breakthrough trial would not be possible without your partnership
Patient Navigator

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

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

Upcoming Guest Speakers:
July 1\textsuperscript{st} - Jeffrey Rosenfeld, MD (Loma Linda University, CA)
July 15\textsuperscript{th} - Shafeeq Ladha, MD (Barrow Neurological Institute, AZ)
July 22\textsuperscript{nd} - Richard Bedlack, MD, PhD, MS (Duke University, NC)
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**

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

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

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.

View recordings here:

www.neals.org