

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 – September 7, 2023



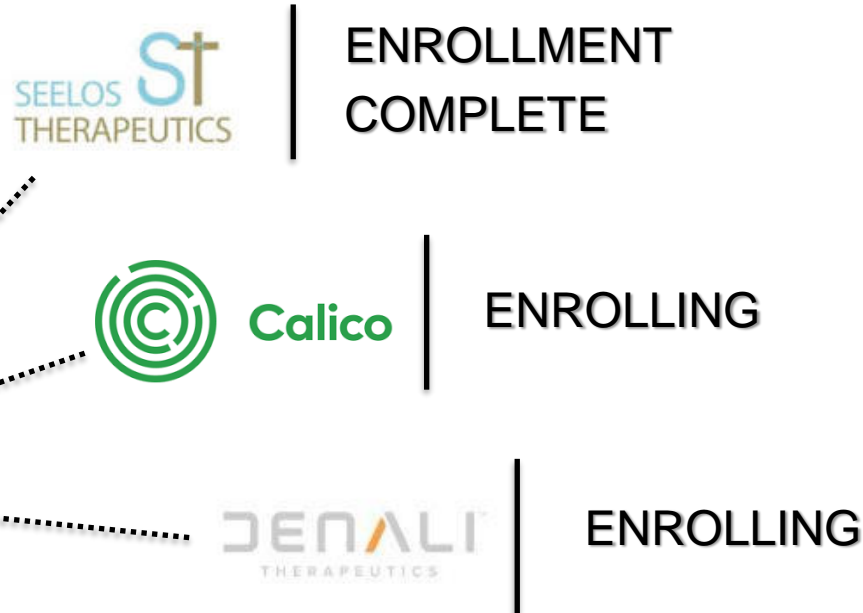
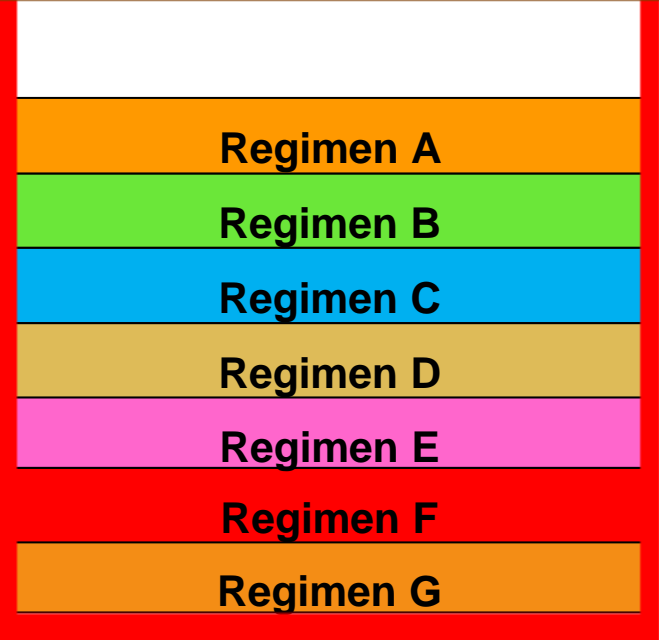
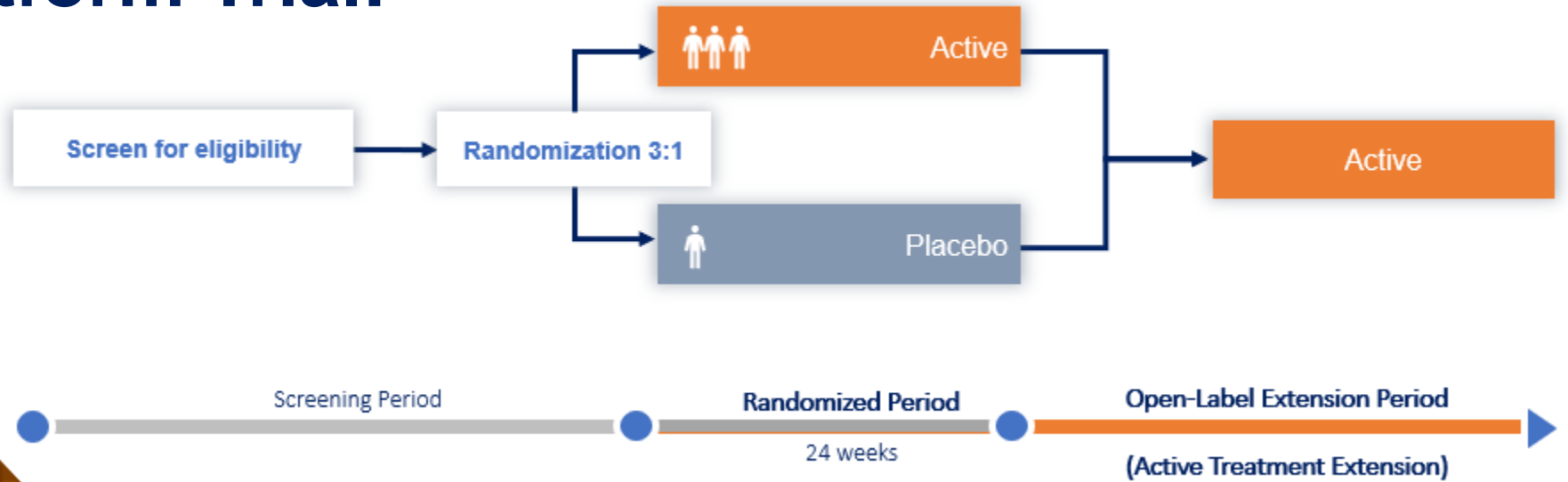
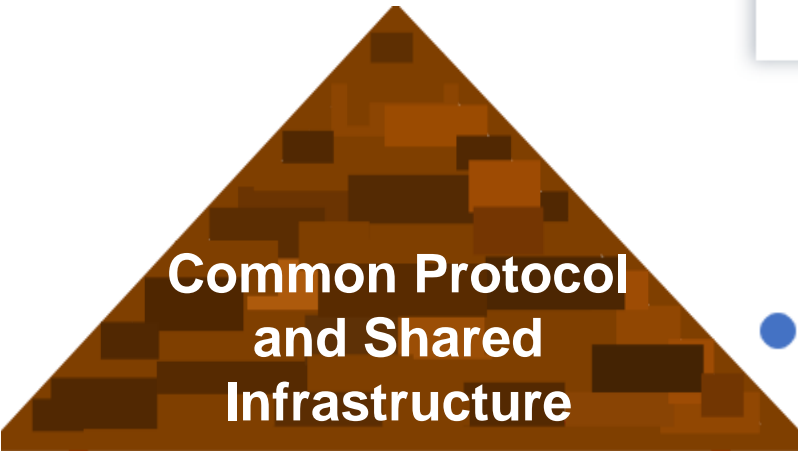
Healey & AMG Center

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

306

Participants consented to Master Protocol since RGF and RGG initiated



251

Participants assigned to RGF or RGG



210

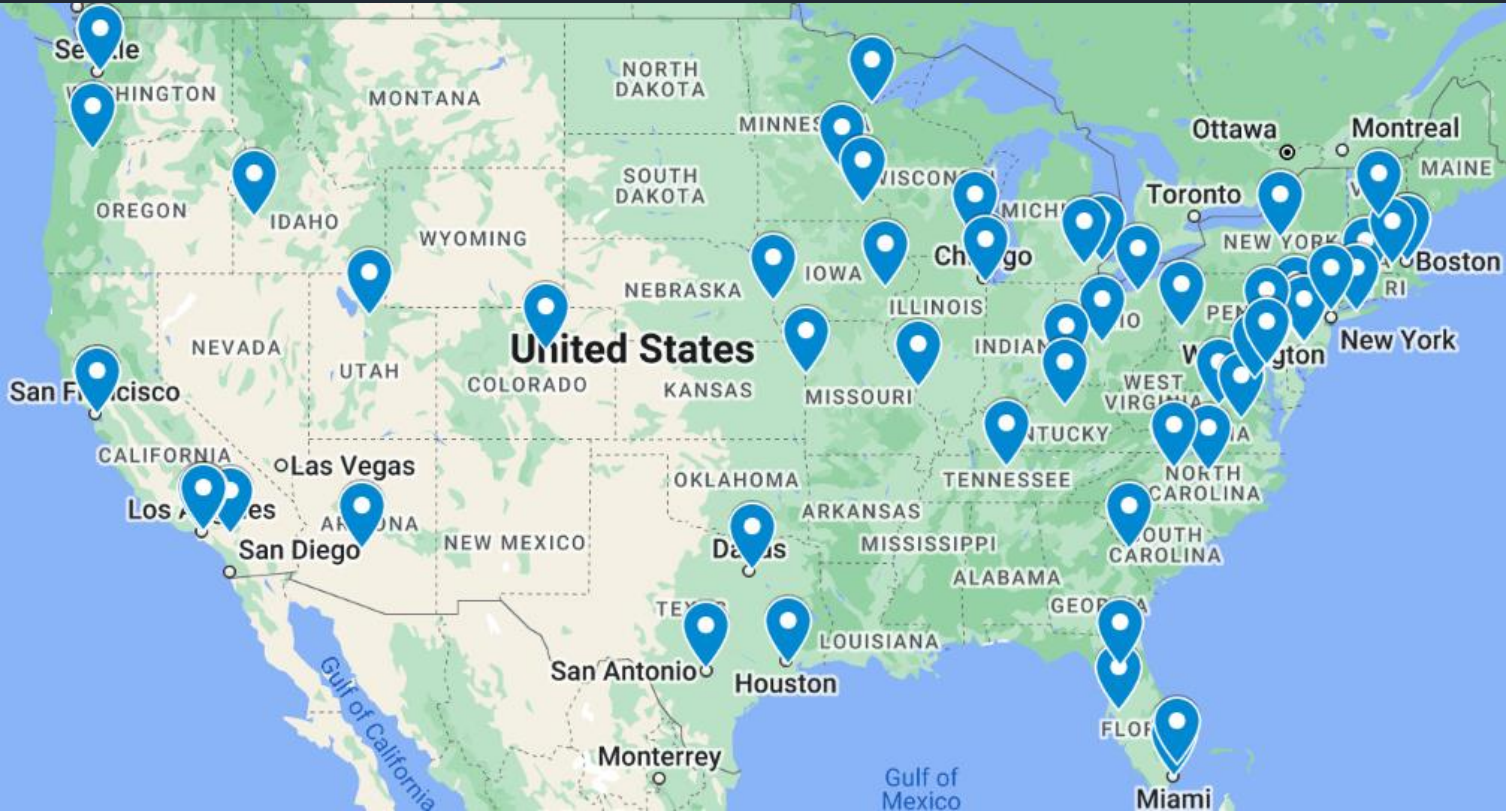
Participants randomized within RGF or RGG

Thank You

for your partnership in ALS research

(as of 9/7/23)

61 Sites Currently Active



(as of 9/7/23)

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

Site Map & Contacts:



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

Checking Site Status Online

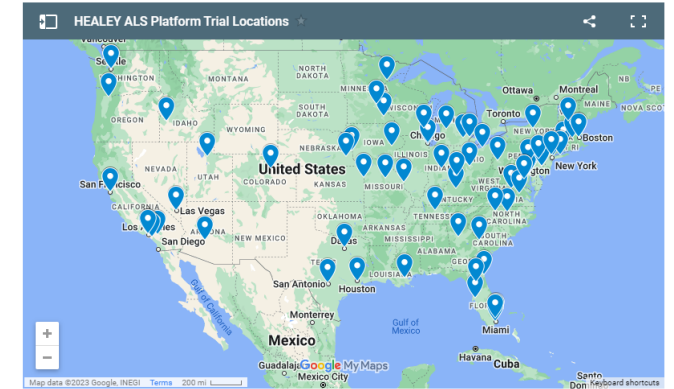
Contact a study team near you to discuss enrollment opportunities

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	Whitney Dailey
Cedars-Sinai Medical Center	CA	Recruiting	Sophia Mostowy
Forbes Norris MDA/ALS Research Center, California Pacific Medical Center	CA	Recruiting	Teji Dulai
Kaiser Permanente, Los Angeles Medical Center	CA	Recruiting	Mary H. Berganza

Map of Participating Sites



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

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.

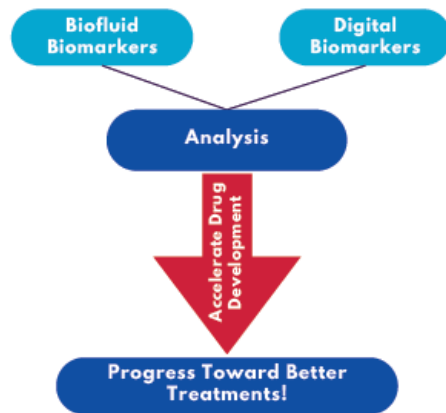
A biomarker may be used to: (1) learn more about a disease, (2) follow disease progression, (3) monitor how a disease responds to a study drug, or (4) serve as a target for treatment.

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

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

Weekly webinar
registration:



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

ALS Link sign-up:



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



Allison Bulat

Upcoming Webinars:

September 14th- weekly Q&A webinar with Jen DiMartino of ALS ONE

September 21st- weekly Q&A webinar with Amanda Lee of ALSA

September 28th- weekly Q&A webinar and EAP update