HEALEY ALS Platform Trial

Weekly Q&A – Oct 28, 2021

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

Megan Miller, PhD
Director of Science and Policy
I AM ALS
Welcome to the ALS Revolution
What we do

Drive patient-centered policy for ALS

Serve hundreds with best-in-class community support services

Support a grassroots movement over 200k strong
I AM ALS Research

Increase federal funding for ALS research
$83 million + ACT for ALS

Engage the whole research system

Remove silos and collaborate to accelerate progress

Fund research

Invest strategically for maximum impact
December 2020
I AM ALS awards a research grant to Dr. Sabrina Paganoni and MGH
to fund an expanded access research program for Verdiperstat

The Proposal:
● Expand access to 35 people living with ALS
● Safety, outcome, and biomarker data
● Training and advocacy materials

The Value to the ALS Advocate Community:
● Thinking creatively about how to both serve those living with ALS and expand research -- this is patient-centric!
● Support this model as the new gold standard in ALS clinical research
Research Team

Dr. Bedlack  Dr. Cudkowicz  Dr. Paganoni

the research team of Drs. Paganoni, Cudkowicz and Bedlack are sharing the learnings

I AM ALS Funds Expanded Access Program at MGH

https://www.youtube.com/watch?v=AEJ28VGCERk
Megan Miller
Director of Science and Policy
megan@iamals.org
EAP Companion Program to the HEALEY ALS Platform Trial
EAP Companion Program Objectives

To provide access to investigational product for people with ALS who do **not** qualify for the HEALEY ALS Platform Trial

To develop processes, training, advocacy and educational materials, and best practices to facilitate the adoption of EAP models beyond this program
HEALEY ALS Platform Trial
Enrollment Updates (as of October 28, 2021)

• 831 individuals with ALS signed informed consent
• 659 individuals were assigned to a regimen
• 595 individuals were randomized within a regimen (active or placebo)
• 251 have entered the Open Label Extension (OLE)
  • 158 individuals were randomized within Regimen A
  • 167 individuals were randomized within Regimen B
  • 161 individuals were randomized within Regimen C
  • 109 individuals were randomized within Regimen D
Patient Navigator

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

Eligibility criteria:  
Enrolling sites:
Finding Other Clinical Trial Opportunities

clinicaltrials.gov

CDC National ALS Registry

NEALS Website

ALS Signal from I AM ALS

ALS Clinical Trial Liaison contact info
(855) 437-4823
alstrials@neals.org

as of 10/28/21
Send us webinar ideas!

- Biostatistics
- Get to know our sites

**Upcoming Guest Speakers:**

Nov 4\textsuperscript{th} - Biostatistics with Lori Chibnik, PhD, MPH

Nov 11\textsuperscript{th} - No webinar (Veterans Day)

Nov 18\textsuperscript{th} - TBD

Nov 25\textsuperscript{th} - No webinar (Thanksgiving)