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

ENROLLMENT COMPLETE
IN START-UP
IN START-UP
Schema for Each Regimen

Screen for eligibility ➔ Randomization 3:1 ➔ Active ➔ Active

Screening Period ➔ Randomized Period

Randomized Period

Open-Label Extension Period

(Active Treatment Extension)
Platform trials are a unique opportunity to advance science

**DNA** – whole genome sequencing

**Neurofilaments** – for all regimens

**Biomarkers** (Blood, Urine, CSF) – several drug-specific biomarkers

**Speech Analysis** – emerging digital biomarker

**Home Spirometry** – critical during the pandemic

Additional biomarkers/outcome measures for upcoming regimens (new patient-reported outcomes; PBMCs for stem cell generation)
Recent RGE Press Release

The HEALEY ALS Platform Trial completes enrollment for Regimen E evaluating SLS-005 (Trehalose) by Seelos Therapeutics

Trehalose marks the fifth regimen to complete trial enrollment in this innovative design paradigm.

“This milestone is a pivotal step forward in the HEALEY ALS Platform Trial and the fight against ALS. This achievement would not be possible without our partnerships with people living with ALS; the advice and feedback they provide allow us to develop effective ALS treatments.”

Merit Cudkowicz, MD, MSc
Director, Sean M. Healey & AMG Center for ALS, Massachusetts General Hospital

View Press Release: https://bit.ly/3ImH0YM
Regimen F

ABBV-CLS-7262 is being developed by AbbVie Inc and Calico Life Sciences.

- Targets eIF2B, a key regulator of the integrated stress response (ISR). In neurons exposed to cellular stressors, inhibition of the ISR by ABBV-CLS-7262 restores protein synthesis and dissolves pre-formed TDP-43 containing stress granules.
- TDP-43 containing stress granules are thought to lead to TDP-43 inclusions, a hallmark of ALS pathology.

https://bit.ly/3WStSPz
Regimen G

**PRESS RELEASE - DEC | 5 | 2022**

Healey & AMG Center for ALS announces new drug regimen for testing DNL343 in HEALEY ALS Platform Trial

- DNL343 is being developed by Denali Therapeutics.
- Targets eIF2B, a key regulator of the integrated stress response, to restore protein synthesis and dissolve pre-formed TDP-43 containing stress granules.

“By adding one more drug to the platform, we continue to **push research forward** in hopes of soon finding many more effective treatments for ALS.”

Merit Cudkowicz, MD, MSc

Director, Sean M. Healey & AMG Center for ALS, Massachusetts General Hospital

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 Hotchkine (Chief Development Officer at Cline 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](https://bit.ly/3g4kzfv) | [Download slides](https://example.com/soundofthenight213.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](https://bit.ly/3g4kzfv) | [Download slides](https://example.com/soundofthenight213.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: [QR Code]
https://bit.ly/3r6Nd2L

ALS Link sign-up: [QR Code]
https://bit.ly/3o2Ds3m

Upcoming Webinars:
February 23rd- Weekly Q&A
March 2nd- Weekly Q&A
March 9th- Weekly Q&A