

HEALEY ALS Platform Trial

Weekly Q&A – Feb 16, 2023



Healey Center

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



Calico



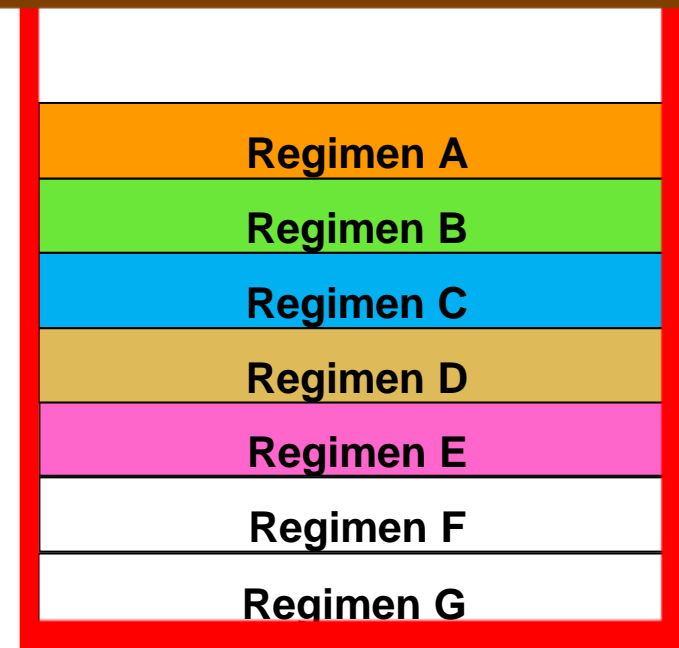
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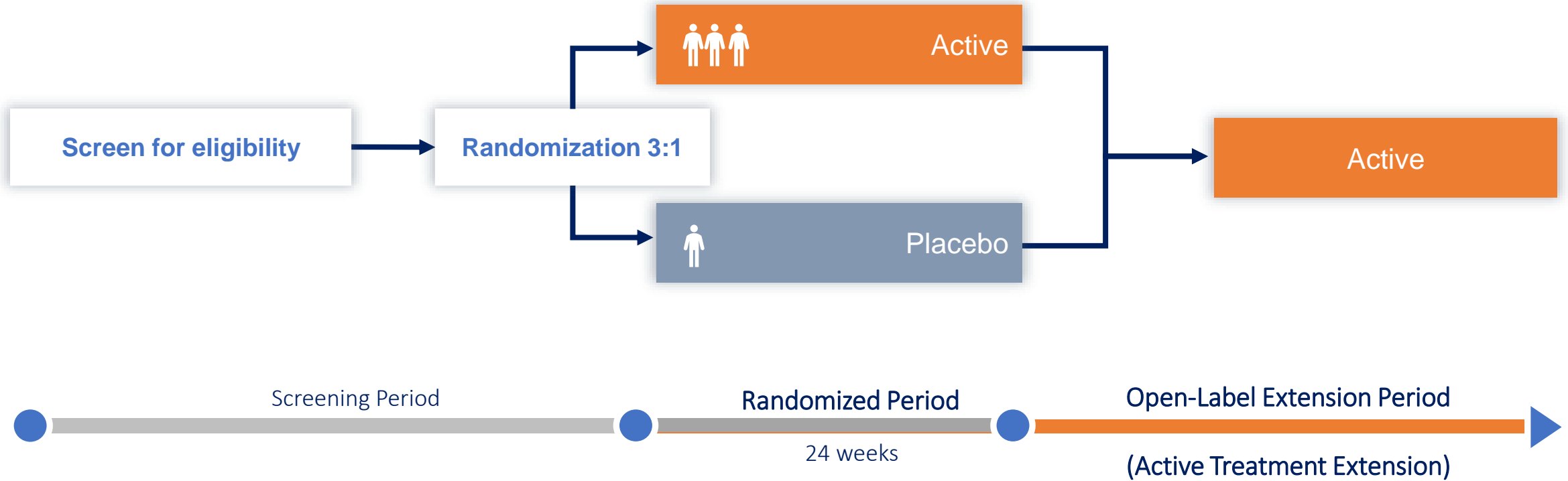
The AMG Foundation



MASTER PROTOCOL



Schema for Each Regimen



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

PRESS RELEASE · 3 MINUTE READ · FEB | 13 | 2023

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:



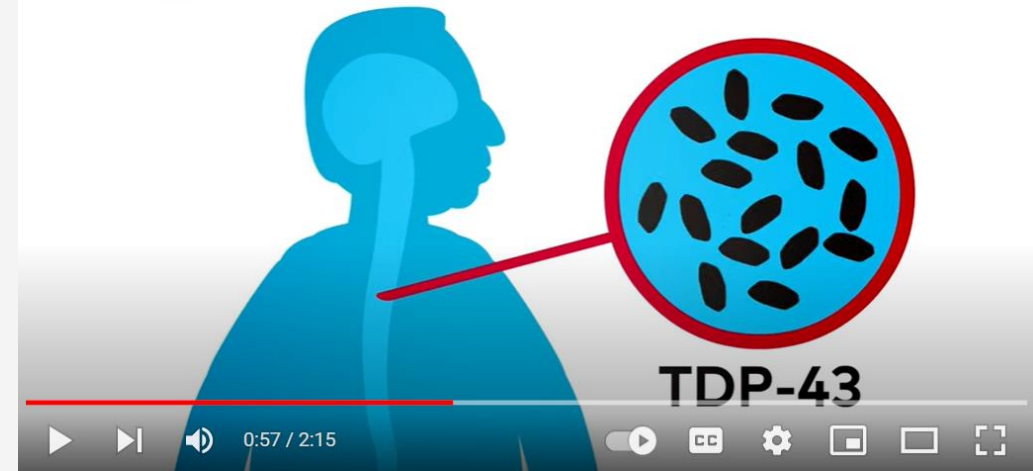
<https://bit.ly/3ImH0YM>

Regimen F

Integrated Stress Response (ISR)

PRESS RELEASE · 5 MINUTE READ · JUL | 12 | 2022

Investigational drug ABBV-CLS-7262 initiates design phase for entry into the HEALEY ALS Platform Trial



- **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.



Calico



<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

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- **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



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

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 \(PDF\).](#)

Visit the Website:



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

Patient Navigation

Central resource for people living with ALS



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Weekly webinar
registration:



<https://bit.ly/3r6Nd2L>

ALS Link sign-up:



<https://bit.ly/3o2Ds3m>



Allison Bulat

Upcoming Webinars:

February 23rd- Weekly Q&A

March 2nd- Weekly Q&A

March 9th- Weekly Q&A