

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 23, 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

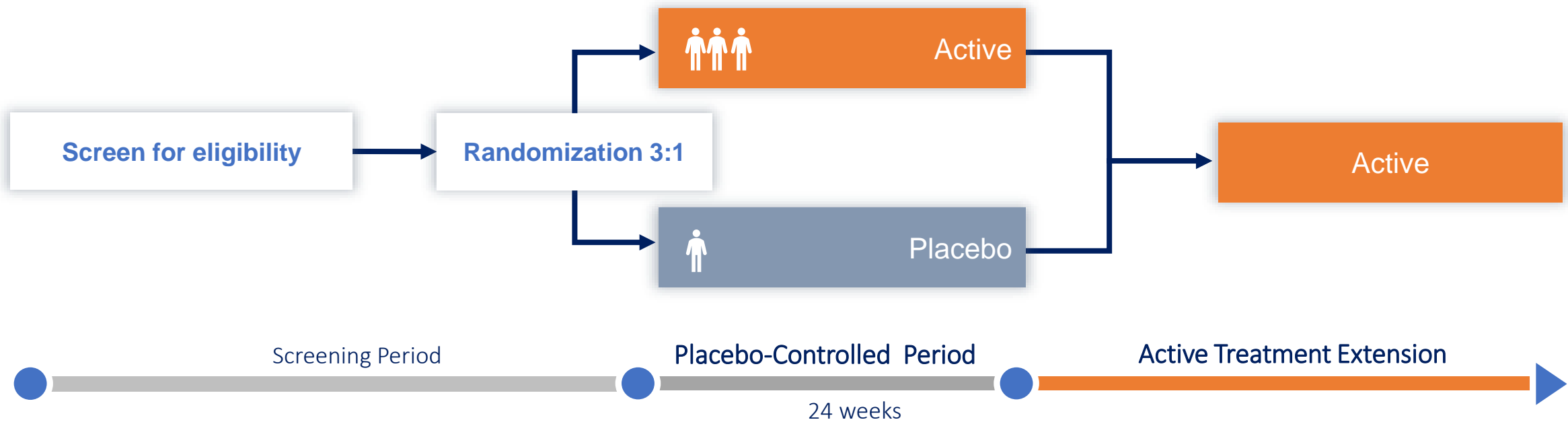
Regimen A

**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)

Biomarker Brochure!



Healey & AMG Center

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



Massachusetts General Hospital

Founding Member, Mass General Brigham

HEALEY ALS Platform Trial Innovation through Biomarkers

Learn how your participation makes a difference

JULY 2023

What is a Biomarker?

A biomarker is an indicator of what is happening inside of your body. Biomarkers include everything from vital signs to laboratory tests of blood and other tissues.

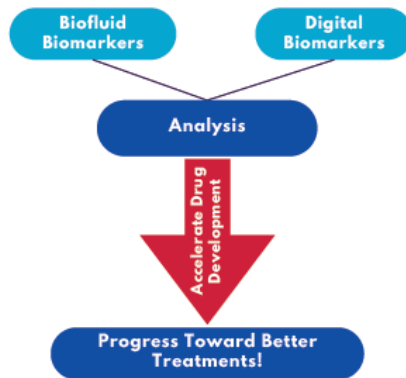
A biomarker may be used to: (1) learn more about a disease, (2) follow disease progression, (3) monitor how a disease responds to a study drug, or (4) serve as a target for treatment.

How do your biomarkers contribute to ALS research?

In the HEALEY ALS Platform Trial, biomarkers are studied in your blood, urine, and cerebrospinal fluid (CSF), or assessed via digital technology.

Neurofilaments (NF) are an example of a biomarker found in blood and CSF that has shown great potential in monitoring disease progression and drug effectiveness. NF levels are higher in the blood and CSF of people with ALS. This can be attributed to the breaking down of motor neurons, which spill contents such as neurofilaments into the spinal fluid.

By analyzing your biofluids, the research team is able to gather information on changes in important biomarkers, including NF levels. These analyses enable the team to have a better-informed view of how the study drug works, which may influence the course of drug development and result in a long-lasting impact on ALS research.



Blood Samples

Blood carries nutrients, drugs, and wastes throughout the body. Blood samples allow researchers to measure how the study drug is broken down and used in your body.

In the HEALEY ALS Platform Trial, monitoring changes in various protein, hormone, and NF levels over time may help determine the effectiveness of an investigational drug.

Urine Samples

Urine samples enable researchers to collect valuable biomarkers as they are flushed out of your system. For example, p75 is a protein which increases in concentration with ALS progression. Tracking this protein may help determine drug efficacy and disease progression in certain trials.

Cerebrospinal Fluid (CSF) Samples

CSF, the fluid that surrounds the brain and spinal cord, is in direct contact with motor neurons and the central nervous system. Given its location, we can measure breakthrough biomarkers that are not available to be measured in the blood. CSF samples are collected through lumbar punctures (LP). Scan the QR codes below to learn more about lumbar punctures.

Lumbar Puncture Video



<https://www.youtube.com/watch?v=3sm10Ea18s0>

Lumbar Puncture Brochure



<https://www.massgeneral.org/assets/mgh/pdf/neurology/als/platformtrial/lumbarpuncture.pdf>

At-Home Digital Biomarkers

Recent innovation in digital technology has allowed for the collection of biomarkers from the comfort of your home. In the HEALEY ALS Platform Trial, some regimens may use various digital biomarkers. Examples may include:

Speech Analysis

Voice recordings are an example of digital biomarkers that may be collected in the HEALEY ALS Platform Trial. Speech recordings enable researchers to monitor variables such as pace and clarity of speech, and track whether any changes occur as a result of disease progression. Recordings of your voice may be collected during in-person study visits and at home via a smartphone app.

Spirometry

Spirometry is a lung function test that measures the quality and strength of your breathing. This test may be taken from home.



Speech Analysis Application



Spirometer

Stay Connected to ALS Research

Join the MGH ALS Link to Stay Connected to Research:



<https://lp.constantcontactpages.com/su/saTzwlp/ALSLink>

For more information:

Contact Patient Navigator:
HEALEYALSPlatform@
mgh.harvard.edu or
833-425-8257
(HALT ALS)

Register for HEALEY ALS Platform Trial Q&A Webinars:



<https://www.massgeneral.org/neurology/als/research/platform-trial>

Visit webpage to download: <https://www.massgeneral.org/neurology/als/research/first-platform-trial-treatments>

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/

- **NEW ALS TDI Trial Navigator** – www.als.net/als-trial-navigator/

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

- **NEALS** – neals.org/als-trials/neals-affiliated

- **MDA** – www.mda.org/research/clinical-trials

- **ALSA** – 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/

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 and select “Person with Lived ALS Experience (Virtual)”



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.**



NEALS Educational Webinars

View Recording



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

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



James Berry



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



NEALS

Northeast Amyotrophic Lateral Sclerosis Consortium®



NEALS Educational Webinars

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



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

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...



NEALS

Northeast Amyotrophic
Lateral Sclerosis
Consortium®



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



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu

Weekly webinar
registration:



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

ALS Link sign-up:



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

Upcoming Webinars:

May 30th- Weekly Q&A webinar featuring **Biomarker Talk with Dr. Bob Bowser**
(Barrow Neurological Institute, AZ)

June 6th- Canceled



Allison Bulat