

HEALEY ALS Platform Trial

Weekly Q&A – Feb 2, 2023



Healey Center

Sean M. Healey & AMG Center
for ALS at Mass General



Calico

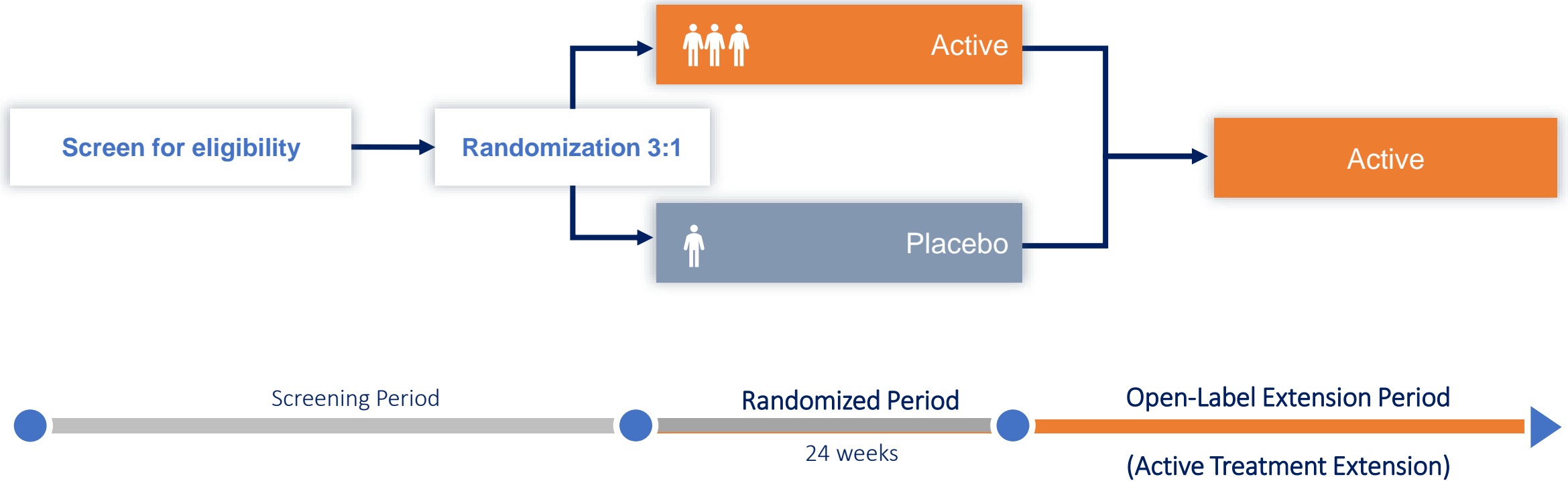


THE ARTHUR M. BLANK
FAMILY FOUNDATION



The AMG Foundation

Schema for Each Regimen





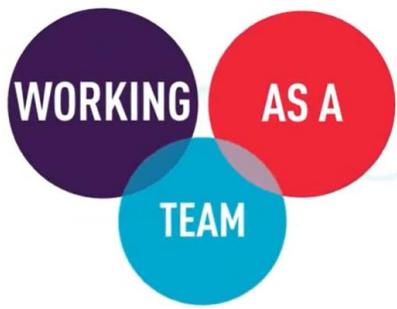
MASTER PROTOCOL

Regimen A
Regimen B
Regimen C
Regimen D
Regimen E
Regimen F
Regimen G

SEELOS **St**
THERAPEUTICS | ENROLLING

 **Calico** | IN START-UP

 **DENALI**
THERAPEUTICS | IN DESIGN



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).

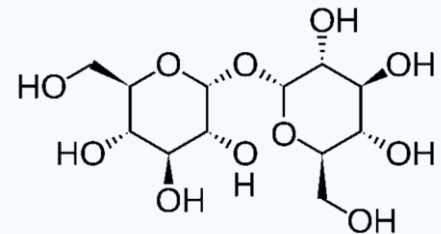
Informational Webinars about **Regimen E**

Trehalose/SLS-005 Drug Science and Mechanism of Action Q&A Webinar

Hosted by Seelos Therapeutics on 10 March 2022

Recording Available!

SEELOS **St**
THERAPEUTICS



<https://bit.ly/3L4hrdB>

Recording available under “science and mechanism of action series”

The ALS Association/Northeast ALS Consortium Educational Webinar

Update on Healey ALS Platform Trial Regimen E: Trehalose for ALS

UPDATE ON REGIMEN E

TREHALOSE FOR ALS



Merit Cudkowicz, MD, MSc

SEAN M. HEALEY & AMG
CENTER FOR ALS AT MGH



Shafeeq Ladha, MD

BARROW NEUROLOGICAL
INSTITUTE

TUESDAY | 4 OCTOBER
3 PM EDT

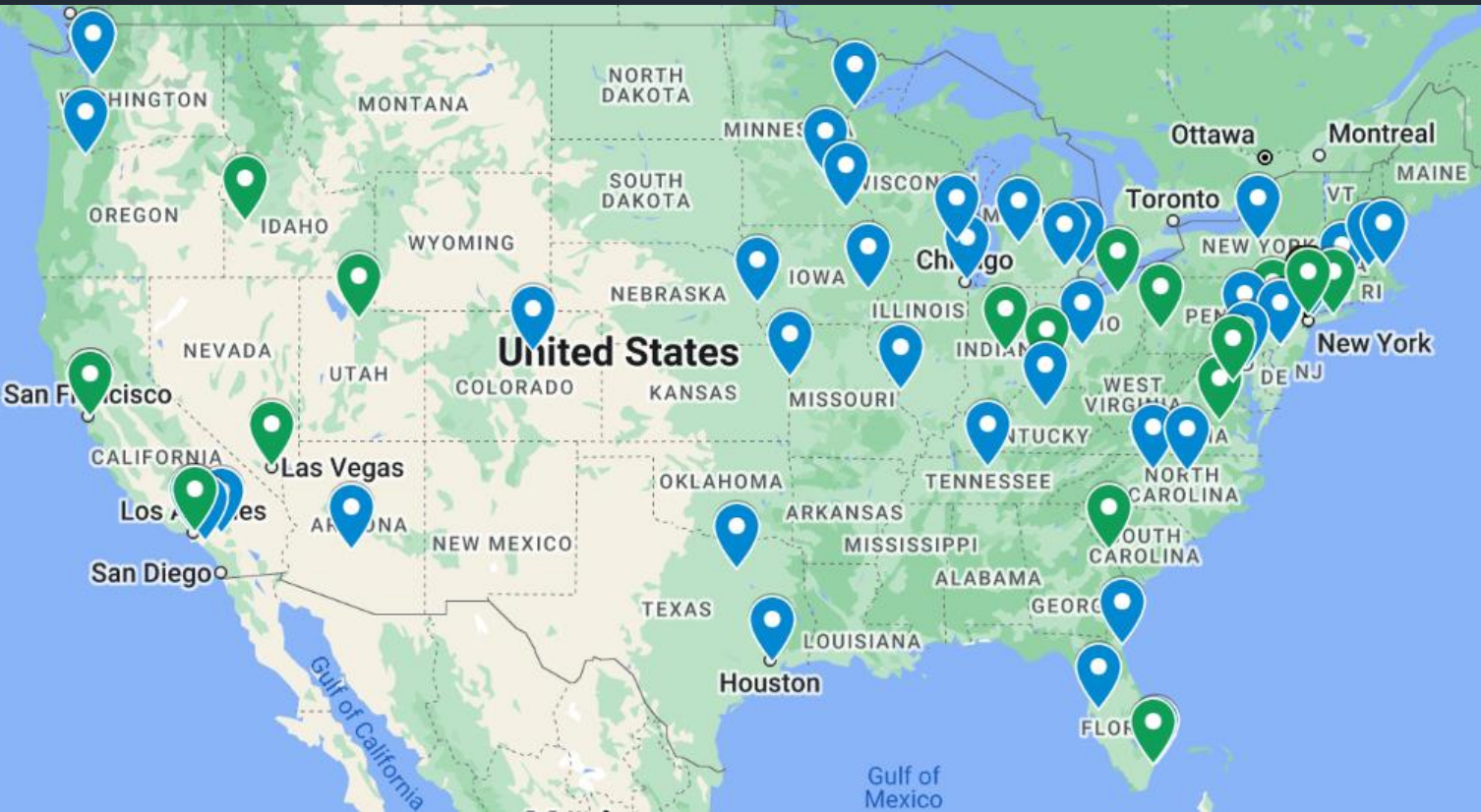
Recording Available!



<https://bit.ly/3X2u004>

Recording available under “educational webinars” on neals.org

60 Sites Currently Activated for Regimen E



(as of 2/2/23)

Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!

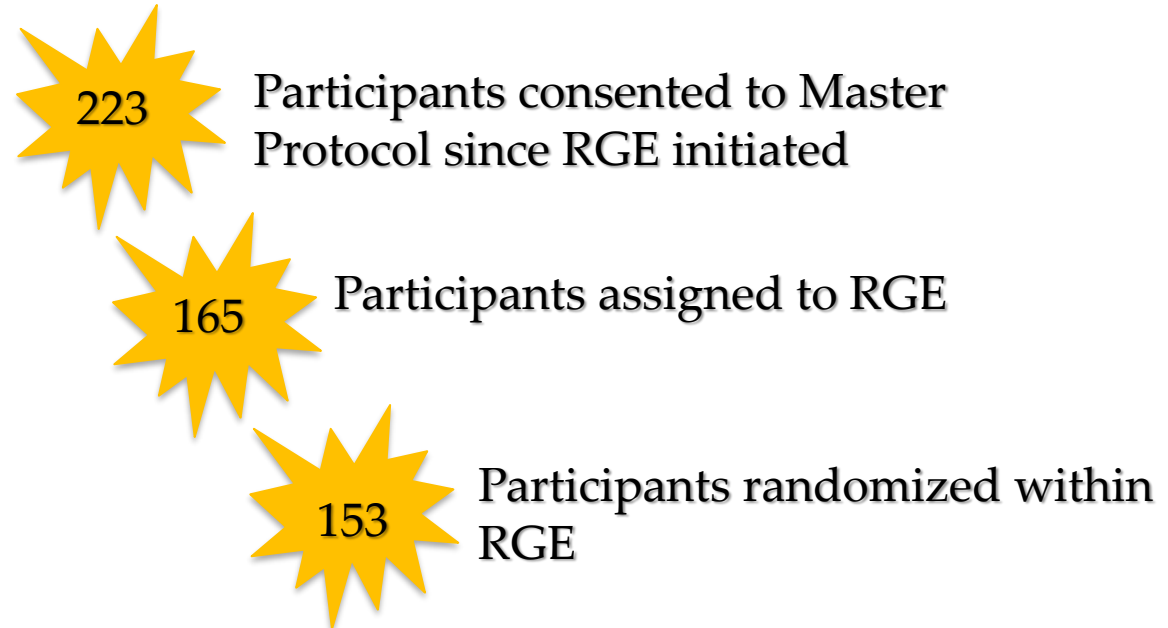
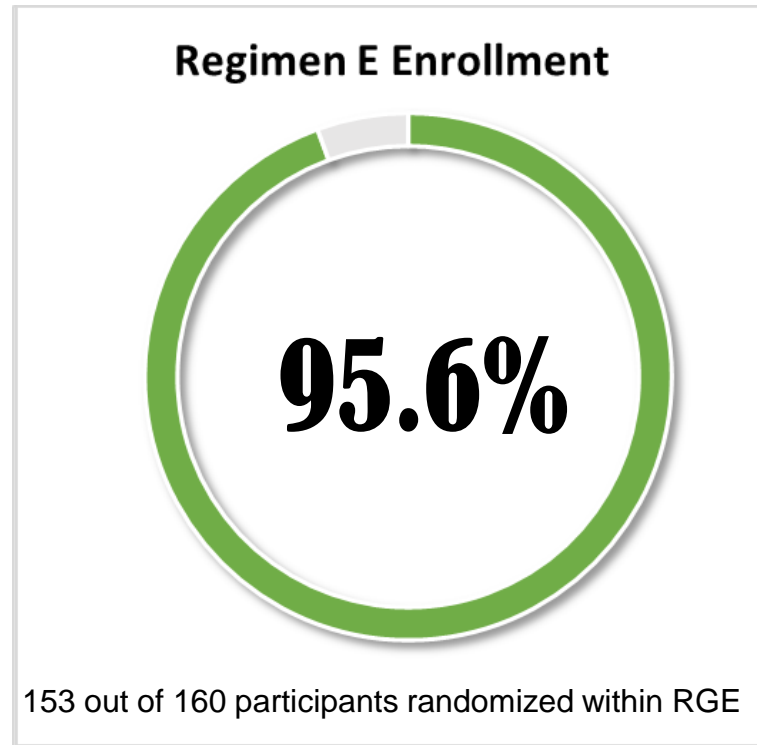
- Lehigh Valley Health Network
- Mass General Hospital
- University of Kansas
- University of Maryland
- California Pacific Medical Center
- Northwestern University
- Virginia Commonwealth University
- University of Nebraska
- Washington University
- Wake Forest University
- Hospital for Special Care
- Saint Alphonsus Regional
- University of Massachusetts
- Duke University
- Barrow Neurological Institute
- Georgetown University
- Texas Neurology
- Beth Israel Deaconess Medical Center
- SUNY Upstate
- Spectrum Health
- Henry Ford Hospital
- Essentia Health
- University of Southern California
- University of South Florida
- University of Colorado
- Providence Brain and Spine
- University of Minnesota
- Loma Linda University
- University of Iowa
- Swedish Medical Center
- Ohio State University
- University of Cincinnati
- Thomas Jefferson University
- UC San Francisco
- Mayo Rochester
- University of Washington
- Vanderbilt University
- UPMC
- Indiana University
- Augusta University
- University of Utah
- Holy Cross Hospital
- Penn State Hershey
- University of CA, Irvine
- Cedars Sinai Medical Center
- University of Pennsylvania
- Nova Southeastern University
- Johns Hopkins University
- Columbia University
- Stony Brook University
- Kaiser, Los Angeles
- Cleveland Clinic
- Medical College of Wisconsin
- University of Michigan
- Las Vegas Clinic
- George Washington University
- Mayo Clinic Florida
- University of Kentucky
- Houston Methodist
- Hackensack University

Site Map & Contacts:



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

Regimen E Updates (as of Feb 2, 2023)



Weekly Recordings Available on MGH Website

Webinar Recordings

Science & Mechanism of Action Series +

Weekly & Monthly Updates: 2023 -

January 26, 2023: Weekly Q&A and Regimen C Update

Sabrina Paganoni, MD, PhD presented updates on the HEALEY ALS Platform Trial and answered questions from the audience. Guest speakers Michael Hotchkin (Chief Development Officer at Clene Nanomedicine, Inc) and James Berry, MD, MPH (Regimen C co-Lead, Mass General Hospital) joined the webinar again this week to recap the latest news and updates regarding Regimen C.

[Watch recording.](#) [Download slides \(PDF\).](#)

January 19, 2023: Weekly Q&A

Sabrina Paganoni, MD, PhD presented this week's updates on the HEALEY ALS Platform Trial and answered questions from the audience.

[Watch recording.](#) [Download slides \(PDF\).](#)

Visit the Website:



<https://bit.ly/3g4kzfv>

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:

February 9th, 16th- Weekly Q&A

February 23rd- Jonathan Katz, MD (Site Investigator at CPMC in California)



Allison Bulat