

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 2, 2024



## Healey & AMG Center

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



als FINDING a CURE™



Calico



THE ARTHUR M. BLANK  
FAMILY FOUNDATION



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**

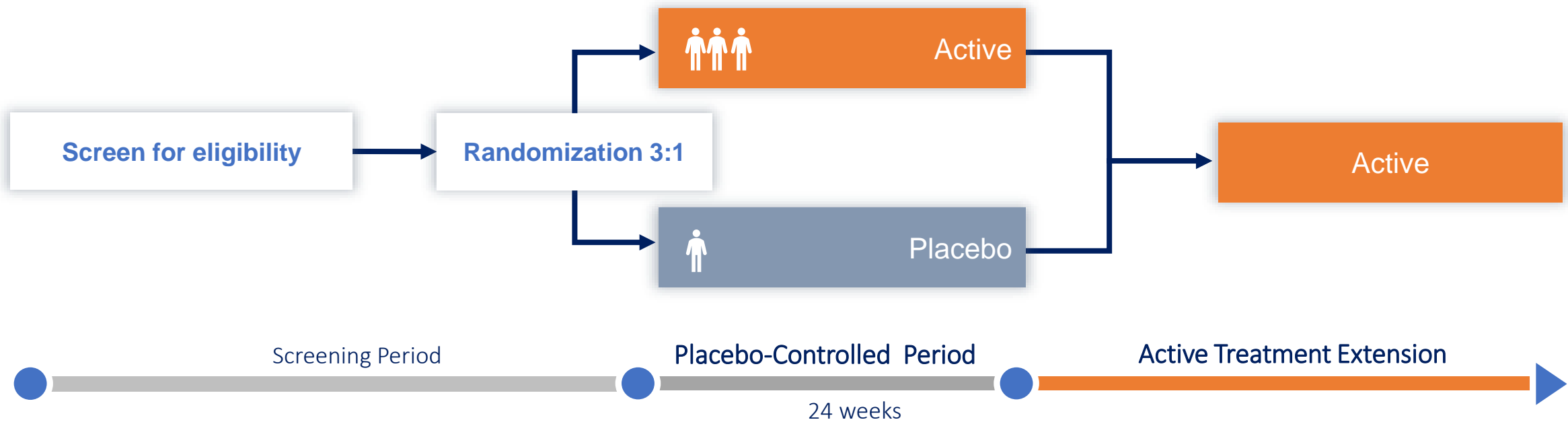
**1 Protocol  
(Phase 2/3)  
1 single IRB  
Central Governance**

**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

(as of 5/2/24)

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

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



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



May 8, 2024 / 4:00 pm–5:00 pm

### An Overview of The ALS Better Care Act

Join Dr. Terry Heiman-Patterson, Dr. Kelly Gwathmey and Dr. Sabrina Paganoni, and as they discuss the importance of multidisciplinary care and the role of the ALS Better Care Act to ensure that quality care at Multidisciplinary Centers...

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...



# 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!

[Home](#) - [Neurology](#) - [ALS](#) - [News](#)

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

Juliana Araujo · [jaraujo5@mgh.harvard.edu](mailto:jaraujo5@mgh.harvard.edu)



[Home](#) - [Neurology](#) - [ALS](#) - [News](#)

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)

Juliana Araujo · [jaraujo5@mgh.harvard.edu](mailto:jaraujo5@mgh.harvard.edu)

# 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>

### Upcoming Webinars:

**May 9<sup>th</sup>**- EAP Discussion with Shawn Sarbacker from Tech vs ALS

**May 16<sup>th</sup>**- Weekly Q&A webinar

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



Allison Bulat