

Thank you for joining the weekly webinar!

We are admitting audience members from the waiting room.

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



# HEALEY ALS Platform Trial

Weekly Q&A – May 16, 2024



## Healey & AMG Center

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



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**

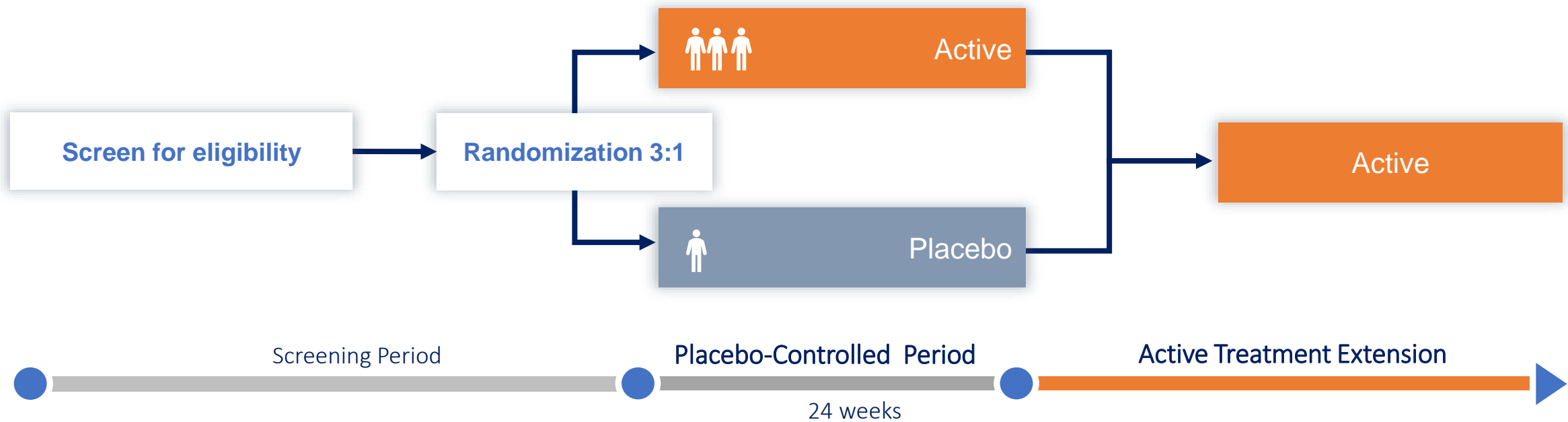
**Regimen A**

**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)

# Enrollment Update: Regimen F and Regimen G

NEWS · 5 MINUTE READ · APR | 11 | 2024

The HEALEY ALS Platform Trial completes enrollment for testing investigational product ABBV-CLS-7262 from Calico Life Sciences

NEWS · 5 MINUTE READ · MAY | 1 | 2024

The HEALEY ALS Platform Trial completes enrollment for Regimen G evaluating investigational product DNL343 from Denali Therapeutics



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

*Thank You*  
for your partnership in ALS research



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

# 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/)



# NEALS Educational Webinars

## View Recording



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

## An Overview of The ALS Better Care Act

Hosted May 8, 2024 / 4:00 pm–5:00 pm



## ALS Better Care Act

- Establishes a single, flat \$800 payment per visit to providers caring for patients with ALS on Medicare beginning January 1, 2025.
- Providing additional payment to ALS-related service providers will:
  - Improve access to care for patients
  - Extend survival
  - Catalyze clinical research
  - May offer financial savings due to improved outcomes (lower preventable costs)

**SUPPORT THE ALS BETTER CARE ACT**  
(HR 5663/S.3258)


The Act will give people living with ALS desperately needed access to multidisciplinary care and clinical trials. People with ALS who receive multidisciplinary care live longer and have a higher quality of life.

**AMYOTROPHIC LATERAL SCLEROSIS (ALS)** (aka Lou Gehrig's disease) is a neurodegenerative condition affecting nerve cells that control voluntary muscular movement.

**PEOPLE LIVING WITH ALS (PLWALS)** decline rapidly and require specialized care from multidisciplinary teams to address medical needs.

Medicare reimbursement does not adequately cover timely and effective care, hampering the ability of providers to research innovations to improve survival and quality of life, and reduce complications.

Adequately funded clinics could **lower preventable costs** and **long wait times**, **extend survival**, and simultaneously catalyze cutting-edge clinical research to **accelerate the path to a cure**.





## NEALS Educational Webinars

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



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



# NEALS

Northeast Amyotrophic Lateral Sclerosis Consortium®

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

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

National Institutes of Health (NIH) funded “ALL ALS” consortium is a multi-institutional effort that aims to organize the ALS clinical research landscape in the US. ALL ALS is operating in partnership with several stakeholders...

June 12, 2024 / 12:00 pm-1:00 pm

### Promising Alternative and Off-Label Treatments for ALS

In this educational webinar, Dr. Richard Bedlack of ALSUntangled will review what he considers to be the most promising alternative and off-label ALS treatments currently. These products, which include vitamins, supplements, and...





# EAP Webpages and Resources



Link to EAP Website:



<https://bit.ly/3uni3lc>

Navigation: About the Healey & AMG Center for ALS | Clinical Care & Resources | Research Opportunities | Platform Trial | Expanded Access | News & Events

Home - Neurology - ALS - Research

## Expanding Access to Investigational Products

EAP Opportunities > EAP News & Webinars > Information for Patients >

Overview > EAP Opportunities > For Patients > News & Webinars >

**Overview**

At the Sean M. Healey & AMG Center for ALS, we are dedicated to developing effective treatments for ALS and to providing people with ALS access to experimental therapies through Expanded Access Protocol (EAP) programs.

EAPs are a pathway for people with a life-threatening condition or serious disease to gain access to an investigational medical product when they are not eligible for a clinical trial. These investigational products – drugs, biologics, or medical devices – are currently being studied, but not yet approved by the US Food and Drug Administration (FDA). Alongside traditional data, EAPs can also provide data that may be useful in developing new therapies.

We have built a dedicated team at the Healey & AMG Center to rapidly implement EAPs for people with ALS at Mass General and are working with several other research centers across the US.

**Supporting Expanded Access**

Expanded Access Protocol programs at the Sean M. Healey and AMG Center for ALS are funded by multiple sources, including contributions from donors like you. Thank you for partnering with us in the quest to discover life-saving therapies for individuals affected by ALS.

Give Now > Community Support >

**EAP Opportunities**  
Current and upcoming expanded access protocols  
Learn more >

**EAP News & Webinars**  
Get the latest news and updates about expanded access protocols  
Learn more >

**Information for Patients**  
Common questions and helpful resources about expanded access  
Learn more >

# First Participants Enrolled in the NIH-Funded Pridopidine and Rapa EAPs!



<https://www.massgeneral.org/neurology/als/research/expanded-access-news>

## Press Releases

- **May 2024:** [The Sean M. Healey & AMG Center for ALS and Rapa Therapeutics announce first participant enrolled in Expanded Access to RAPA-501 Protocol \(EAP\)](#)
- **April 2024:** [The Sean M. Healey & AMG Center for ALS and Prilenia Therapeutics Announce First Participant Enrolled in NIH-Sponsored ACT for ALS Expanded Access Protocol for Pridopidine](#)

NEWS · 5 MINUTE READ · MAY | 2 | 2024

The Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital and Rapa Therapeutics have announced the first participant enrolled in the Expanded Access to RAPA-501 Protocol (EAP)

NEWS · 5 MINUTE READ · APR | 29 | 2024

The Sean M. Healey & AMG Center for ALS and Prilenia Therapeutics Announce First Participant Enrolled in NIH-Sponsored ACT for ALS Expanded Access Protocol for Pridopidine

# Patient Navigation

## Central resource for people living with ALS



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Weekly webinar  
registration:



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

ALS Link sign-up:



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

### Upcoming Webinars:

**May 23<sup>rd</sup>**- Weekly Q&A webinar

**May 30<sup>th</sup>**- Weekly Q&A webinar

**June 6<sup>th</sup>**- Canceled



Allison Bulat