

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 26, 2023



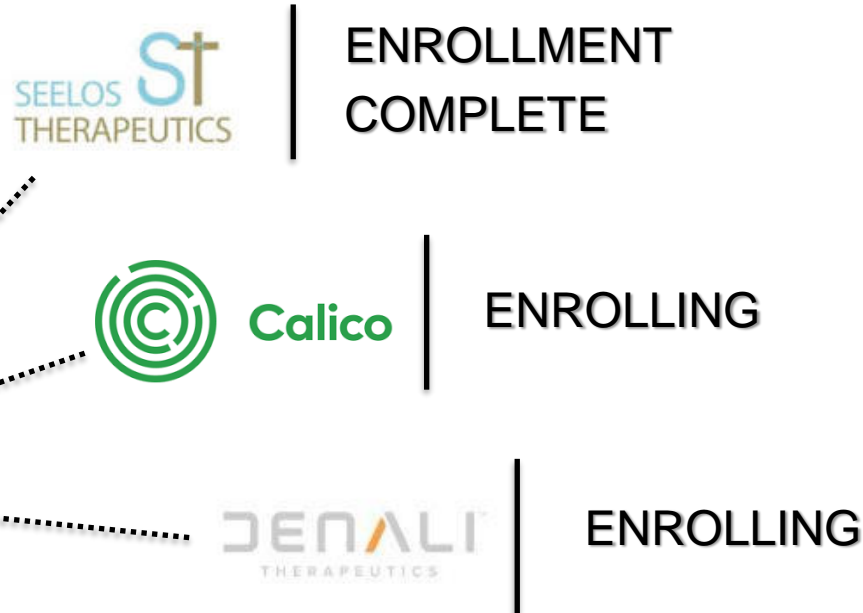
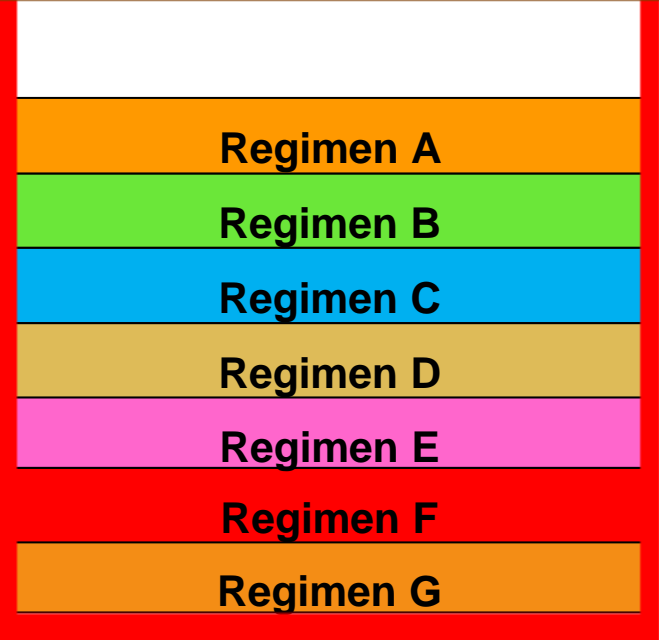
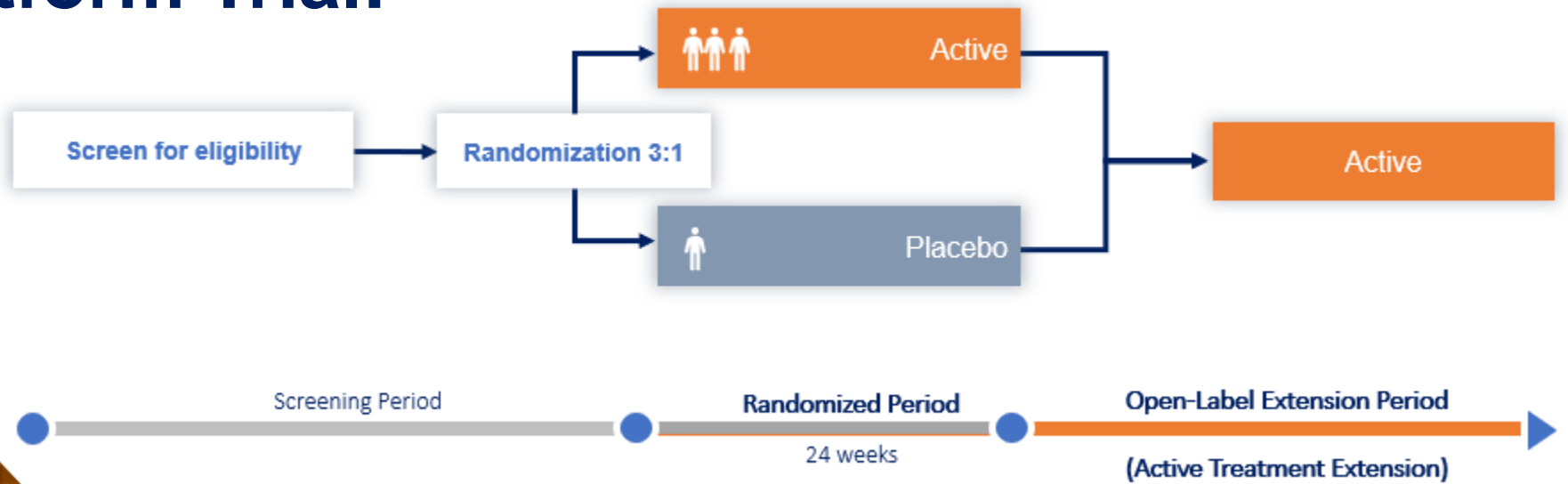
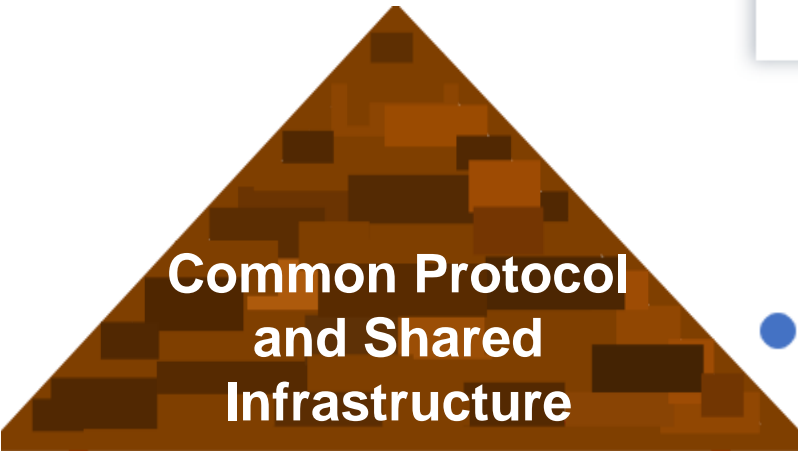
Healey & AMG Center

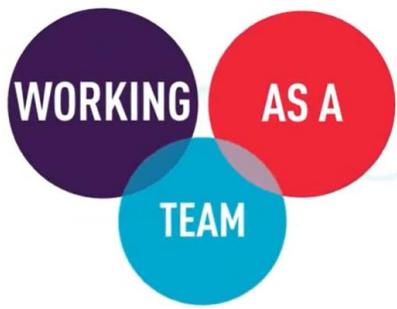
Sean M. Healey & AMG Center for ALS
at Massachusetts General Hospital



The AMG Foundation

HEALEY ALS Platform Trial:





Therapy Evaluation Committee

- All investigational products in the Platform Trial were chosen by a committee of expert ALS scientists and clinicians
- Industry partners from selected companies work with the Healey Center Trial Design Team, the Northeast ALS Consortium (NEALS), Barrow Neurological Institute and Berry Consultants to tailor a new regimen to their experimental drug
- Each investigational product added to the Platform Trial is believed to have an equal chance of success for all forms of ALS based on available scientific evidence

Criteria for selection included:

- Robust preclinical data (data from the lab that support the scientific rationale for testing these products in ALS)
- Previous human experience in ALS or other neurological diseases (to support the dose, safety and target engagement of the product and its inclusion in a late-stage trial such as the Platform Trial).

Common Protocol – Endpoints

Primary Endpoint

- Change in disease severity through 24 weeks
- ALS Functional Rating Scale-Revised (ALSFRS-R) + Mortality
- Potential to provide confirmatory evidence with overall type I error of 5%

Secondary Endpoints

1. Change in respiratory function - slow vital capacity (SVC)
2. Change in muscle strength - hand held dynamometry (HHD)
3. Survival

Safety Endpoints

Biomarkers and Exploratory Endpoints (DNA, NfL, Speech app, Home Spirometry)

Enrollment Update: Regimen F and Regimen G

404

Participants consented to Master Protocol since RGF and RGG initiated



339

Participants assigned to RGF or RGG



298

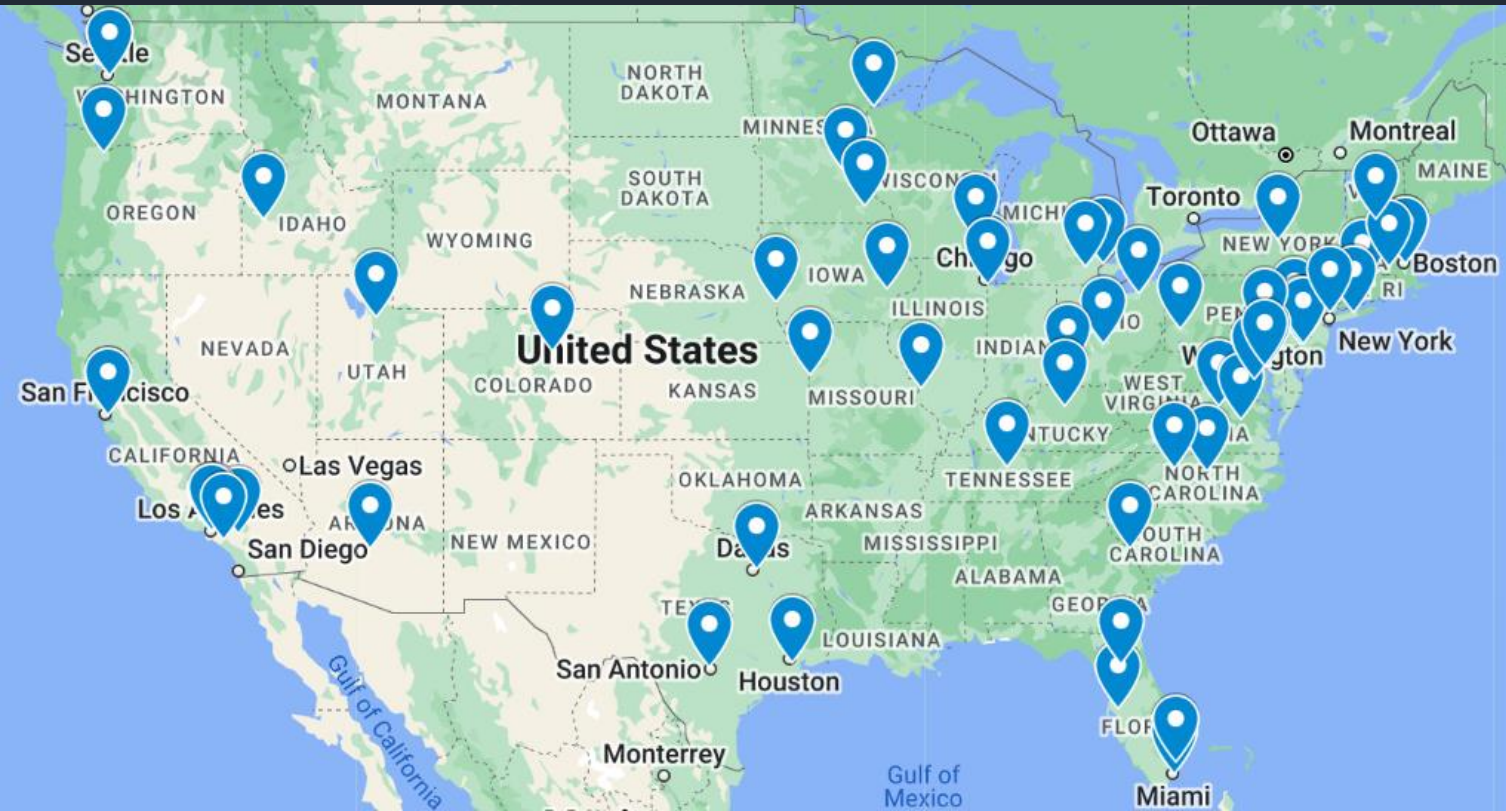
Participants randomized within RGF or RGG

Thank You

for your partnership in ALS research

(as of 10/26/23)

63 Sites Currently Active



(as of 10/26/23)

- ✓ 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

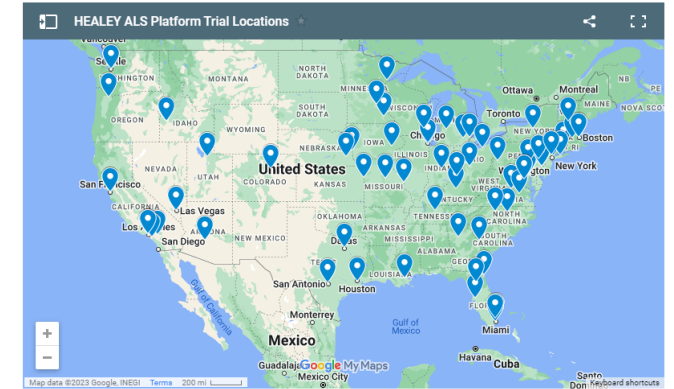
Contact a study team near you to discuss enrollment opportunities

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	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>

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>

Upcoming Webinars:

November 2nd- weekly Q&A

November 9th- weekly Q&A and EAP discussion with Dr. Suma Babu (Mass General)

November 16th- Regimen G science Q&A with Denali Therapeutics



Allison Bulat