

HEALEY ALS Platform Trial

Weekly Q&A – Oct 28, 2021



Healey Center

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



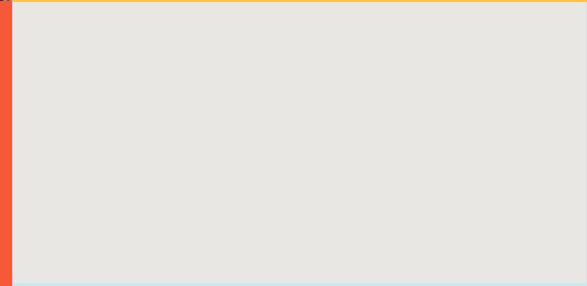
The AMG Foundation

Guest Speaker

Megan Miller, PhD
Director of Science and Policy
I AM ALS



Welcome to the ALS Revolution



I AM ALS

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

I AM ALS Funds Expanded Access Program at MGH

<https://www.youtube.com/watch?v=AEJ28VGCERk>



Megan Miller
Director of Science and
Policy

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IAM ALS

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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

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Eligibility criteria:



Enrolling sites:



Finding Other Clinical Trial Opportunities

clinicaltrials.gov



[CDC National ALS Registry](#)



[NEALS Website](#)



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

[ALS Signal
from I AM ALS](#)



Send us webinar ideas!

- **Biostatistics**
- **Get to know our sites**



Upcoming Guest Speakers:

Nov 4th - Biostatistics with Lori Chibnik, PhD, MPH

Nov 11th- No webinar (Veterans Day)

Nov 18th- TBD

Nov 25th- No webinar (Thanksgiving)