HEALEY ALS Platform Trial

Weekly Q&A – Jan 19, 2023

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

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

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

Shared infrastructure and common protocol allow sharing of placebo data

**Randomization**
Schema for Each Regimen

Screening Period

Randomization 3:1

Placebo

Active

Active

Randomized Period

24 weeks

Open-Label Extension Period

(Active Treatment Extension)
Regimen A
Regimen B
Regimen C
Regimen D
Regimen E
Regimen F
Regimen G

MASTER PROTOCOL

2023

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!
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 1/19/23)

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 19, 2023)

- 206 participants consented to Master Protocol since RGE initiated
- 151 participants assigned to RGE
- 144 participants randomized within RGE

Regimen E Enrollment

90%

144 out of 160 participants randomized within RGE
## Weekly & Monthly Updates: 2023

**January 12, 2023: Weekly Q&A and Regimen C Update**
Sabrina Paganoni, MD, PhD presented this week’s updates on the HEALEY ALS Platform Trial and answered questions from the audience. We were joined by guest speakers Michael Hotchkin (Chief Development Officer at Clene Nanomedicine, Inc) and James Barry, MD, MPH (Regimen C co-Lead, Mass General Hospital) to discuss the latest news and updates regarding Regimen C.
[Watch recording](#) | [Download slides (PDF)](#)

**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)](#)

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## Weekly & Monthly Updates: 2022

## Weekly & Monthly Updates: 2021
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 26 - Weekly Q&A
February 2  - Weekly Q&A
February 9 - Weekly Q&A