Schema for Each Regimen

- **Screen for eligibility**
  - **Randomization 3:1**
    - **Placebo**
    - **Active**
  - **Randomized Period**: 24 weeks
  - **Open-Label Extension Period** (Active Treatment Extension)
Regimen G
Regimen C
Regimen E
Regimen F
Regimen A
Regimen B
Regimen D
Regimen E
Regimen F
Regimen G

2023

MASTER PROTOCOL

ENROLLING
IN START-UP
IN DESIGN
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!
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

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

(as of 2/2/23)

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

https://bit.ly/3g2NZr5
Regimen E Updates (as of Feb 2, 2023)

- 223 Participants consented to Master Protocol since RGE initiated
- 165 Participants assigned to RGE
- 153 Participants randomized within RGE

153 out of 160 participants randomized within RGE
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)
Patient Navigation
Central resource for people living with ALS

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:
February 9th, 16th - Weekly Q&A
February 23rd - Jonathan Katz, MD (Site Investigator at CPMC in California)