

HEALEY ALS Platform Trial

Weekly Q&A – Feb 23, 2023



Healey Center

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



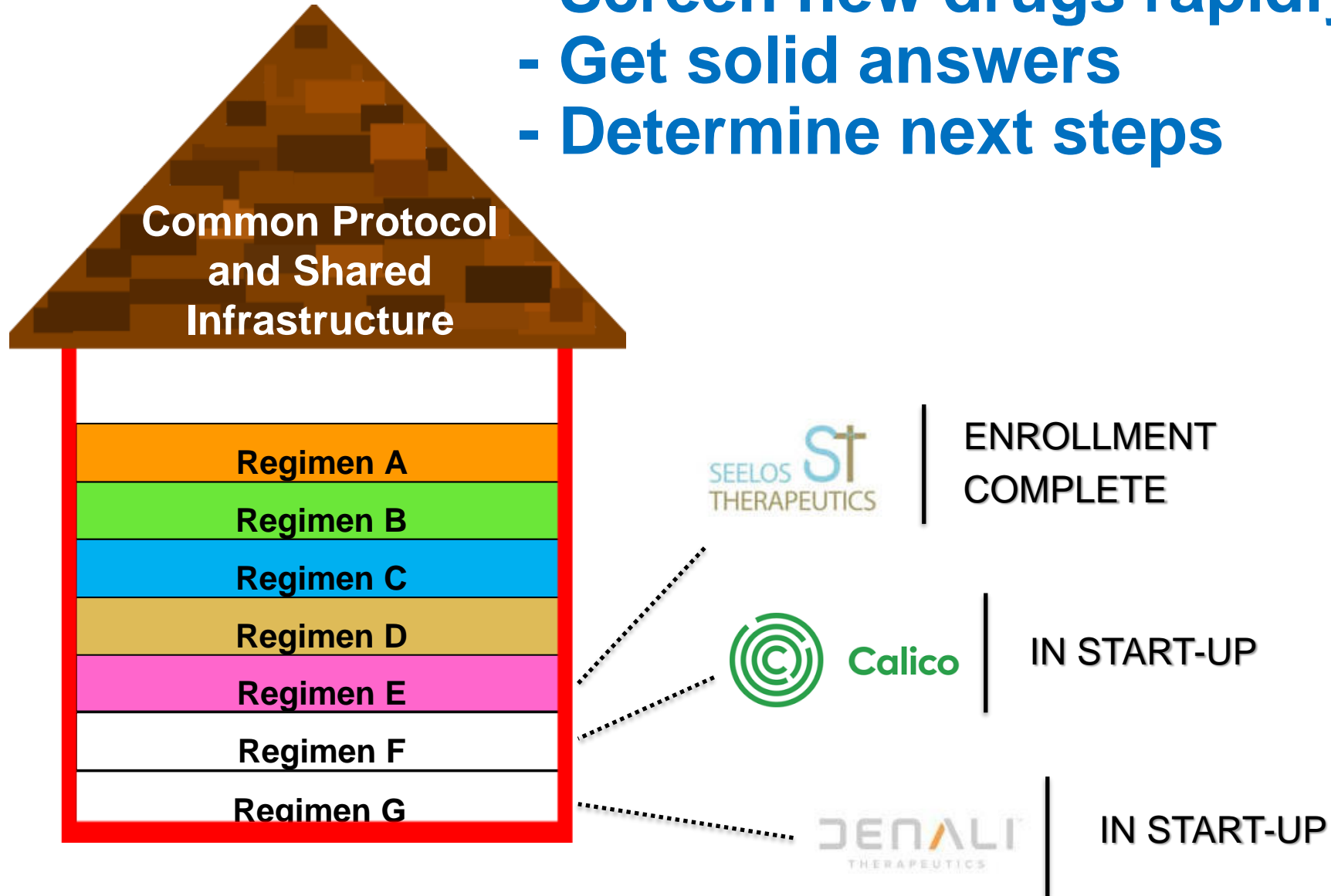
Calico



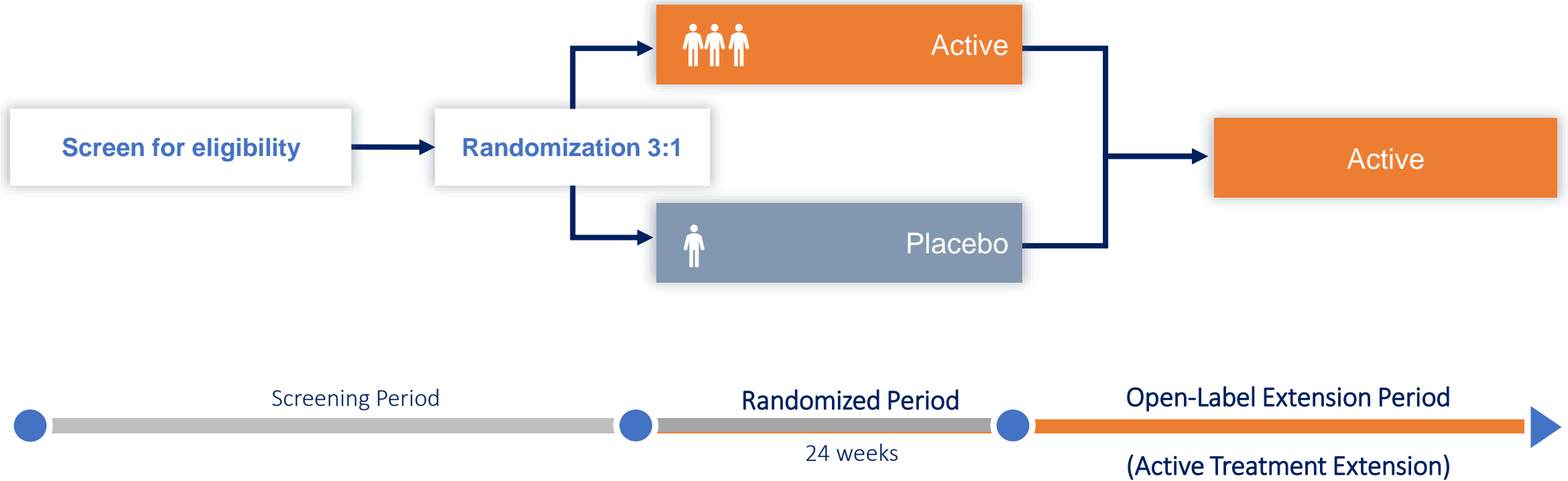
The AMG Foundation

HEALEY ALS Platform Trial Goals:

- Screen new drugs rapidly and efficiently
- Get solid answers
- Determine next steps



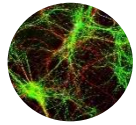
Schema for Each Regimen



The HEALEY ALS Platform Trial is 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 are being considered for upcoming regimens (e.g., new patient-reported outcomes; PBMCs for stem cell generation)

Recent RGD Press Release

PRESS RELEASE · FEB | 23 | 2023

Healey & AMG Center & the Northeast ALS Consortium announce results in platform trial with pridopidine

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We are thankful to the many people who participated in this study and the staff at all the trial sites.

*“The positive results on speech and bulbar function, and on overall function and breathing in people earlier in disease course, are very encouraging and **deserve further investigation** in a phase III trial. This was a successful phase II trial where we **learned a tremendous amount** that will inform the follow-on clinical trials of pridopidine in ALS.”*

Merit Cudkowicz, MD, MSc

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

View Press Release:



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

Upcoming Webinars about Regimen D



Webinar for Participants in Regimen D

Monday, February 27th

Platform Trial sites will communicate details to their participants.



Community Webinar (Open to Public)

Monday, March 6th at 10:00 am Eastern Time

Register here: <https://bit.ly/3KQ3q6L>



Patient Navigation

Central resource for people living with ALS



Catherine Small

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:

March 2nd- Weekly Q&A

March 9th- Weekly Q&A



Allison Bulat