

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 14, 2023



## Healey & AMG Center

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



Calico



The AMG Foundation

**ALS**  
**ONE** ↑



**UNITED**  
in the fight against ALS.



**ALS**  
**ONE** ↑

**HEALEY ALS PLATFORM TRIAL WEEKLY WEBINAR**  
**September 14, 2023**

Jennifer DiMartino, Executive Director, ALS ONE  
jen@alsONE.org







**UNITED**  
in the fight against ALS.

## OUR BACKGROUND



**ALS ONE was founded in 2016 by Massachusetts resident, Kevin Gosnell, a prominent business leader who was diagnosed with ALS at the prime of his life at age 46.**

- Kevin's vision was to break down the barriers that traditional silos created and open the door for unity in the fight against a disease that needed it so desperately. To create a research and care team, joining as **ONE**, to help reduce redundancy, advance science more effectively & efficiently, and to help individuals living with ALS navigate the complexities of the disease by supporting them as a team. **ALS ONE** was formed, uniting the Massachusetts leaders, among the world-leaders in ALS research and care:





## COMMUNICATION *is everything*

Regular meetings are held with our research and care teams, fostering a unified approach to advancing critical ALS studies while ensuring that individuals living with ALS have the equipment, resources, and support needed and deserved to live their best life while navigating the complexities of the disease.

### RESEARCH



**Merit Cudkowicz, MD, MSc**

Chief of Neurology & The Healey Ctr. for ALS at MGH



**Robert Brown MD, DPhil**

Chair of Neurology UMass Medical School



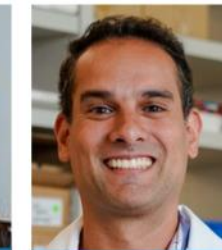
**James Berry, MD, MPH**

Chief, Div. of ALS & Director, NCRI at MGH



**Sabrina Paganoni, MD, PhD**

Co-Director, NCRI at MGH



**Fernando Vieira, MD**

Chief Science Office and CEO at ALS TDI

### CARE



**Darlene Sawicki MSN, NP-BC**

Director of Advanced Practice Providers at MGH



**Jen Scalia, MSN, NP**

Assoc. Director of The Healey Ctr. at MGH



**Judi Carey, RN**

Clinical Research Access Nurse at MGH



**Ron Hoffman**

Founder and Executive Director at CCALS





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in the fight against ALS.



## COMMITMENT *to our mission*

Our founder lost his battle to ALS just 7 months after our launch, but since then, we have raised almost \$17 Million to advance key initiatives in ALS research with and for our partners.

Over two-thirds of what we have raised to date has come from grassroots fundraising - from events big, small & everything in between.

A small staff with low overhead yet with a very large footprint.





## Funding: *ways we have and continue to make a difference*

We continue to help make significant advancements by funding the following initiatives, among others:

### MGH

- THE HEALEY ALS Platform Trial
  - (founding partner and benefactor).
- Expanded Access Program (EAP)
- Parenting at a Challenging Time (PACT) Program
- ALS House Call program,
- Full-time ALS Research Access Nurse
- Nutrition and Data studies
- Biomarker studies
- Neutotechnology initiatives

### UMASS

- Preclinical studies of microRNA-based suppression of SOD1 and divalent siRNA suppression of SOD1
- Preclinical studies of ASO afinersen to suppress C9orf72
- Preclinical studies of ASO to modify splicing of cryptic STMN2
- Single-cell transcriptomic analysis of SOD1 mice

### CCALS

- \$2M+ in equipment
- Care Resources & emotional support
- Completion of the Retreat Center
- Expanded resources in NY/NJ area

### ALS TDI

- Completion of AT-1501
- Phase 1 trial of anti-CD40-Ligand Antibody
- Preclinical mice studies





# UNITED

in the fight against ALS.

6TH ANNUAL  
**ALS RESEARCH SYMPOSIUM**  
NOV 16, 17 & 20TH 2023  
*Together from afar*

A Conference uniting researchers, clinicians and individuals living with ALS from around the world for three days in the sharing of important ALS research and much hope.

HOSTED ALONG WITH OUR ALS ONE RESEARCH TEAM:  
Dr.'s James Berry, MD, MPH, Robert H. Brown, Jr, MD, DPhil, Merit Cudkowicz, MD, MSc, Sabrina Paganoni, MD, PhD, and Fernando Vieira, MD.

Keynote Speakers and Moderators *more to come!*


ALS ONE RESEARCH SYMPOSIUM . ORG

## Research & Care joint initiatives: *Profound examples of unity*

- Bi-Weekly team conference calls
- In-Services to major hospitals to train on caring for individuals with ALS. – Hospital Grand Rounds, VNA's, hospice & palliative groups.
- Webinars for patients and families.







# UNITED

in the fight against ALS.



## WAYS to get involved



### Host an Event

Events as small as a car wash and as large as a golf tournament are held regularly by pALS, families, and supporters and mean the world to us - and we help every step of the way!



### Drive with our Plate

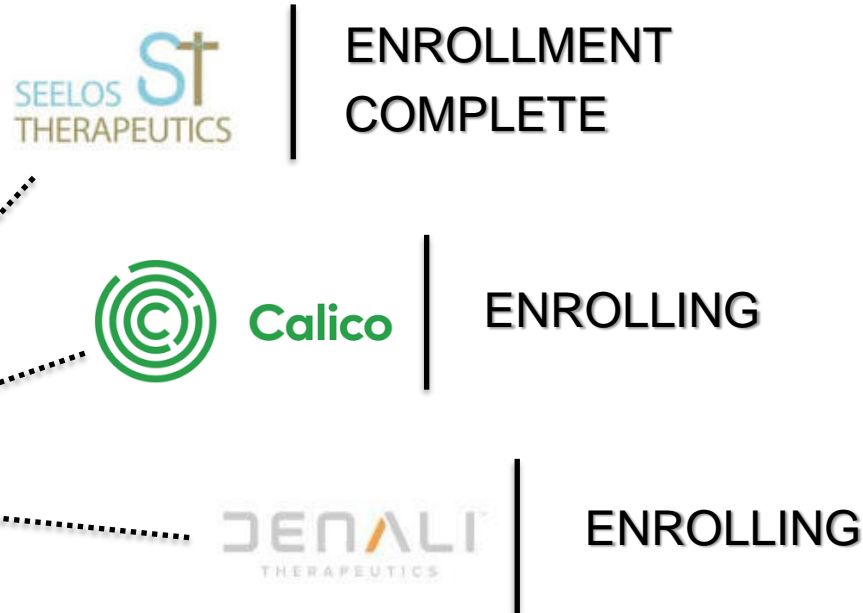
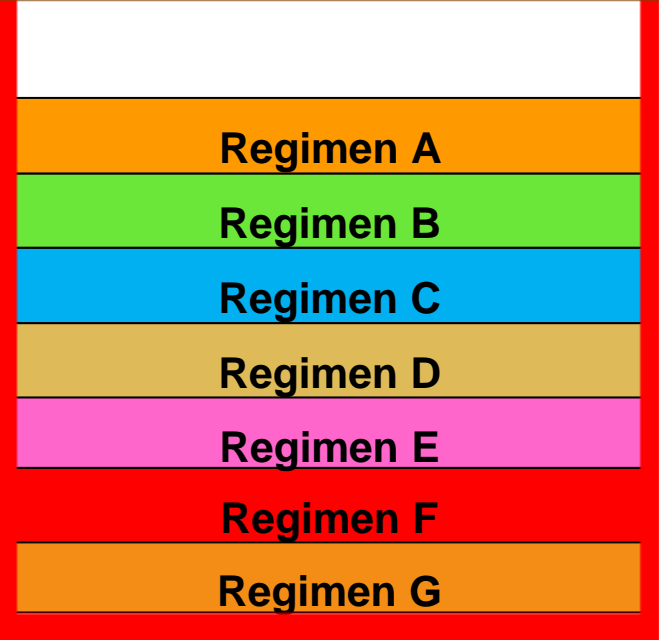
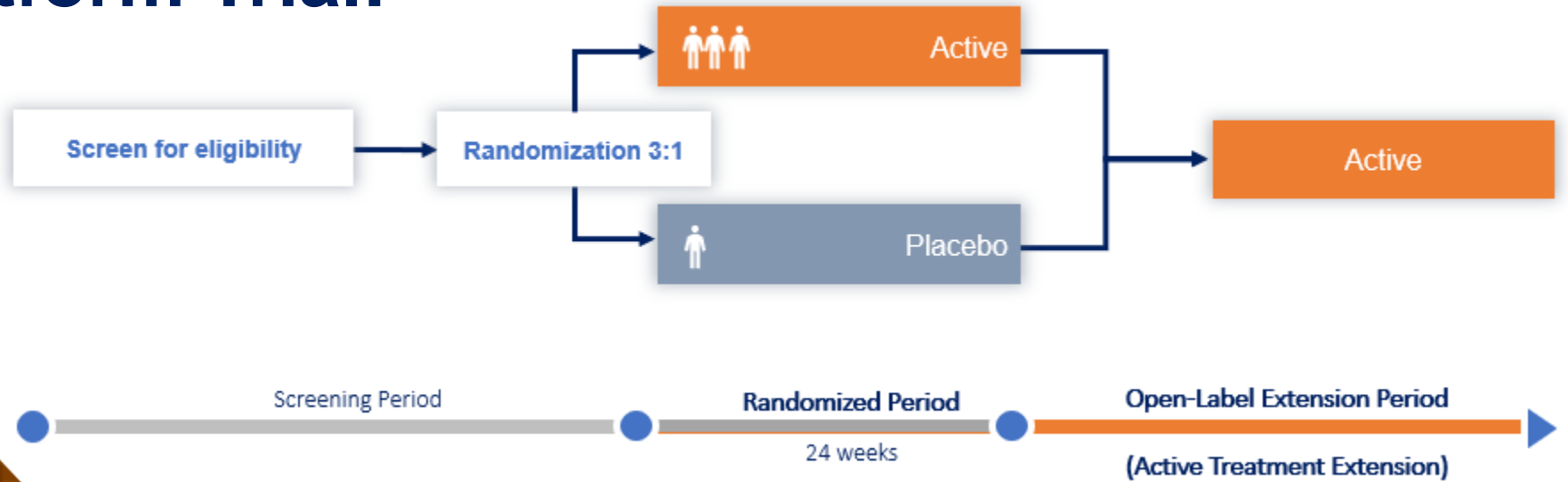
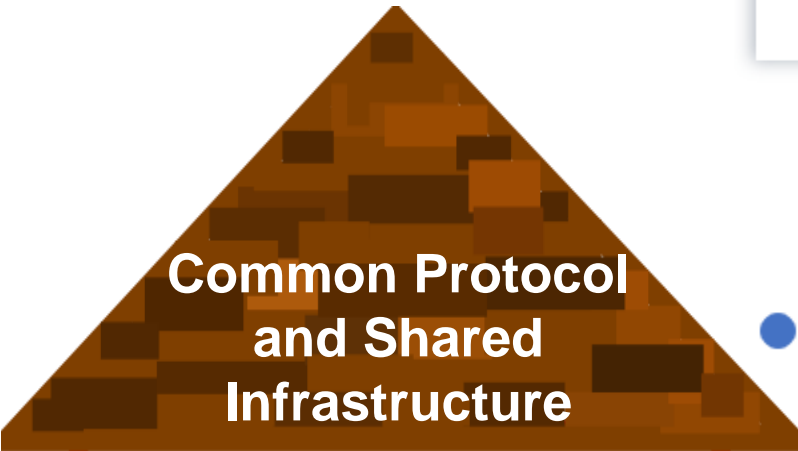
Massachusetts drivers can support ALS research and care every single day while driving with the Mass. ALS License Plate.

## Attend one of our events

- 9.16.23: Roy-ALS Ride for ALS
- 9.16.23: 1/2 Way to St. Patrick's Day Party
- 9.25.23: cALSea Crew Golf Tourney
- 10.1.23: Rise Against ALS: Decades Party
- 10.2.23: Cars & Coffee
- 10.7-9.23: Southborough Golf Weekend
- 10.9.23: Team NSF Drive Golf Tourney
- 10.21.23: Tucker Toss Cornhole Tourney
- 10.26.23: Go On Be Brave! Movie Screening
- 11.5.23: NYC Marathon Team
- 11.16-20.23: ALS ONE Research Symposium



# HEALEY ALS Platform Trial:





# Enrollment Update: Regimen F and Regimen G

328

Participants consented to Master Protocol since RGF and RGG initiated



267

Participants assigned to RGF or RGG



226

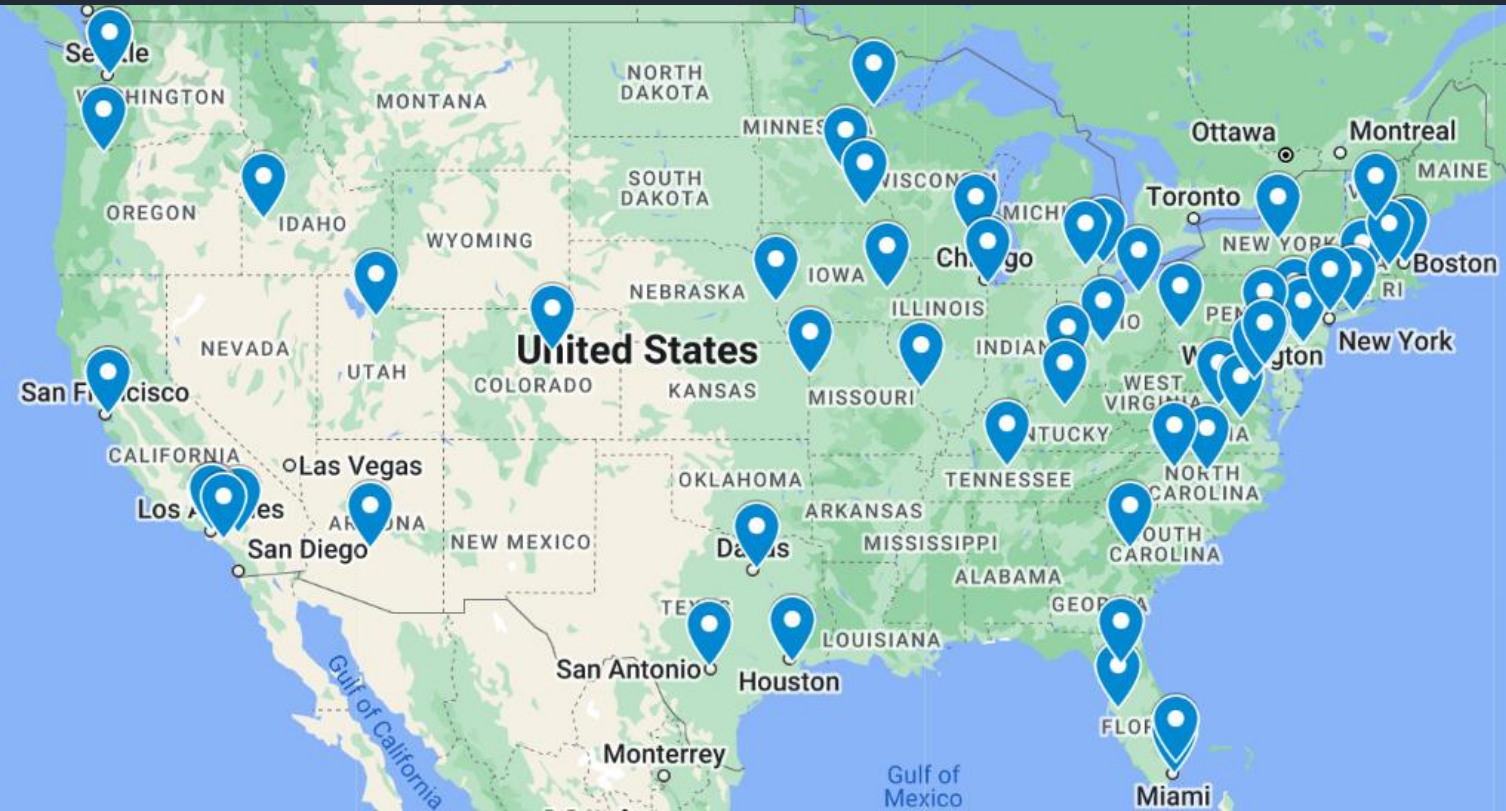
Participants randomized within RGF or RGG

*Thank You*

for your partnership in ALS research

(as of 9/14/23)

# 62 Sites Currently Active



(as of 9/14/23)

- ✓ 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	<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>

# 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>



Allison Bulat

### Upcoming Webinars:

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

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

**October 5th-** canceled for NEALS conference