

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 – June 29, 2023



Healey & AMG Center

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



als FINDING a CURE™



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The AMG Foundation



Calico

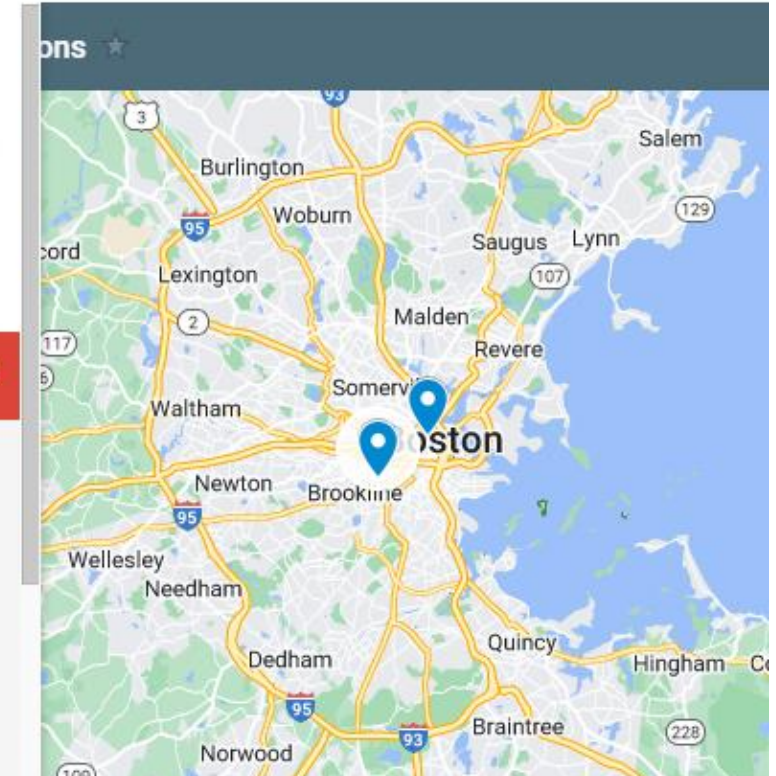
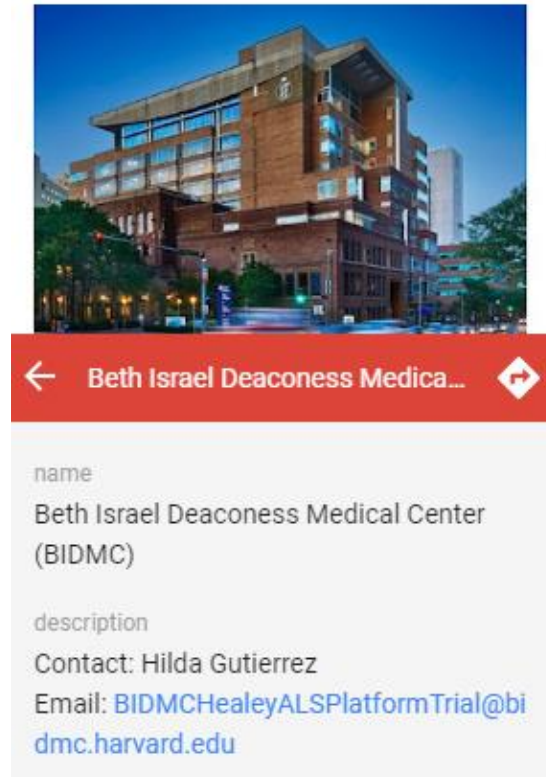


Guest Speaker



Hilda Gutierrez, MD

Research Operations Coordinator
Beth Israel Deaconess Medical Center
Boston, MA



HEALEY ALS Research Team at BIDMC



Dr. Courtney McIllduff
Principal Investigator



Dr. Seward Rutkove
Sub-Investigator



Hilda Gutierrez
Lead Study Coordinator

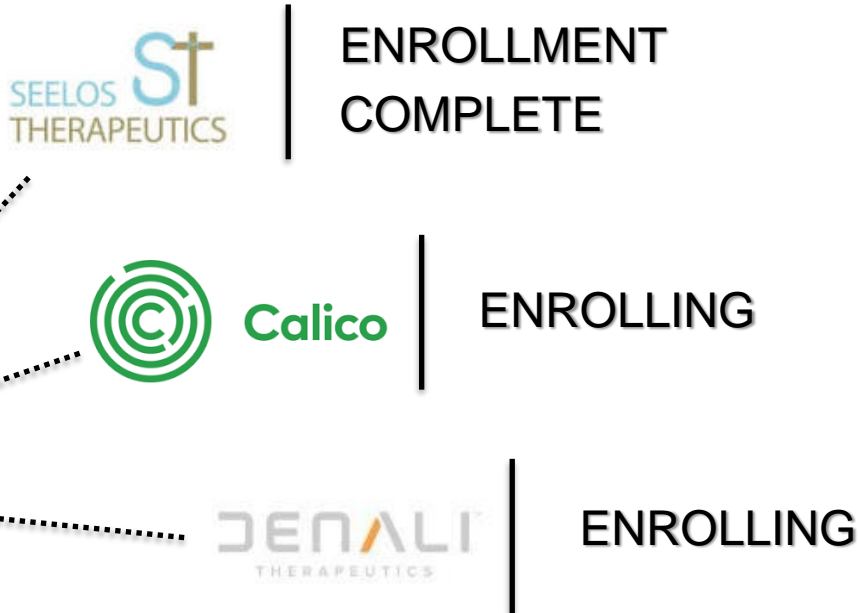
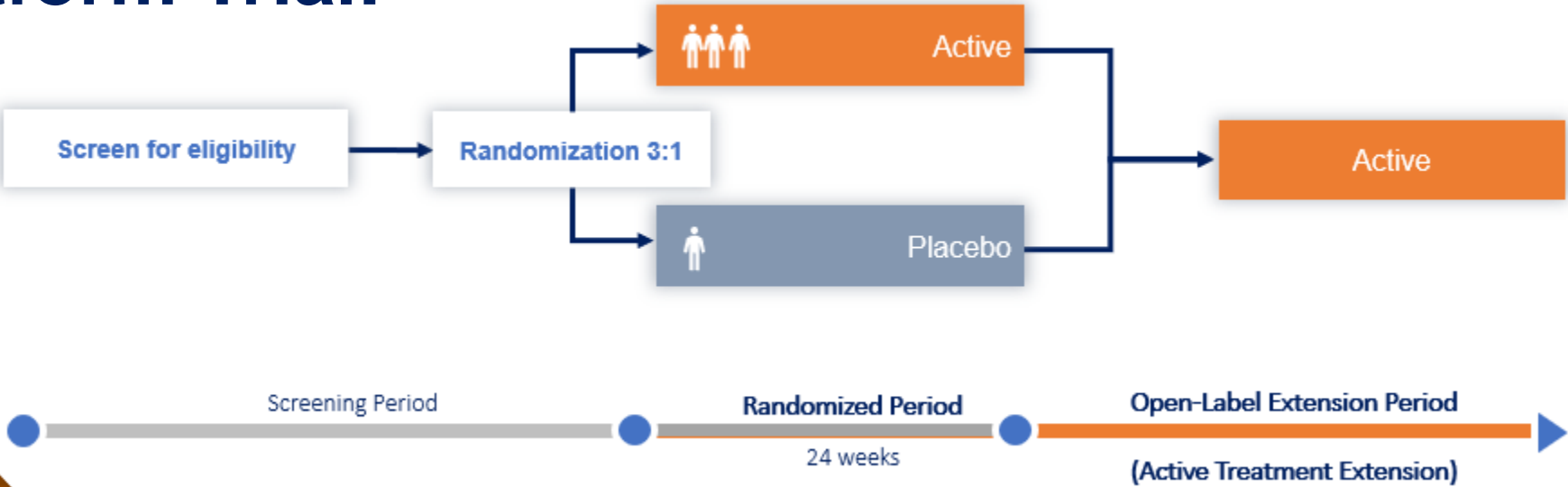
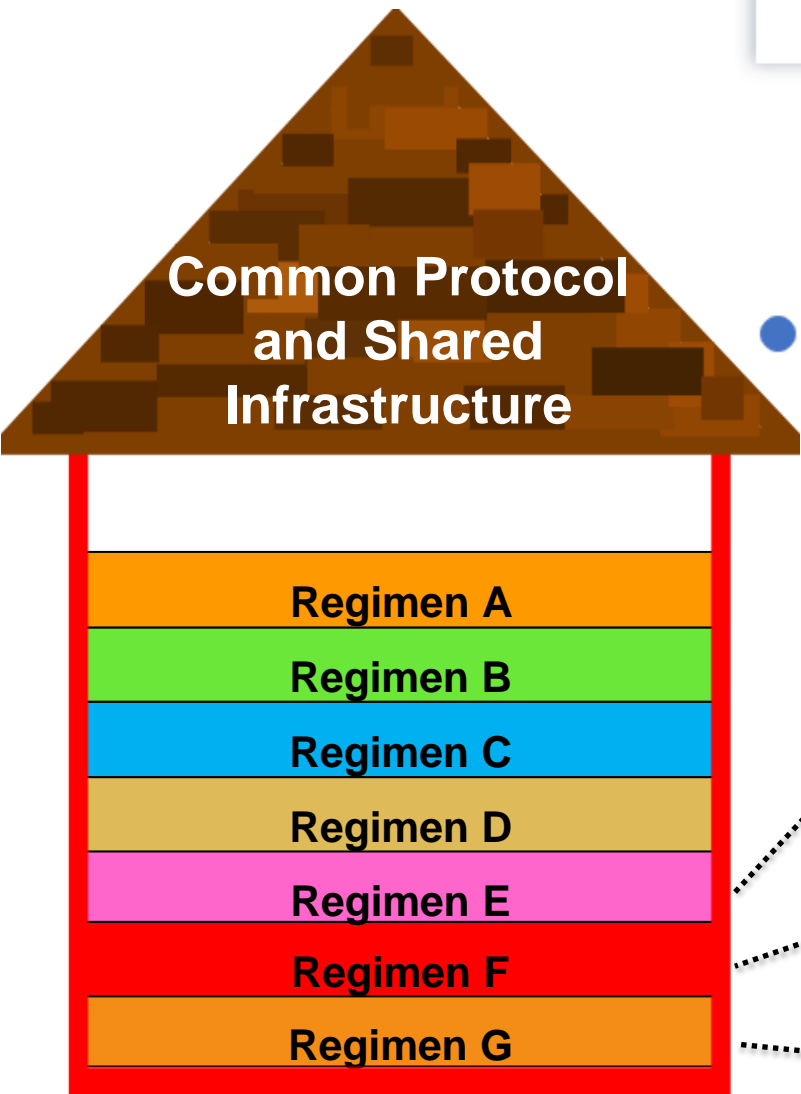


Soleil Samaan
Study Coordinator



Diane Healy
Study Nurse

HEALEY ALS Platform Trial:




First Published
Article! - 2021



<https://bit.ly/3CSeD1g>

Adaptive Platform Trials to Transform Amyotrophic Lateral Sclerosis Therapy Development

Sabrina Paganoni, MD, PhD ^{1,2#} James D. Berry, MD, MPH,^{1#} Melanie Quintana, PhD,^{3#}
Eric Macklin, PhD,⁴ Benjamin R. Saville, PhD,^{3,5} Michelle A. Detry, PhD,³
Marianne Chase, BA,¹ Alexander V. Sherman, MSc,¹ Hong Yu, MS,¹ Kristin Drake, MBA,¹
Jinsy Andrews, MD,⁶ Jeremy Shefner, MD, PhD,⁷ Lori B. Chibnik, PhD, MPH,⁴
Matteo Vestrucci, PhD,³ Merit E. Cudkowicz, MD, MSc,¹
and for the Healey ALS Platform Trial Study Group

Current therapeutic development in amyotrophic lateral sclerosis (ALS) relies on individual randomized clinical trials to test a specific investigational product in a single patient population. This approach has intrinsic limitations, including cost, time, and lack of flexibility. Adaptive platform trials represent a novel approach to investigate several interventions for a single disease in a continuous manner. Already in use in oncology, this approach is now being employed more often in neurology. Here, we describe a newly launched platform trial for ALS. The Healey ALS Platform Trial is testing multiple investigational products concurrently in people with ALS, with the goal of rapidly identifying novel treatments, biomarkers, and trial endpoints.

ANN NEUROL 2022;00:1–11

<https://onlinelibrary.wiley.com/doi/10.1002/ana.26285>

New Published
Article - 2023
Open Access!



<https://bit.ly/3pp7H8E>

Design and Statistical Innovations in a Platform Trial for Amyotrophic Lateral Sclerosis

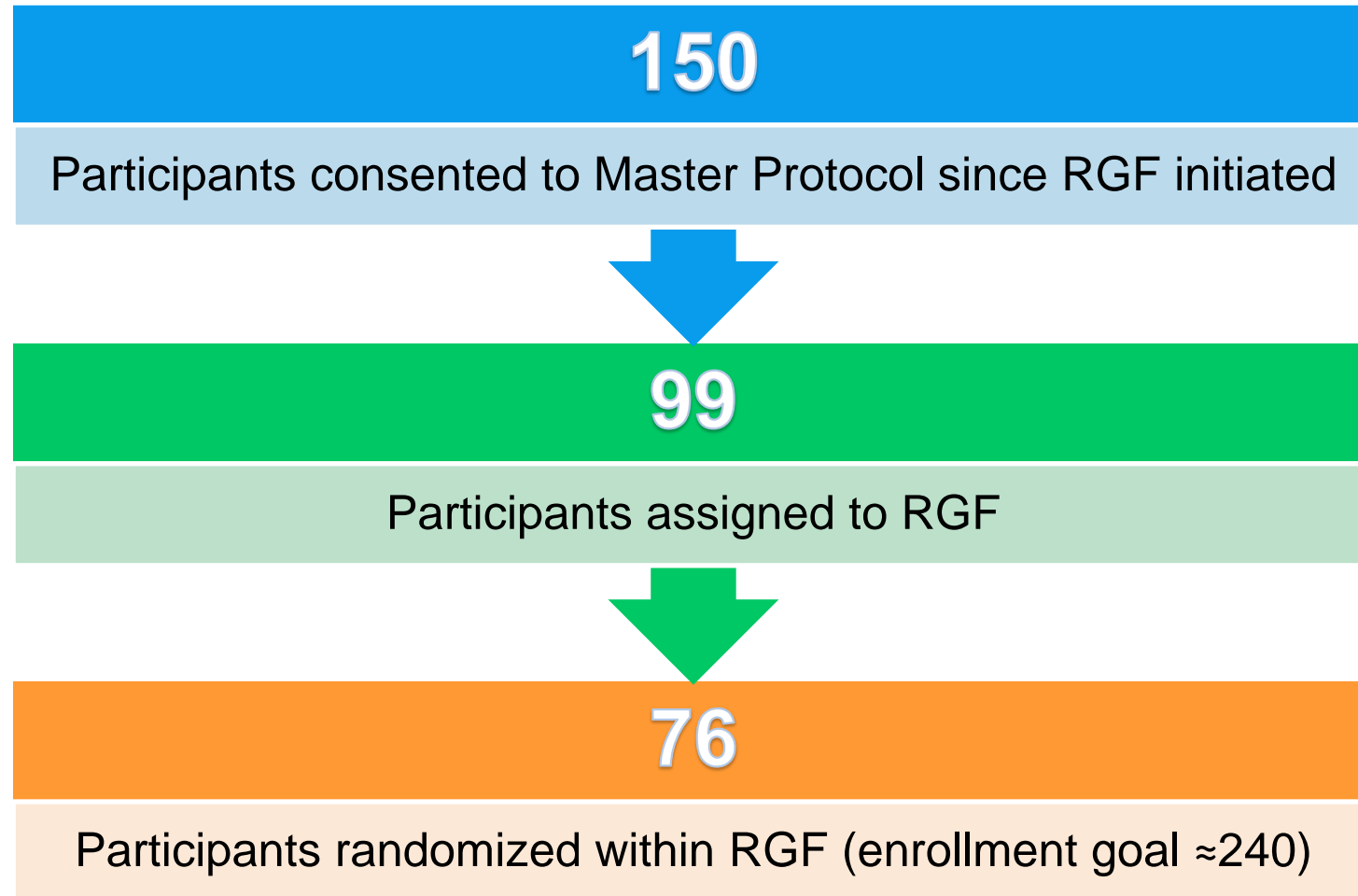
Melanie Quintana, PhD,¹ Benjamin R. Saville, PhD,^{1,2} Matteo Vestrucci, PhD,¹
Michelle A. Detry, PhD,¹ Lori Chibnik, PhD, MPH,^{3,4} Jeremy Shefner, MD, PhD,⁵
James D. Berry, MD, MPH,^{6,7} Marianne Chase, BA,^{6,7} Jinsy Andrews, MD,⁸
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for the HEALEY ALS Platform Trial Study Group

Platform trials allow efficient evaluation of multiple interventions for a specific disease. The HEALEY ALS Platform Trial is testing multiple investigational products in parallel and sequentially in persons with amyotrophic lateral sclerosis (ALS) with the goal of rapidly identifying novel treatments to slow disease progression. Platform trials have considerable operational and statistical efficiencies compared with typical randomized controlled trials due to their use of shared infrastructure and shared control data. We describe the statistical approaches required to achieve the objectives of a platform trial in the context of ALS. This includes following regulatory guidance for the disease area of interest and accounting for potential differences in outcomes of participants within the shared control (potentially due to differences in time of randomization, mode of administration, and eligibility criteria). Within the HEALEY ALS Platform Trial, the complex statistical objectives are met using a Bayesian shared parameter analysis of function and survival. This analysis serves to provide a common integrated estimate of treatment benefit, overall slowing in disease progression, as measured by function and survival while accounting for potential differences in the shared control group using Bayesian hierarchical modeling. Clinical trial simulation is used to provide a better understanding of this novel analysis method and complex design.

ANN NEUROL 2023;00:1–14

[Design and Statistical Innovations in a Platform Trial for Amyotrophic Lateral Sclerosis - Quintana - Annals of Neurology - Wiley Online Library](#)

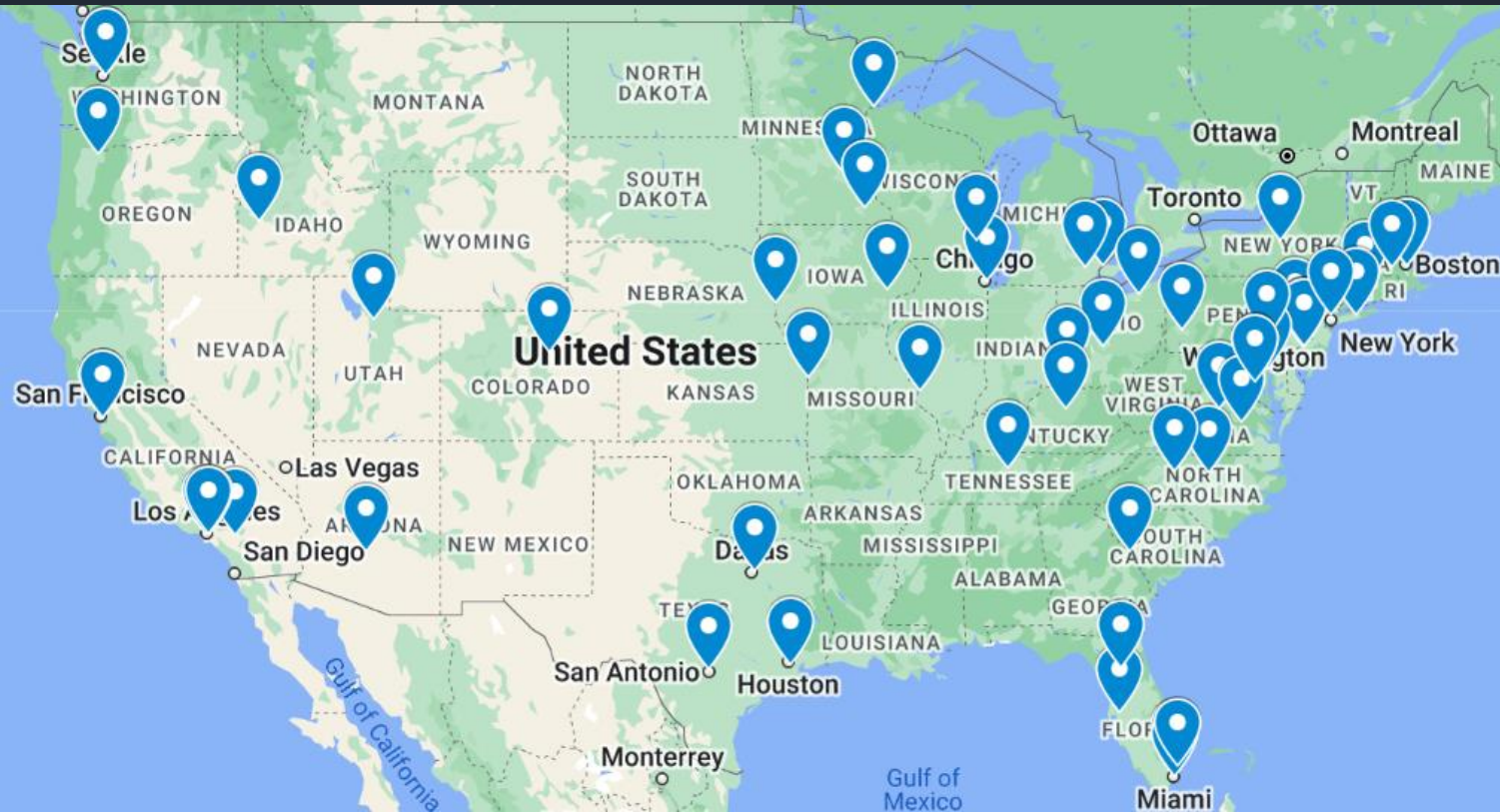
Enrollment Update: Regimen F (as of 6/29/23)



Thank You

for your partnership in ALS research

58 Sites Currently Active for Regimen F



(as of 6/29/23)

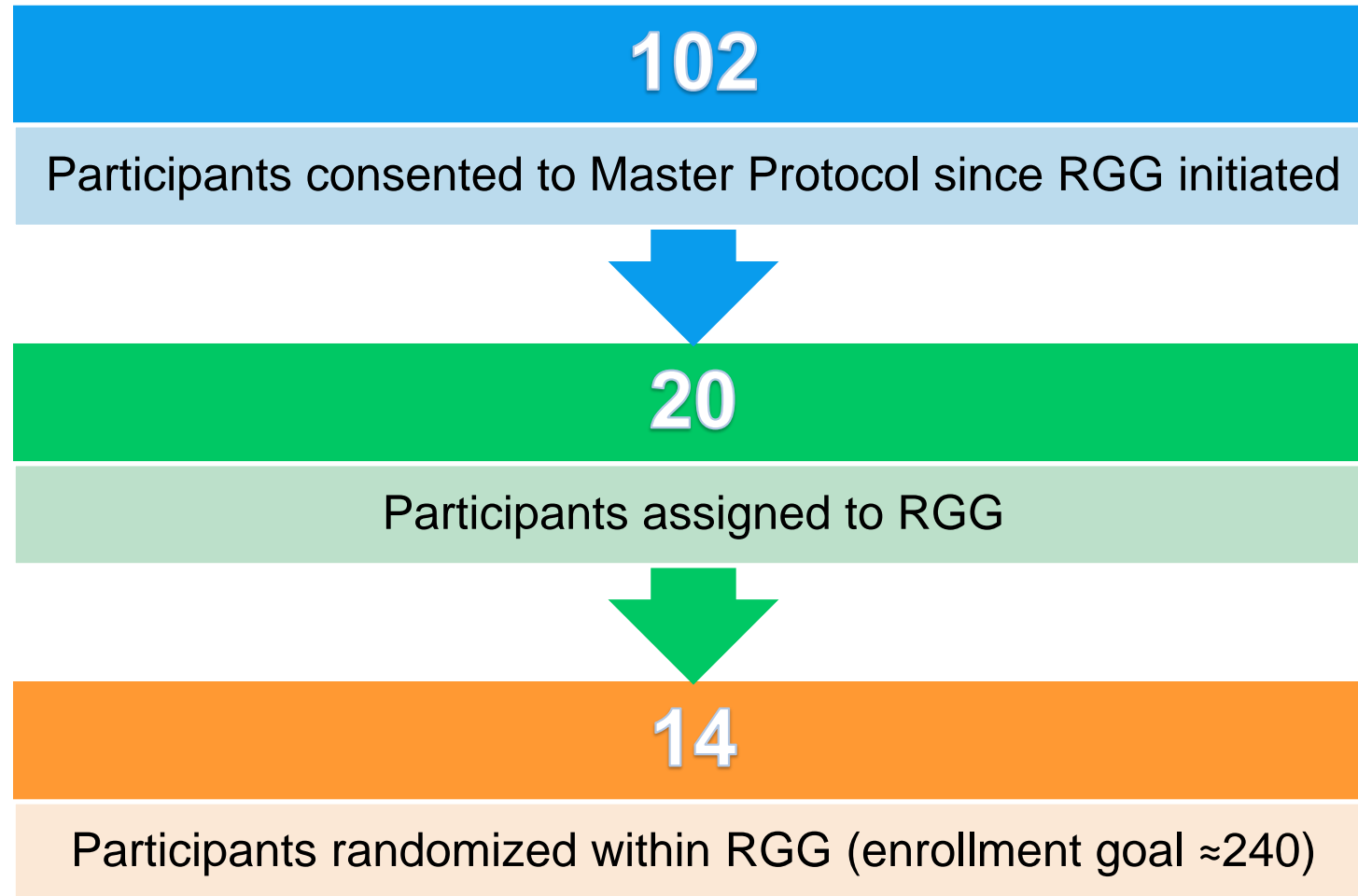
- ✓ Nova Southeastern University
- ✓ Essentia Health
- ✓ Texas Neurology
- ✓ Mass General Hospital
- ✓ University of Nebraska
- ✓ Hospital for Special Care
- ✓ Henry Ford Hospital
- ✓ Augusta University
- ✓ Beth Israel Deaconess
- ✓ University of Texas HSC
- ✓ University of Colorado
- ✓ Loma Linda University
- ✓ 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

Site Map & Contacts:



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

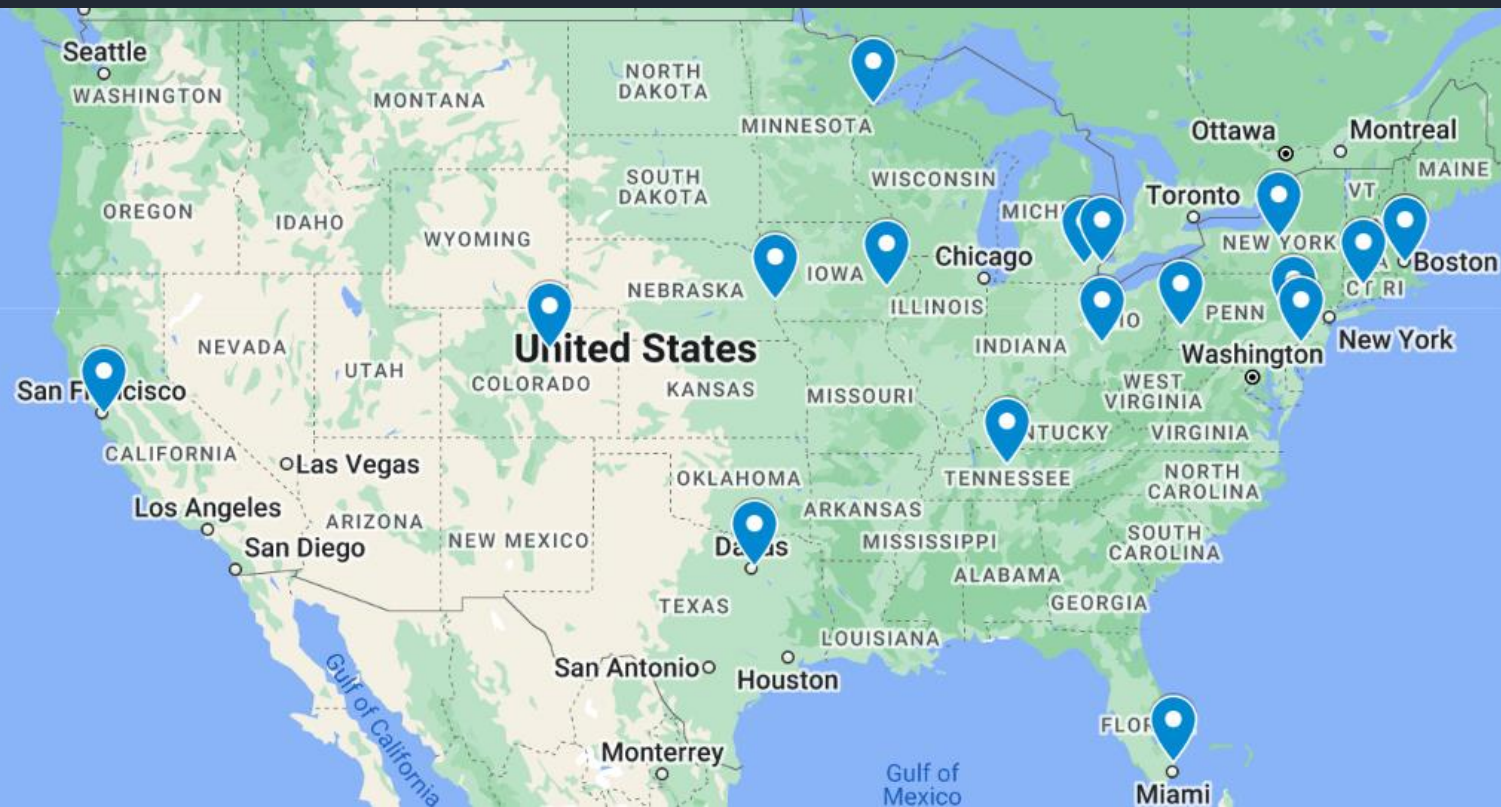
Enrollment Update: Regimen G (as of 6/29/23)



Thank You

for your partnership in ALS research

18 Sites Currently Active for Regimen G



(as of 6/29/23)

- ✓ Nova Southeastern University
- ✓ Texas Neurology
- ✓ University of Colorado
- ✓ Essentia Health
- ✓ University of Pittsburgh
- ✓ Beth Israel Deaconess
- ✓ Mass General Hospital
- ✓ Ohio State University
- ✓ University of Michigan
- ✓ Hospital for Special Care
- ✓ Vanderbilt University
- ✓ Henry Ford
- ✓ University of Iowa
- ✓ University of Nebraska
- ✓ Lehigh Valley
- ✓ CPMC
- ✓ University of Pennsylvania
- ✓ SUNY Upstate

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>

Regimen G Drug Science Q&A Webinar



Open to everyone!
Thursday, July 20th
5:00-6:00pm Eastern

Topic: DNL343 Drug Science and Mechanism of Action

Link to Register: <https://bit.ly/3NqJU1j>





THE ALS ASSOCIATION/NORTHEAST ALS CONSORTIUM

Educational Webinar

UNDERSTANDING STATISTICAL AND CLINICAL SIGNIFICANCE

SPEAKERS



JINSY ANDREWS, MD, MSC
COLUMBIA UNIVERSITY



CHRISTINA FOURNIER, MD, MSC
EMORY UNIVERSITY

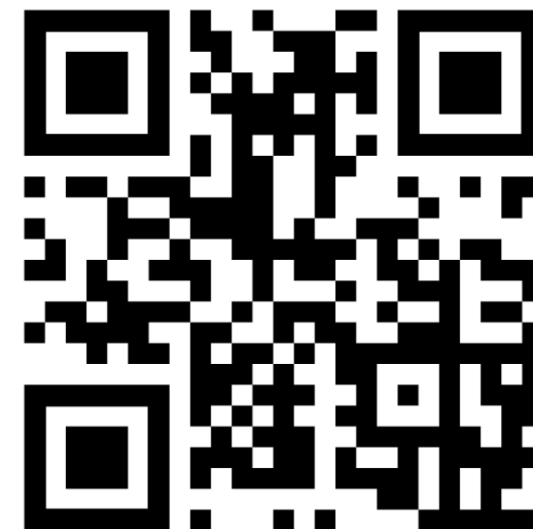


ERIC MACKLIN, PhD
HARVARD MEDICAL
SCHOOL

What do researchers and scientists mean when they describe a study as “statistically significant?” How do people with ALS and clinicians understand whether or not a treatment option is “clinically significant?” This webinar and panel discussion will provide a framework for understanding the results of ALS clinical trials, using several recent studies as examples.

**Recording
Available!**

View Here:



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

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:

July 6th- Weekly Q&A with Bill Cho MD, PhD from Calico Life Sciences (Regimen F)

July 13th- Weekly Q&A with Sarah Heintzman, APRN-CNP (Ohio State University)

July 20th- Regimen G / DNL343 Drug Science Q&A Webinar with Denali