HEALEY ALS Platform Trial

Weekly Q&A – Jan 12, 2023

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

Northeast Amyotrophic Lateral Sclerotic Consortium

Barrow Neurological Institute

Berry Consultants

The Arthur M. Blank Family Foundation

AMG
The AMG Foundation
Shared Infrastructure and Master Protocol Allow for Operational and Scientific Efficiencies

- Cuts time in 1/2
- Reduces placebo
- Cuts costs by 1/3

Enroll in HEALEY ALS Platform Trial

![Participant Flow Diagram]

Shared infrastructure and common protocol allow sharing of placebo data

- Regimen A
- Regimen B
- Regimen C
- Regimen D

- Active
- Placebo
- Active
- Placebo
- Active
- Placebo
- Shared Placebo

Randomization
Schema for Each Regimen

- Screen for eligibility
- Randomization 3:1
  - Active
  - Placebo
- Randomized Period: 24 weeks
  - Open-Label Extension Period (Active Treatment Extension)
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

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!
Regimen E Updates (as of Jan 12, 2023)

- 202 participants consented to Master Protocol since RGE initiated
- 149 participants assigned to RGE
- 142 participants randomized within RGE

142 out of 160 participants randomized within RGE
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:
January 19- Weekly Q&A
January 26- Jonathan Katz, MD (Site Investigator at CPMC in California)
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/3g4kzf
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.
Regimen C Update –

➢ 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
  ➢ For more news from Clene, visit: https://bit.ly/3izshQo