

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 – June 27, 2024



## Healey & AMG Center

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



Calico



The AMG Foundation

# Common protocol and shared infrastructure allow for operational and scientific efficiencies



**COMMON  
PROTOCOL**

**Regimen G**

**Regimen F**

**Regimen E**

**Regimen D**

**Regimen C**

**Regimen B**

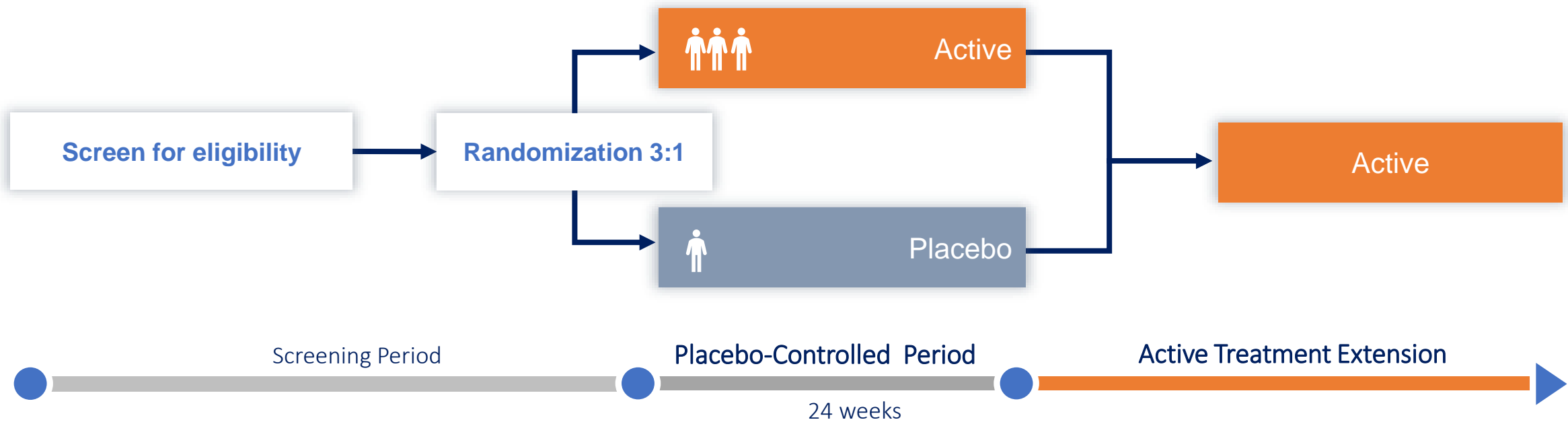
**Regimen A**

**1 Protocol  
(Phase 2/3)  
1 single IRB  
Central Governance**

**7 Regimens  
70+ Enrolling Sites  
~1300 Participants**

Regimen: Active Study Drug + Matching Placebo

The objective of each regimen is to provide a go / no go decision to inform the clinical development program of the regimen study drug



### **Primary Endpoint (Placebo-Controlled Period)**

Change from baseline through week 24 in disease severity as measured by the ALSFRS-R total score and survival

### **Safety, Secondary, and Exploratory Endpoints**

(respiratory function, muscle strength, survival, biomarkers + regimen-specific endpoints)

# Navigating Clinical Trial Opportunities



- **ClinicalTrials.gov** – [clinicaltrials.gov/](https://clinicaltrials.gov/)

- **NEW ALS TDI Trial Navigator** – [www.als.net/als-trial-navigator/](http://www.als.net/als-trial-navigator/)

- **ALS Signal (I AM ALS)** – [www.iamals.org/get-help/als-signal-clinical-research-dashboard/](http://www.iamals.org/get-help/als-signal-clinical-research-dashboard/)

- **NEALS** – [neals.org/als-trials/neals-affiliated](http://neals.org/als-trials/neals-affiliated)

- **MDA** – [www.mda.org/research/clinical-trials](http://www.mda.org/research/clinical-trials)

- **ALSA** – [www.als.org/research/finding-als-research-opportunities](http://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/](http://www.als-mnd.org/support-for-pals-cals/research/understanding-als-mnd-research/)

# ALS & Participation in Clinical Research



## ALS & PARTICIPATION IN CLINICAL RESEARCH

A Les Turner ALS Foundation Guide for People Living with ALS



Scan to view guide

<https://bit.ly/4eBi2mF>



## ELA Y PARTICIPACIÓN EN INVESTIGACIONES CLÍNICAS

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



Escanear para ver la guía

<https://bit.ly/3xvKJ3T>





# What's the Latest on ALS Research and Clinical Trials?

Read Article Here



<https://bit.ly/4dQLPaK>



## HEALEY ALS Platform Trial

To accelerate the discovery of new treatments for ALS, Mass General Brigham launched the [HEALEY ALS Platform Trial](#) in 2020. Led by chief of Neurology at Massachusetts General Hospital [Merit Cudkowicz, MD, MSc](#), and Dr. Paganoni, the trial is coordinated by the Healey and AMG Center and designed in partnership with the Northeast ALS (NEALS) Consortium. Patients can participate at more than 70 sites throughout the United States, including leading academic medical centers and community practices.

So far, more than 1,300 people have participated in the platform trial and seven drugs were evaluated. This collaborative effort with research partners from the industry, non-profit foundations, and patient groups has already helped move two promising drugs to the next phase of testing.



**NEALS Consortium**  
@NEALSConsortium

Registration for our annual meeting has opened! People with lived ALS experience are welcome to join us virtually this year and be a part of this incredible event. Register here [meeting.neals.org](https://meeting.neals.org) and select “Person with Lived ALS Experience (Virtual)”



[meeting.neals.org](https://meeting.neals.org)  
Home | NEALS Annual Meeting

3:37 PM · May 21, 2024 · **547** Views

# 2024 ANNUAL NEALS MEETING

OCTOBER 21 - 24, 2024

**HYBRID MEETING**  
(Clearwater, FL and Online)



<https://meeting.neals.org/>

## ABOUT THE MEETING

The Annual NEALS Meeting brings together researchers, clinicians, industry leaders, and individuals with lived ALS experience to discuss the latest advancements in ALS research and treatment.

With a focus on member initiatives and potential new therapies, the meeting prepares attendees for upcoming clinical trials and fosters collaboration among the ALS community. **Join us as we continue to embrace inclusivity, facilitate knowledge exchange, and collectively shape the future of ALS research.**



# ALS CLINICAL RESEARCH LEARNING INSTITUTE®

## NOW ACCEPTING APPLICATIONS ●

### Become an ALS Research Ambassador

October 19 - 20, 2024  
Clearwater Beach, FL

#### ABOUT ALS CRLI

The ALS Clinical Research Learning Institute® (CRLI) is a two-day program designed to provide education and training to individuals impacted by ALS who are interested in clinical research and the therapy development process. Individuals who complete the training program are considered ALS Research Ambassadors and are eligible to participate in opportunities that influence and improve the ALS research process.

#### WHO SHOULD APPLY

People living with ALS, caregivers, and family members are encouraged to apply.

**APPLICATION DEADLINE:**  
**JULY 15, 2024**

For more information and to apply: [neals.org/for-people-with-als-caregivers/clinical-research-learning-institute-crli/](https://neals.org/for-people-with-als-caregivers/clinical-research-learning-institute-crli/)



**Apply Here:**

<https://www.surveymonkey.com/r/2024CRLI>







[2024 SYMPOSIUM](#) [2023 SYMPOSIUM](#) [2022 SYMPOSIUM](#) [ALS ONE](#)

[REGISTER FOR FREE HERE](#)



# 7TH ANNUAL ALS RESEARCH SYMPOSIUM

November 13, 14 & 15th, 2024

*Together from afar!*

<https://www.alsoneresearchsymposium.org/>

Uniting researchers, clinicians and individuals living with ALS from around the world for three days of sharing important ALS research and hope.



# Getting Ahead of the ALS Curve Through Early Detection and Intervention



## Module 1 - Enabling Earlier Detection of ALS

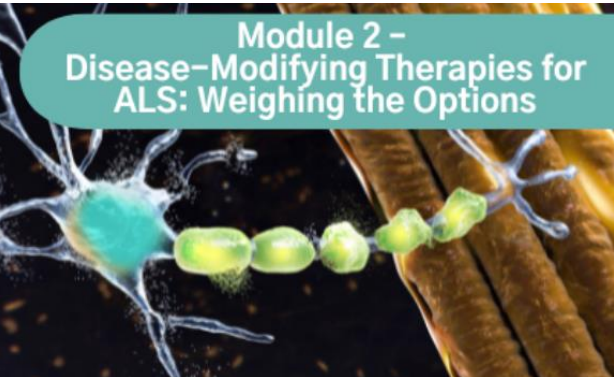


### ESTIMATED TIME TO COMPLETE

30 minutes

<https://bit.ly/4cCdaMj>

## Module 2 - Disease-Modifying Therapies for ALS: Weighing the Options



### ESTIMATED TIME TO COMPLETE

15 minutes

<https://bit.ly/3Xzs9m2>

### TARGET AUDIENCE

Primary care physicians (PCPs), neurologists, nurse practitioners, physician assistants, nurses, pharmacists, and other health care professionals involved in the treatment of patients with amyotrophic lateral sclerosis (ALS).

### LEARNING OBJECTIVES

Upon completion of this activity, participants should be able to

- Evaluate factors that can inform an early and accurate diagnosis of ALS

### REGISTRATION

There is no fee to participate in or claim CME/NCPD/CPE credit for this activity.

### DATES AVAILABLE

May 31, 2024 – May 30, 2025





## NEALS Educational Webinars

Visit the **NEALS website** to explore upcoming webinars or view previous recordings



<https://bit.ly/3S4P0Sb>



## Access for ALL in ALS (ALL ALS) Consortium

Hosted May 16, 2024 / 11:00 am - 12:00 pm ET

### Access for ALL with ALS Consortium

Funding started Oct 2023 by NINDS using ACT for ALS funds



#### Goals:

- Create a large, flexible ALS Research Consortium platform that can grow and be modified
- Provide opportunities for all individuals living with ALS in the United States to participate
- Run longitudinal natural history and biomarker studies
- Build a large openly shared data knowledge portal and biobank
- Provide clinical data and biosamples to better characterize ALS, identify biomarkers, and aid drug development
- Expand our goals to contribute ever more!

18 Apr 2024

7



James Berry



# Patient Navigation

## Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

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

Weekly webinar  
registration:



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

ALS Link sign-up:



<https://bit.ly/3o2Ds3m>

### **Summer Series Upcoming Webinars:**

**July 11<sup>th</sup>**- Monthly EAP Webinar featuring Patient Advisory Committee

**July 25<sup>th</sup>**- Monthly Platform Trial Update

**August 8<sup>th</sup>**- Monthly EAP Webinar

**August 22<sup>nd</sup>**- Monthly Platform Trial Update



Allison Bulat