

Thank you for joining the webinar!

We are admitting audience members from the waiting room.

**Please allow a few moments for the webinar to begin.**



# HEALEY ALS Platform Trial

## Community Q&A – July 24, 2025



Calico



## Healey & AMG Center

Sean M. Healey & AMG Center for ALS  
at Massachusetts General Hospital



THE ARTHUR M. BLANK  
FAMILY FOUNDATION



The AMG Foundation

# Partnership, progress, and pathways forward



ANNIVERSARY

*Thank You*



## COMMON PROTOCOL

Regimen G

Regimen F

Regimen E

Regimen D

Regimen C

Regimen B

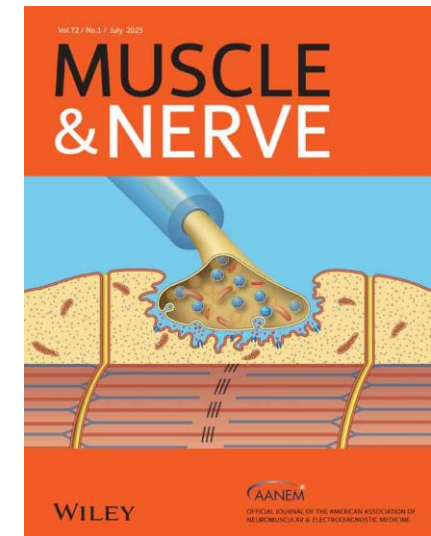
Regimen A

## Sharing our learnings

Operational Development and Launch of an Adaptive Platform Trial in Amyotrophic Lateral Sclerosis: **Processes and Learnings From the First Four Regimens of the HEALEY ALS Platform Trial**

First published: 26 May 2025

**Open Access**



<https://onlinelibrary.wiley.com/doi/10.1002/mus.28442>



# Partnership, progress, and pathways forward

## “Nothing about us without us”

### Acknowledgments

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TABLE 3 | Community and site engagement outcomes for the first four regimens.

Engagement strategy	Description of strategy	Engagement outcomes <sup>a</sup>
Introduction of patient navigator role	Patient Navigator served as a central resource to answer questions about the trial, develop educational resources, update website content, guide families through the trial enrollment process, and connect interested patients with local research centers. The Patient Navigator served as a member of the Recruitment and Retention Committee to seek patient feedback during monthly virtual meetings.	2,477 total emails/phone calls/zoom meetings with ALS families 39 Countries in contact 8 Webpages maintained 103 Trial-related videos uploaded to YouTube 4 Digital brochures created 1 Email response template created
Site support surveys	REDCap surveys were sent to all participating Platform Trial research centers to collect information about site-specific enrollment policies, with a particular focus on understanding variable COVID-19 restrictions across institutions. Survey responses were collected by the Patient Navigator to guide prospective research participants to appropriate local site options and provide individualized site support as needed.	3 Surveys conducted between November 2020–March 2021 41 Sites responded to each survey on average (79% of sites responded on average) 16 Sites reported that they could only enroll participants from within their own clinic 11 Sites reported that they could not enroll participants who lived out of state (due to COVID-19 restrictions)
Weekly Q&A webinars	Central leadership, in collaboration with the Patient Navigator, hosted a public webinar each week to provide trial updates and answer questions from the community. Special topic webinars, such as the regimen-specific drug science and mechanism of action webinars, were offered to address frequently asked questions. Recordings of each webinar were posted on YouTube and the Massachusetts General Hospital Platform Trial website.	102 Total webinars hosted 72 Weekly attendees on average 62 Guest speakers featured 1,871 Questions answered live 34,891 Total subsequent views on YouTube
Online eligibility checking tool (introduced May 2021)	An anonymous REDCap survey available publicly on the Massachusetts General Hospital Platform Trial website allowed people living with ALS to conduct an informal self-screen based on 5 key inclusion criteria for the trial. Survey outputs instructions for next steps based on someone’s potential eligibility.	580 Complete survey responses submitted 407 Respondents deemed potentially eligible 173 Respondents deemed potentially ineligible

<sup>a</sup>For the first 2 years of study conduct. Data collection for this table was completed on May 18, 2023, covering the period from September 29, 2020 to December 31, 2022. Webinar metrics for YouTube views are reported annually.

# Navigating Clinical Research Opportunities

- **ClinicalTrials.gov** – [clinicaltrials.gov/](https://clinicaltrials.gov/)
- **NEALS** – [neals.org/als-trials/neals-affiliated](https://neals.org/als-trials/neals-affiliated)
- **ALS TDI Trial Navigator** – [www.als.net/als-trial-navigator/](https://www.als.net/als-trial-navigator/)
- **MGH** – [www.massgeneral.org/neurology/als/research/als-clinical-trials](https://www.massgeneral.org/neurology/als/research/als-clinical-trials)
- **ALS Signal (I AM ALS)** – [www.iamals.org/get-help/als-signal-clinical-research-dashboard/](https://www.iamals.org/get-help/als-signal-clinical-research-dashboard/)
- **MDA** – [www.mda.org/research/clinical-trials](https://www.mda.org/research/clinical-trials)
- **ALSA** – [www.als.org/research/finding-als-research-opportunities](https://www.als.org/research/finding-als-research-opportunities)
- **International Alliance of ALS/MND Associations** – [www.als-mnd.org/support-for-pals-cals/research/understanding-als-mnd-research/](https://www.als-mnd.org/support-for-pals-cals/research/understanding-als-mnd-research/)



## **NEALS Clinical Trial Liaison**

Phone: (855) 437- 4823

Email: [alstrials@neals.org](mailto:alstrials@neals.org)

# ALS & Participation in Clinical Research

LES  
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ALS  
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Lateral Sclerosis  
Consortium



## ALS & PARTICIPATION IN CLINICAL RESEARCH

A Les Turner ALS Foundation Guide for People Living with ALS

Scan to view guide in English



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Northeast Amyotrophic  
Lateral Sclerosis  
Consortium



## ELA Y PARTICIPACIÓN EN INVESTIGACIONES CLÍNICAS

Una guía hecha por la Les Turner ALS Foundation para personas viviendo con ELA

Escanea para ver la guía en español

# “Your ALS Guide”

## Information about participating in various types of research

- ALS Clinical Trials
- Expanded Access
- Off Labels and Alternative Therapies
  - ALS Untangled: [www.alsuntangled.com/](http://www.alsuntangled.com/)
- Observational Studies
- Tissue Donation



[www.youralsguide.com/research-and-clinical-trials.html](http://www.youralsguide.com/research-and-clinical-trials.html)

# VA ALS Website

## *Veterans Health Administration*

- Where to go for care at VA (interactive maps)
- VA resources for ALS
- Educational videos for veterans with ALS and caregivers
- Research information



<https://www.va.gov/health/als.asp>





# Added Considerations

## What to expect in the enrollment process

- You don't always have to be a patient at the center where a study is being offered, but it's helpful to connect with a doctor who knows about ALS research
- Finding an ALS specialist or research center near you
  - NEALS member site finder: [neals.org/about/neals-member-sites](https://neals.org/about/neals-member-sites)
  - ALS Geospatial Hub Clinic Locator: [als-geospatial-hub-nonprofit.hub.arcgis.com/pages/als-clinic-advisor](https://als-geospatial-hub-nonprofit.hub.arcgis.com/pages/als-clinic-advisor)

## Note about international participation

- There are important logistics to consider if looking to participate in a US-based trial while living internationally
  - International Alliance of ALS/MND Associations: [www.als-mnd.org/support-for-pals-cals/research/understanding-als-mnd-research/](https://www.als-mnd.org/support-for-pals-cals/research/understanding-als-mnd-research/)
  - Clinical Trials in the European Union: [euclinicaltrials.eu](https://euclinicaltrials.eu)
  - Clinical Trials throughout Europe: [www.tricals.org/centres](https://www.tricals.org/centres)



Judi Carey



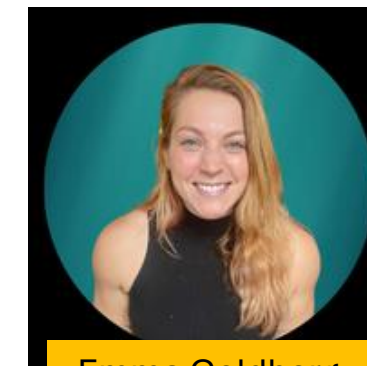
Catherine Small



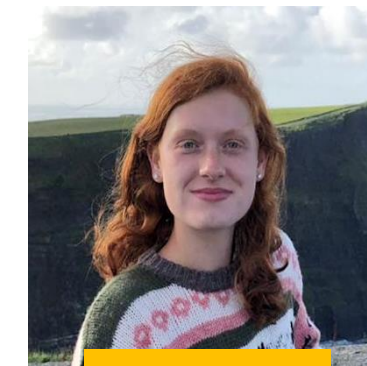
Michelle Redenz



Christina Smith



Emma Goldberg



Emma Allen

# Multi-Center Clinical Research Opportunities

## Investigational Medication Trials

- ☐ [HEALEY ALS Platform Trial Phase 2-3s](#)  
NCT 04297683
- ☐ [Healey ALS MyMatch Trial Program Phase 1-2s](#)  
ACACIA trial: NCT 07047209
- ☐ [Novartis VHB937 Phase 2-3](#)  
NCT 06643481
- ☐ [Amylyx AMX0114 Phase 1](#)  
NCT 06665165

## Biomarker & Observational Studies

- ☐ [ASSESS ALL ALS](#)  
NCT 06578195
- ☐ [PREVENT ALL ALS](#)  
NCT 06581861
- ☐ [Target ALS](#)  
NCT 05137665

MGH website:



# Educational Webinar Opportunities

- **NEALS** – [neals.org/people-living-with-als/educational-webinars](https://neals.org/people-living-with-als/educational-webinars)
- **Les Turner ALS Foundation** – [lesturnerals.org/support-services/als-learning-series/](https://lesturnerals.org/support-services/als-learning-series/)
- **Everything ALS** – [www.everythingals.org/events](https://www.everythingals.org/events)
- **ALL ALS** – [www.all-als.org/webinars/](https://www.all-als.org/webinars/)

Missed a webinar?  
View recordings  
online!





# Patient Navigation

## Central resource for people living with ALS



Catherine Small

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E-mail: [healeyalsplatform@mgh.harvard.edu](mailto:healeyalsplatform@mgh.harvard.edu)

Two webinars  
per month

Sign up for  
ALS Link:



<https://bit.ly/3CqGbhb>

Register for  
webinars:



<https://bit.ly/3r6Nd2L>

### Upcoming Webinars (Thurs, 5:00- 5:30pm EST):

**August 14** – Ibudilast EAP Update with Björn Oskarsson, MD (Mayo Clinic Florida)

**August 28** – HEALEY ALS Platform Trial Biomarker Learnings with James Berry, MD, MPH and Eric Macklin, PhD (Mass General Hospital)

august  
2025

MON	TUE	WED	THU	FRI	SAT	SUN
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