

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 – October 12, 2023



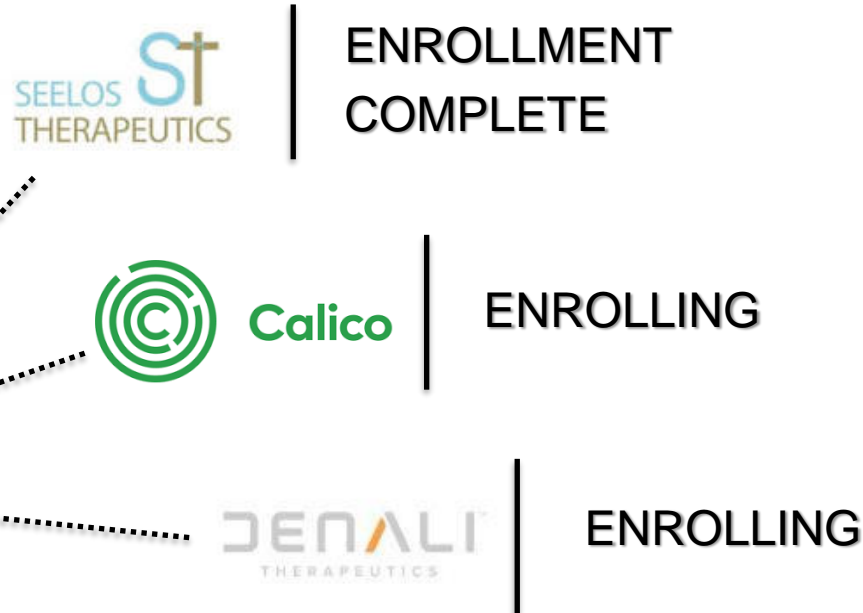
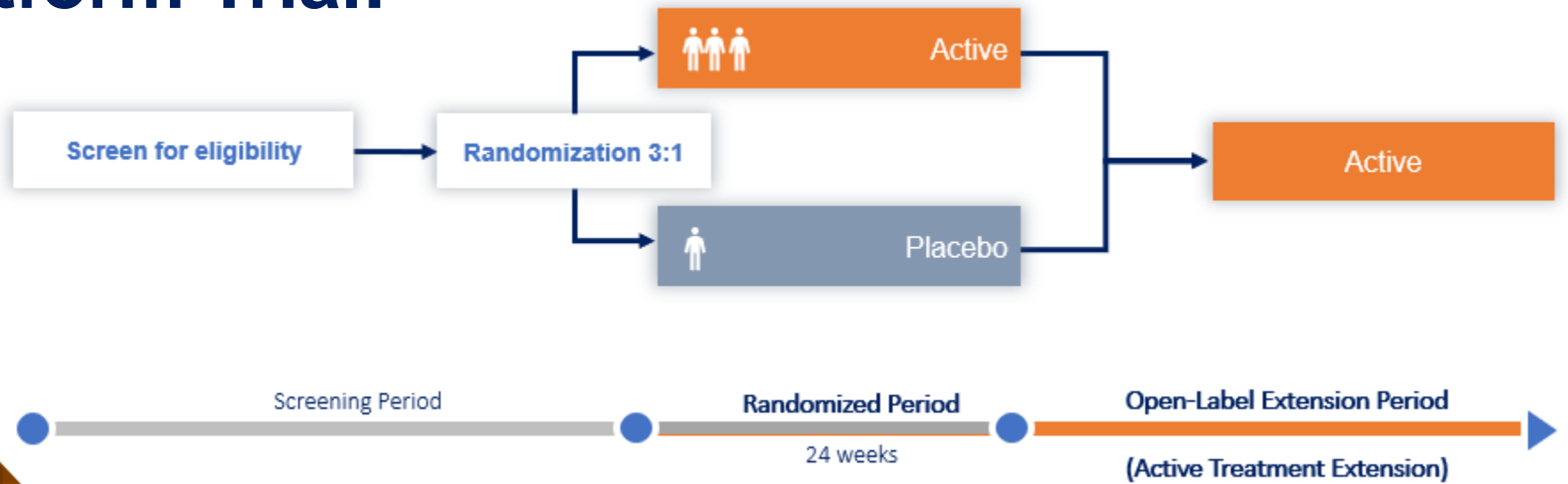
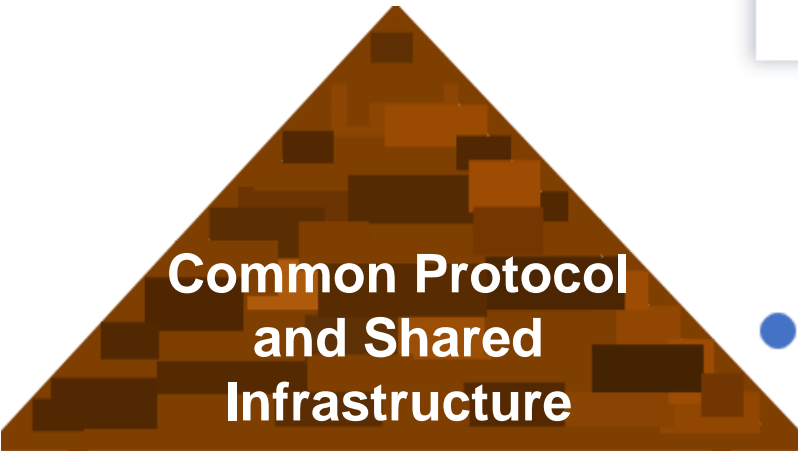
## Healey & AMG Center

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



The AMG Foundation

# HEALEY ALS Platform Trial:



# Enrollment Update: Regimen F and Regimen G

382

Participants consented to Master Protocol since RGF and RGG initiated



318

Participants assigned to RGF or RGG



272

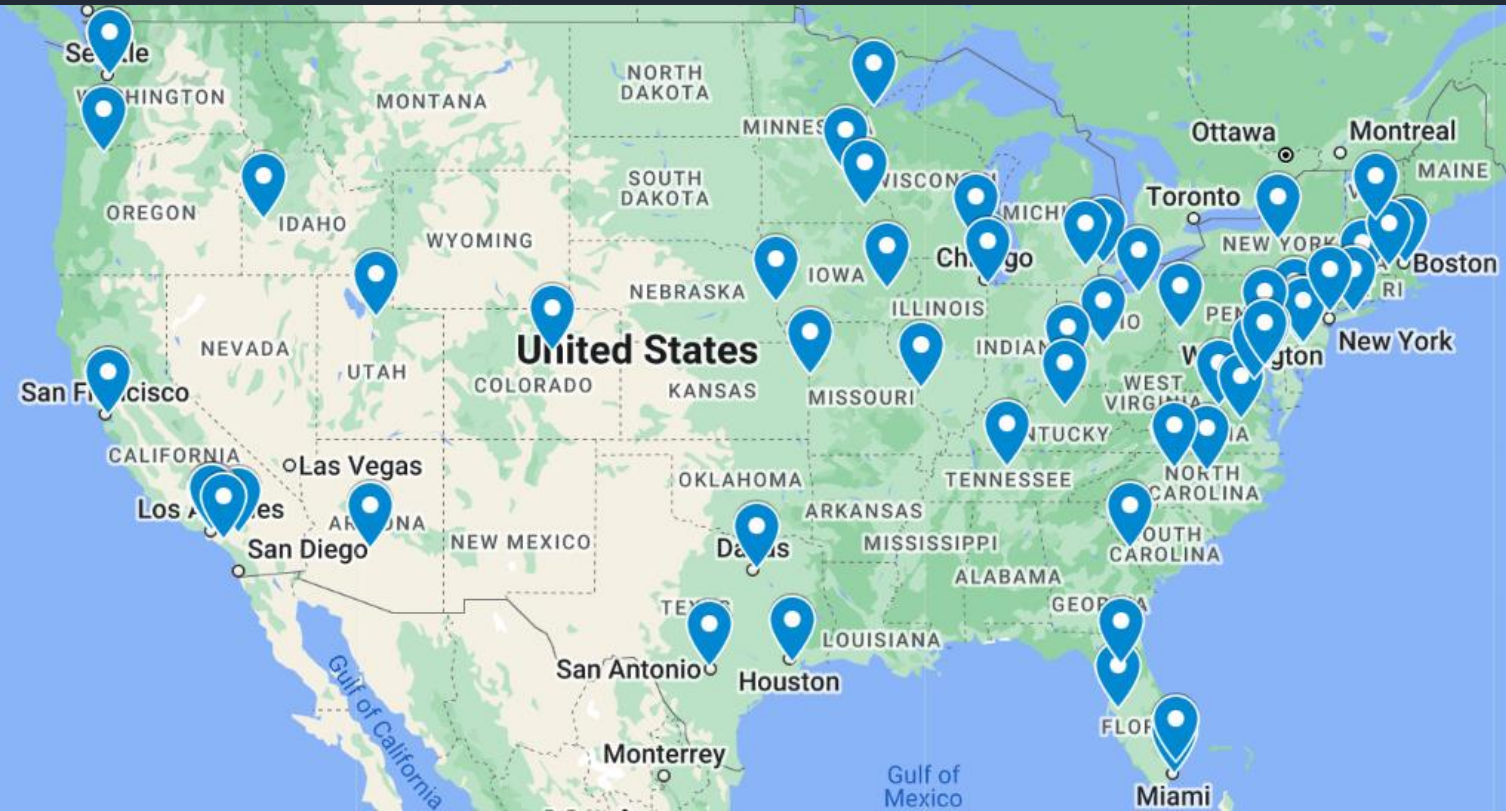
Participants randomized within RGF or RGG

*Thank You*

for your partnership in ALS research

(as of 10/12/23)

# 63 Sites Currently Active



(as of 10/12/23)

- ✓ Nova Southeastern University
- ✓ Essentia Health
- ✓ Texas Neurology
- ✓ Mass General Hospital
- ✓ University of Nebraska
- ✓ Hospital for Special Care
- ✓ Henry Ford Hospital
- ✓ Augusta University
- ✓ Beth Israel Deaconess
- ✓ University of Texas HSC
- ✓ University of Colorado
- ✓ Loma Linda University
- ✓ Ohio State University
- ✓ Cedars Sinai Medical Center
- ✓ Duke University
- ✓ Wake Forest University
- ✓ Saint Alphonsus
- ✓ UMass Worcester
- ✓ Lehigh Valley
- ✓ Thomas Jefferson
- ✓ University of South Florida
- ✓ University of Pennsylvania
- ✓ SUNY Upstate
- ✓ University of Iowa
- ✓ California Pacific Med Center
- ✓ Houston Methodist
- ✓ Vanderbilt University
- ✓ University of Minnesota
- ✓ Washington University
- ✓ Barrow Neurological Institute
- ✓ University of Miami
- ✓ Temple University
- ✓ University of Virginia
- ✓ Johns Hopkins University
- ✓ University of Southern CA
- ✓ Holy Cross Hospital
- ✓ University of Washington
- ✓ University of Utah
- ✓ Penn State Hershey
- ✓ University of Michigan
- ✓ University of Kansas
- ✓ Stony Brook University
- ✓ University of Cincinnati
- ✓ Mayo Clinic Rochester
- ✓ Northwestern University
- ✓ Georgetown University
- ✓ Kaiser, Los Angeles
- ✓ University of Pittsburgh
- ✓ Virginia Commonwealth
- ✓ Med College Wisconsin
- ✓ University of CA, San Fran
- ✓ University of Florida, Gainesville
- ✓ Providence Brain and Spine
- ✓ Cleveland Clinic
- ✓ George Washington University
- ✓ Hackensack University
- ✓ Swedish Medical Center
- ✓ University of Kentucky
- ✓ Dartmouth Hitchcock
- ✓ University of Maryland
- ✓ University of Chicago
- ✓ University of CA, Irvine
- ✓ Columbia University

# Checking Site Status Online

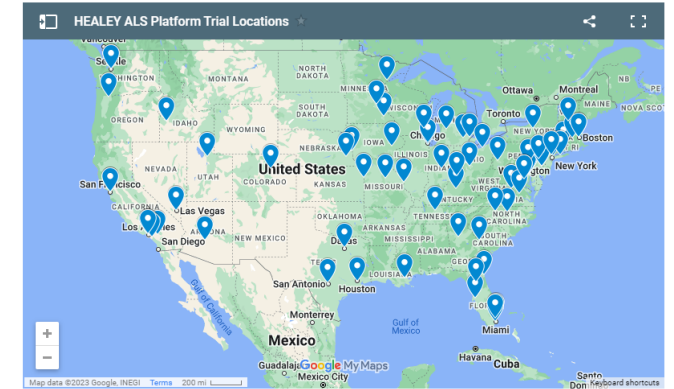
Contact a study team near you to discuss enrollment opportunities

## List of Participating Sites

Sites marked "Recruiting" are currently enrolling participants. Sites marked "Active, Not recruiting" are active in the Platform Trial (for example, they are following participants in ongoing regimens that have already completed enrollment) but are not enrolling new participants at this time.


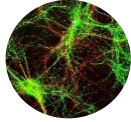



Site	State	Enrollment Status	Trial Contact Information
Barrow Neurological Institute	AZ	Recruiting	<a href="#">Whitney Dailey</a>
Cedars-Sinai Medical Center	CA	Recruiting	<a href="#">Sophia Mostowy</a>
Forbes Norris MDA/ALS Research Center, California Pacific Medical Center	CA	Recruiting	<a href="#">Teji Dulai</a>
Kaiser Permanente, Los Angeles Medical Center	CA	Recruiting	<a href="#">Mary H. Berganza</a>

Map of Participating Sites



<https://bit.ly/3g2NZr5>

# Assessing emerging biomarkers/outcome measures

-  **DNA** – genetic analysis for all regimens
-  **Neurofilaments** – NfL analysis for all regimens
-  **Biomarkers** (Blood, Urine, CSF) – several drug-specific biomarkers in each regimen
-  **Speech Analysis** – data collected for regimens A-D
-  **Home Spirometry** – critical during the pandemic for regimens A-D

**Additional biomarkers/outcome measures being considered for upcoming regimens (PBMCs, ROADS)**

# 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.

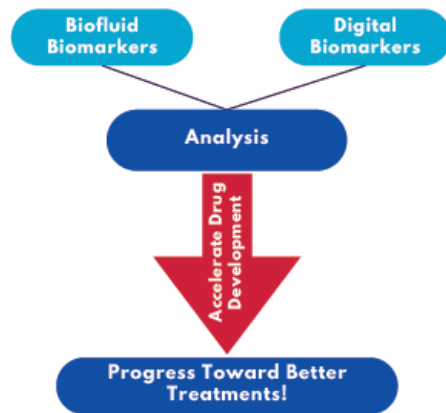
A biomarker may be used to: (1) learn more about a disease, (2) follow disease progression, (3) monitor how a disease responds to a study drug, or (4) serve as a target for treatment.

## 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=3omlQEnll8g>

### Lumbar Puncture Brochure



[https://www.massgeneral.org/assets/mgh/pdf/neurology/als/platformtrial\\_lumbarpuncture.pdf](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>



# Study Drugs Webpage

## This public webpage provides:

1. General Brochure and Biomarkers Brochure
2. Overview of science behind Regimen F and Regimen G
3. Links to download relevant brochures, including the Lumbar Puncture Brochure
4. Links to view relevant videos and webinar recordings

### Regimen F: ABBV-CLS-7262, by Calico and AbbVie- Now Recruiting

[Watch a webinar about the science behind ABBV-CLS-7262](#)

[Watch this video](#) for more information on the mechanism of action behind ABBV-CLS-7262.

[Download Regimen F Brochure](#)

[Download Lumbar Puncture Brochure](#)

Step by Step Guide to Lumbar Punctures: [Watch short video](#)

### Regimen G: DNL343, by Denali Therapeutics

[Download Regimen G Brochure](#)

[Watch a webinar about the science behind DNL343](#)

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



**Healey & AMG Center**

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



# 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:

**October 19th-** weekly Q&A hosted by Merit Cudkowicz, MD MSc

**October 26th-** weekly Q&A

**November 2nd-** weekly Q&A and Regimen G science (tentative date)



Allison Bulat