

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 21, 2023



Healey & AMG Center

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



Calico



The AMG Foundation



RESEARCH | CARE | ADVOCACY

ALS Focus Survey Program

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Amanda Lee, MPH, MA

September 21, 2023

What is ALS Focus?



- Research study for people with ALS and their caregivers in the United States
- Online surveys that ask about needs, preferences, and experiences
- De-identified survey data is shared for free to inform:
 - Clinical trial design
 - Care and service programs
 - Public policy



Patient and caregiver-led research



- ALS Focus Patient and Caregiver Advisory Committee (PCAC) drives survey topics, questions, and other research components
- ALS Focus study brings the perspectives of the community to the forefront
- Data from large surveys is a powerful tool

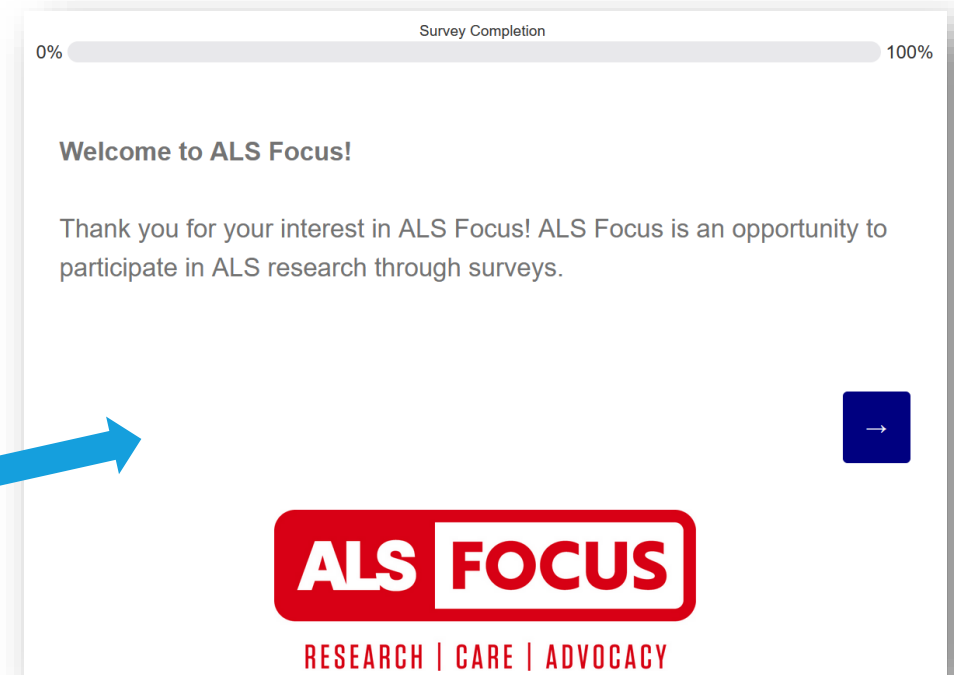


How do I participate in ALS Focus surveys?

Go to www.ALSFocus.org!



The screenshot shows the ALS Focus landing page. At the top left is the ALS Association logo. The navigation menu includes 'Understanding ALS', 'Navigating ALS', 'Local Support', 'Research' (highlighted), 'Advocacy', and 'Get Involved'. Below the navigation is a breadcrumb trail: 'Home / Research / Participate in Research / ALS Focus Survey Program'. The main heading is 'ALS Focus' under the sub-header 'RESEARCH | CARE | ADVOCACY'. A paragraph describes the program: 'ALS Focus™ is a patient- and caregiver-led survey program that asks people with ALS and current and past caregivers about their needs and challenges. The goal is to learn as much as possible about individual experiences throughout the disease journey so that the whole ALS community can benefit. All data collected is de-identified and shared free of charge with the entire ALS community.' At the bottom, there is a red button that says 'CLICK HERE TO TAKE ALS FOCUS SURVEYS →'. A blue arrow points from this button towards the survey completion screen on the right.



The screenshot shows the survey completion screen. At the top, there is a progress bar labeled 'Survey Completion' with '0%' on the left and '100%' on the right. Below the progress bar, the text reads: 'Welcome to ALS Focus!' followed by 'Thank you for your interest in ALS Focus! ALS Focus is an opportunity to participate in ALS research through surveys.' At the bottom right, there is a blue button with a white right-pointing arrow. At the bottom center, there is a red box containing the text 'ALS FOCUS' in white, with 'RESEARCH | CARE | ADVOCACY' in red below it.

OPEN NOW: Mobility at Home Survey



- Survey is open: **August 23 – October 23**
- People with ALS, current caregivers, and past caregivers are eligible to participate
- This survey takes about 20 minutes to complete
- Sign up at www.ALSFocus.org or check for an email from ALSFocus@als.org



Thank You!



Acknowledgements

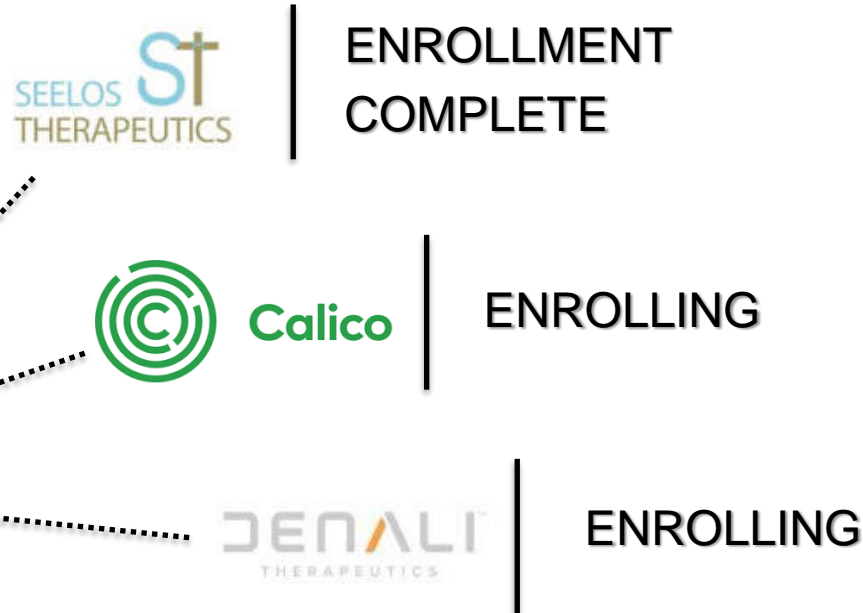
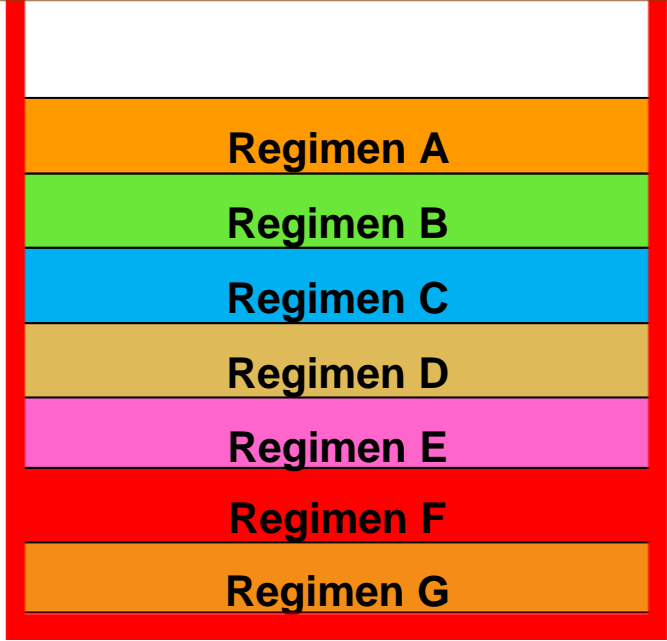
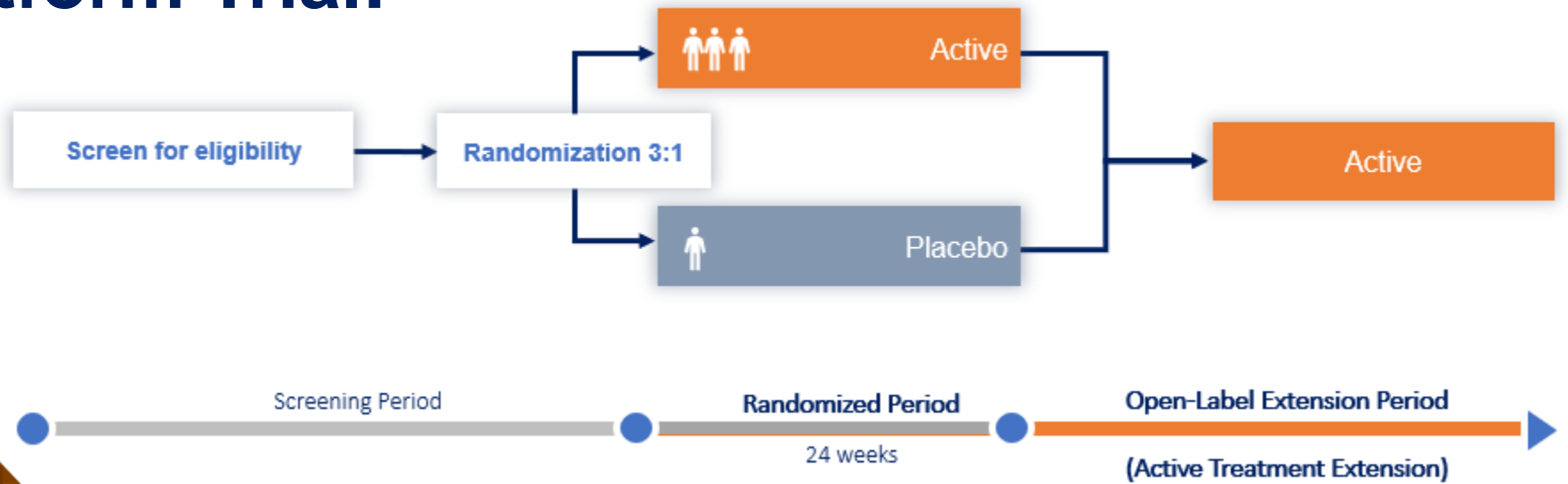
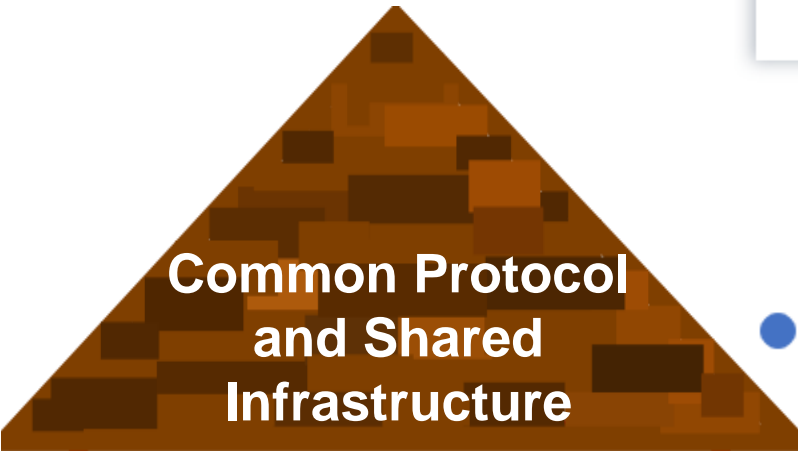
- ALS Focus Participants
- Patient and Caregiver Advisory Committee
- Steering Committee
- Working Groups
- Massachusetts General Hospital Neurological Clinical Research Institute
- Industry Sponsors

Contact

Amanda Lee, MPH, MA
Associate Director, ALS Focus Program
Focus Inquiries: ALSFocus@als.org

Register Today!
www.ALSFocus.org

HEALEY ALS Platform Trial:



Enrollment Update: Regimen F and Regimen G

343

Participants consented to Master Protocol since RGF and RGG initiated



281

Participants assigned to RGF or RGG



236

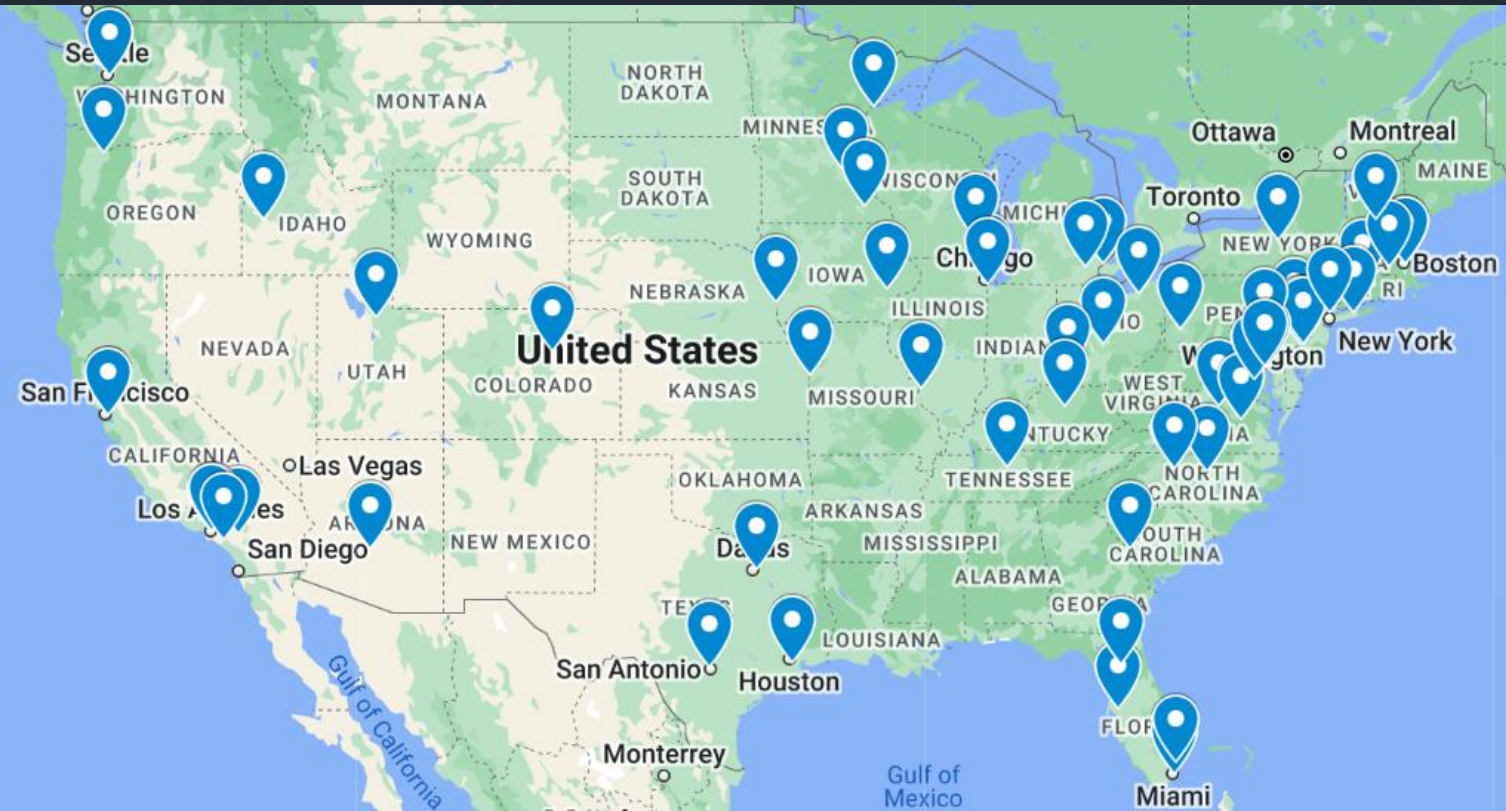
Participants randomized within RGF or RGG

Thank You

for your partnership in ALS research

(as of 9/21/23)

62 Sites Currently Active



(as of 9/21/23)

- ✓ 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

Checking Site Status Online

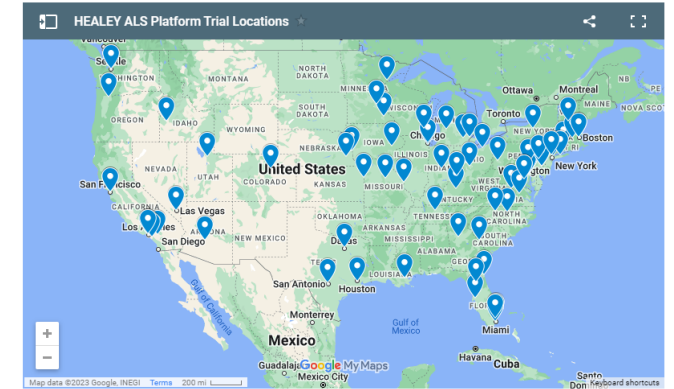
Contact a study team near you to discuss enrollment opportunities

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

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E-mail: healeyalsplatform@mgh.harvard.edu

Weekly webinar
registration:



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

ALS Link sign-up:



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

Upcoming Webinars:

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

October 5th- canceled for NEALS conference

October 12th- weekly Q&A



Allison Bulat