

HEALEY ALS Platform Trial

Weekly Q&A – Jan 12, 2023



Healey Center

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



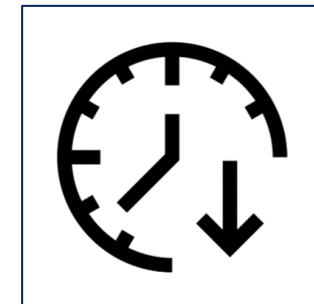
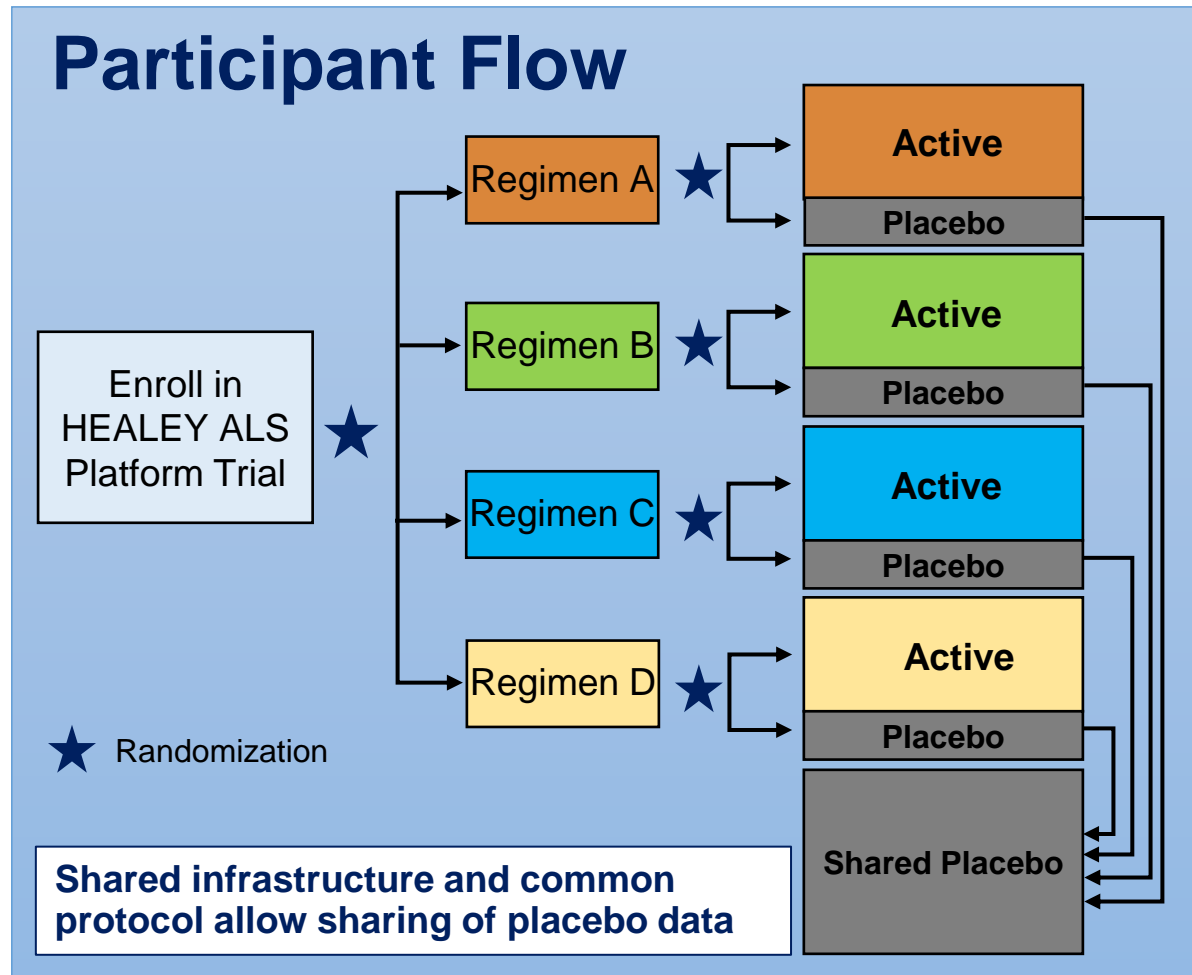
Calico



The AMG Foundation



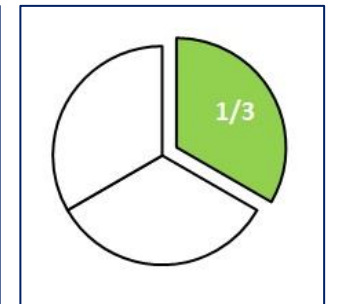
Shared Infrastructure and Master Protocol Allow for Operational and Scientific Efficiencies



Cuts time
in 1/2

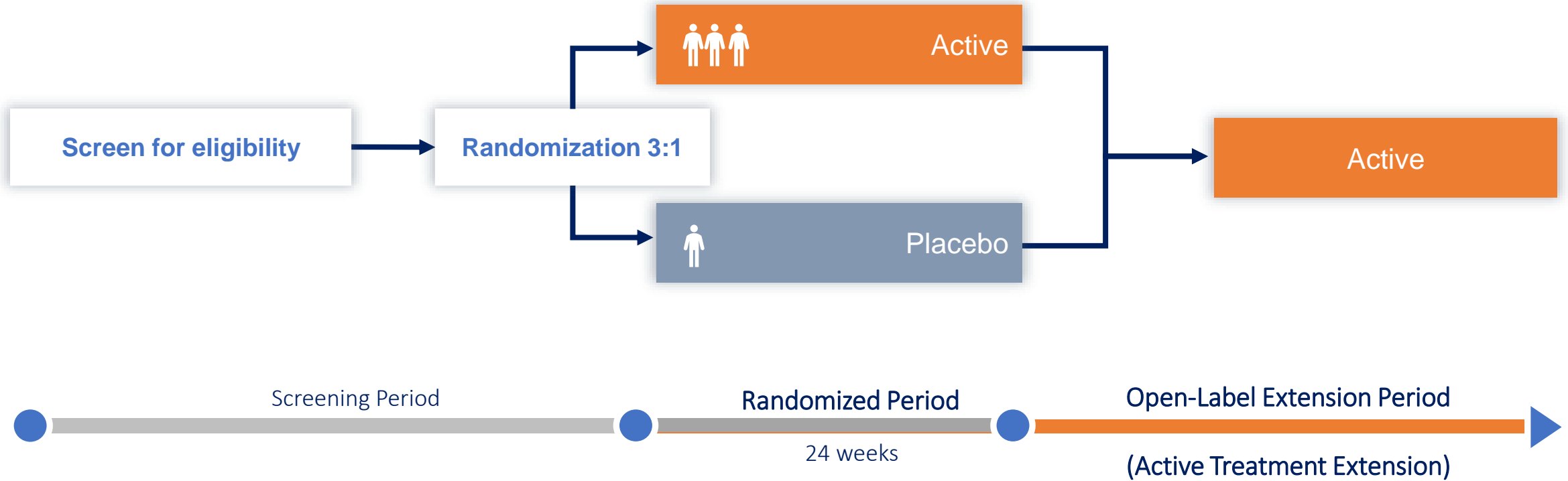


Cuts costs
by 1/3



Reduces
placebo

Schema for Each Regimen





MASTER PROTOCOL

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



ENROLLING

IN START-UP

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!



<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!

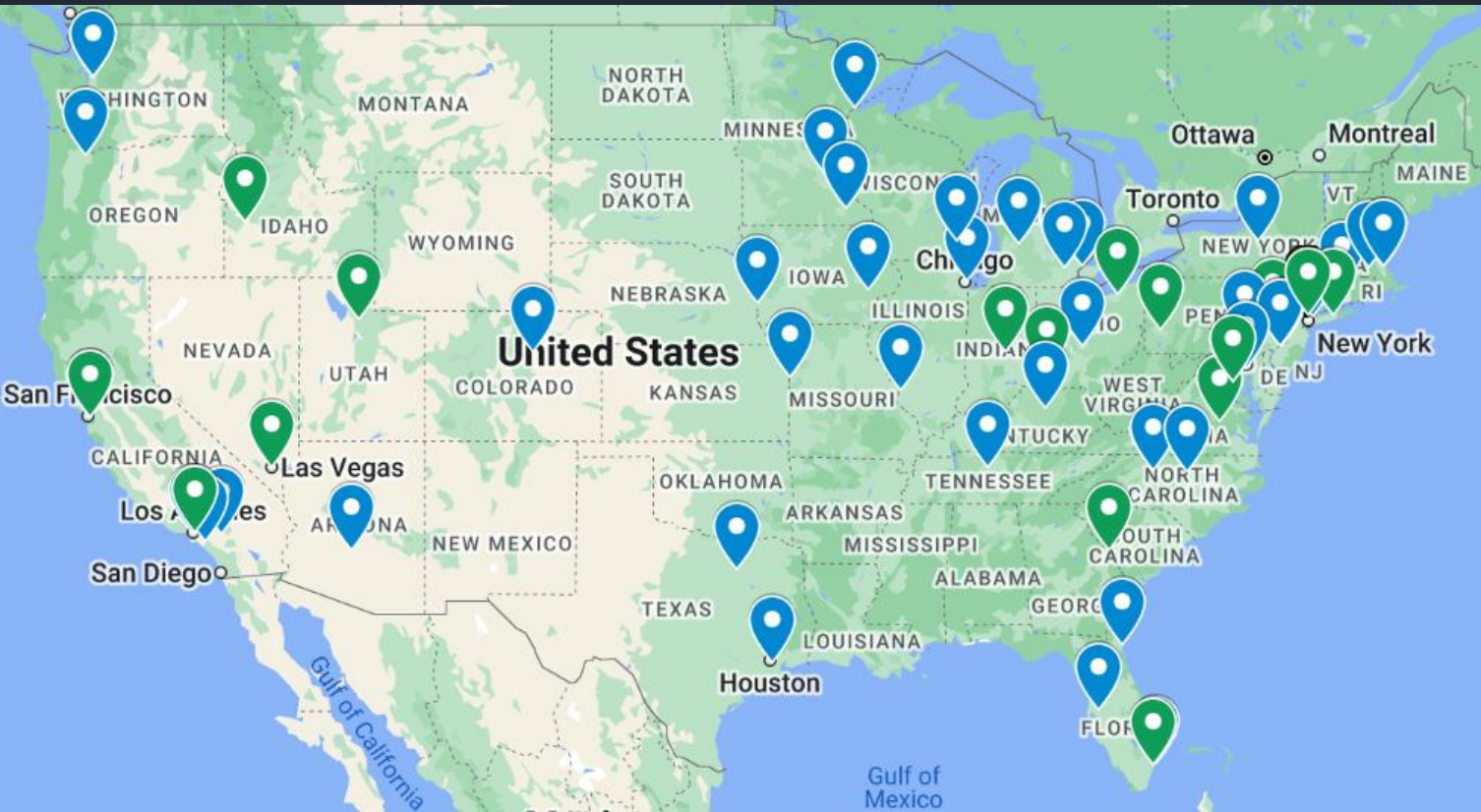


<https://bit.ly/3X2u004>

Recording available under “educational webinars” on neals.org



60 Sites Currently Activated for Regimen E



(as of 1/12/23)

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

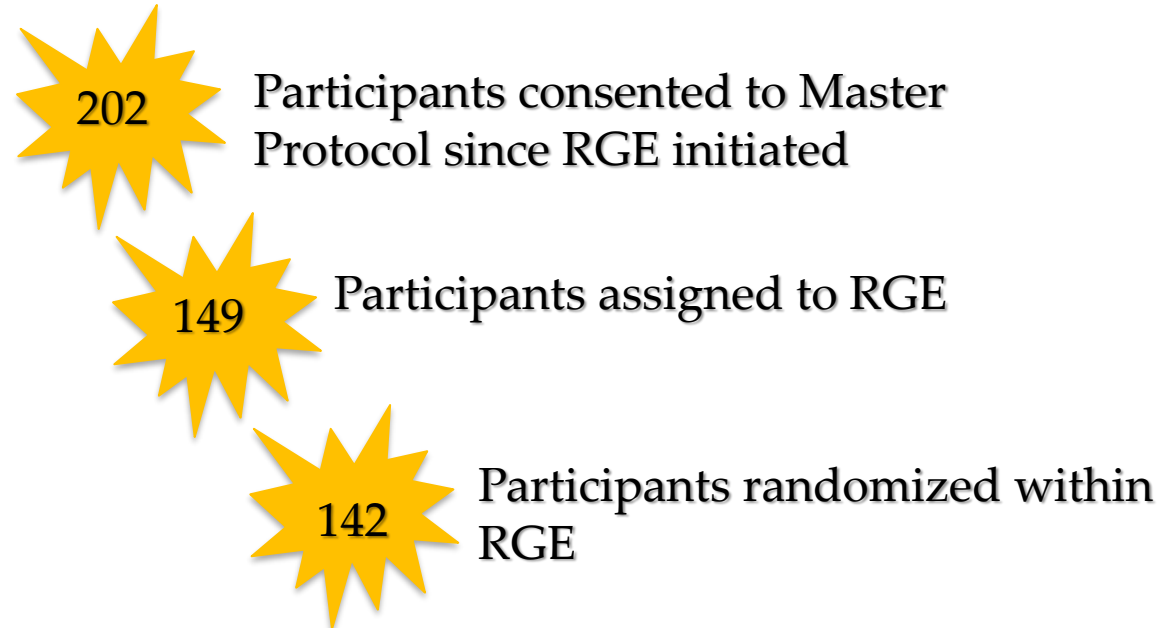
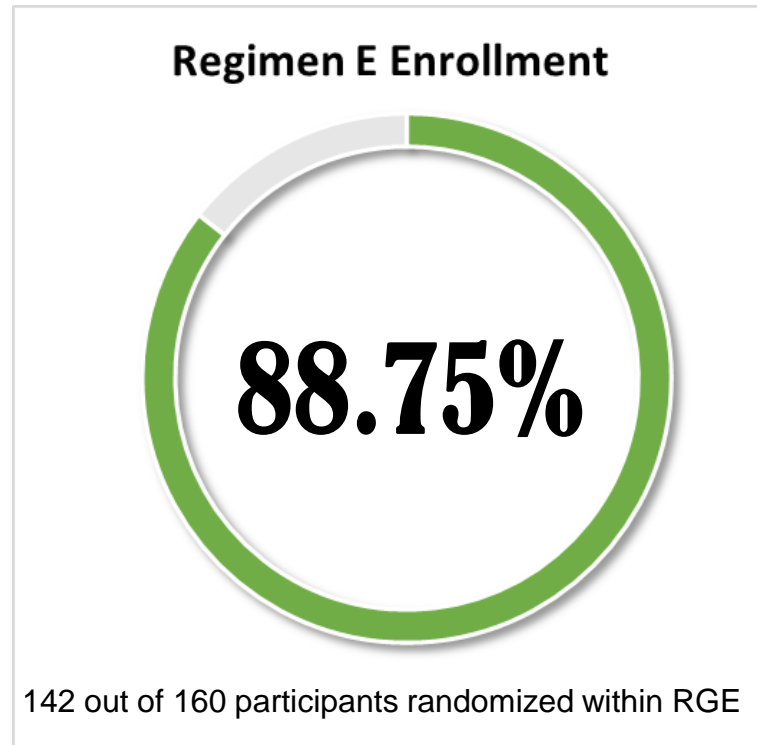
- Lehigh Valley Health Network
- Mass General Hospital
- University of Kansas
- University of Maryland
- California Pacific Medical Center
- Northwestern University
- Virginia Commonwealth University
- University of Nebraska
- Washington University
- Wake Forest University
- Hospital for Special Care
- Saint Alphonus 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 Jan 12, 2023)



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:

January 19- Weekly Q&A

January 26- Jonathan Katz, MD (Site Investigator at CPMC in California)



Allison Bulat

Weekly Recordings Available on MGH Website

Webinar Recordings

Science & Mechanism of Action Series



Weekly & Monthly Updates: 2023



January 5, 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. We looked back at Platform Trial progress since enrollment started in 2020 and discussed upcoming plans for the trial in 2023.

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

Weekly & Monthly Updates: 2022



Weekly & Monthly Updates: 2021



Weekly & Monthly Updates: 2020



Visit the Website:



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

Regimen C Update –

➤ **October 2022: Regimen C top line results announced**

While the primary endpoint was not met, a secondary endpoint analysis of survival demonstrated a significant reduction in risk of death or permanently assisted ventilation when adjusting for baseline risk imbalances in the CNM-Au8 regimen for the 30 mg dose; full study results, including data on biomarkers and exploratory measures, are expected in the coming months



James Berry, MD, MPH

Regimen Co-Lead (Regimen C)
Massachusetts General Hospital



Michael Hotchkin

Chief Development Officer
Clene Nanomedicine, Inc.

- Open Label Extension (OLE) to transition to Clene Expanded Access Program (EAP)
 - Participants in OLE for Regimen C will be prioritized for the Clene EAP
 - During transition from OLE to EAP, participants in Regimen C will have option to transfer to a different Platform Trial site
 - EAP broadens access to CNM-Au8, so it will be available to more people (including those who were not eligible to participate in Platform Trial Regimen C)
 - EAP details, eligibility criteria, site locations, and contact info will be posted on ClinicalTrials.gov
- Based on Phase 2 study results, Clene is preparing for a Phase 3 Trial of CNM-Au8
 - Survival Data from RESCUE-ALS Trial: press release 9/21/2022- <https://bit.ly/3QwMWkF>
 - Topline results from Platform Trial Regimen C: press release 10/3/2022- <https://bit.ly/3Zx4aCj>
 - For more news from Clene, visit: <https://bit.ly/3izshQo>