

Thank you for joining the weekly webinar, **Happy New Year!**
We are admitting audience members from the waiting room.
Please allow a few moments for the webinar to begin.



HEALEY ALS Platform Trial

Weekly Q&A – January 4, 2024



Healey & AMG Center

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



The AMG Foundation

SOMETHING
NEW



<https://youtu.be/AkUVI-KsUfU?si=iCpCNc0MbVU1nQg->



This trial is **dedicated to all people living with ALS**, their families, and friends. Your **partnership** in research is what keeps us filled with passion, dedication, and the commitment to develop new treatments for ALS

This breakthrough trial would not be possible without your participation.

Every research participant, whether on the active drug or placebo, plays a critical role in making the hope of finding a cure for ALS a reality

Thank You

Common Protocol and Shared Infrastructure Allow for Operational and Scientific Efficiencies

**COMMON
PROTOCOL**



Regimen G

Regimen F

Regimen E

Regimen D

Regimen C

Regimen B

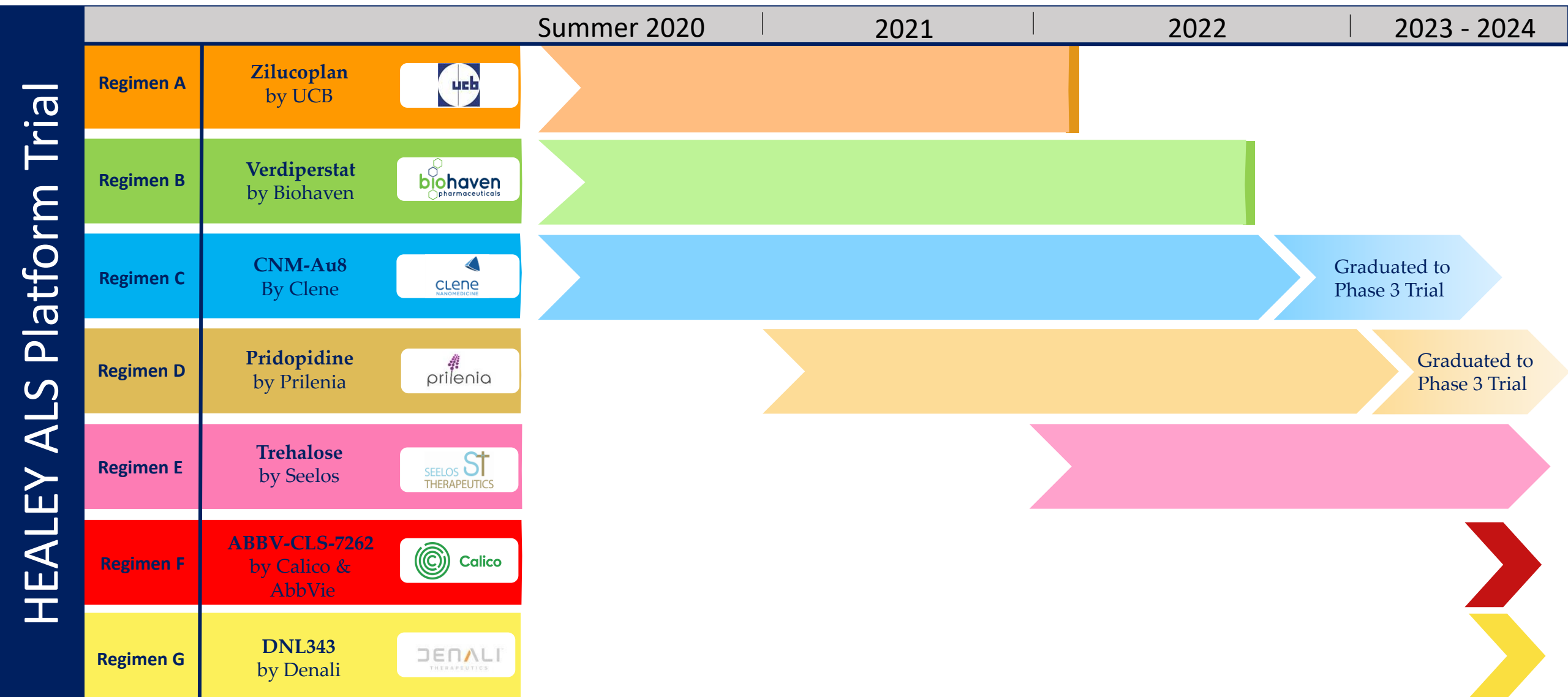
Regimen A

**1 Protocol
(Phase 2/3)
1 single IRB
Central Governance**

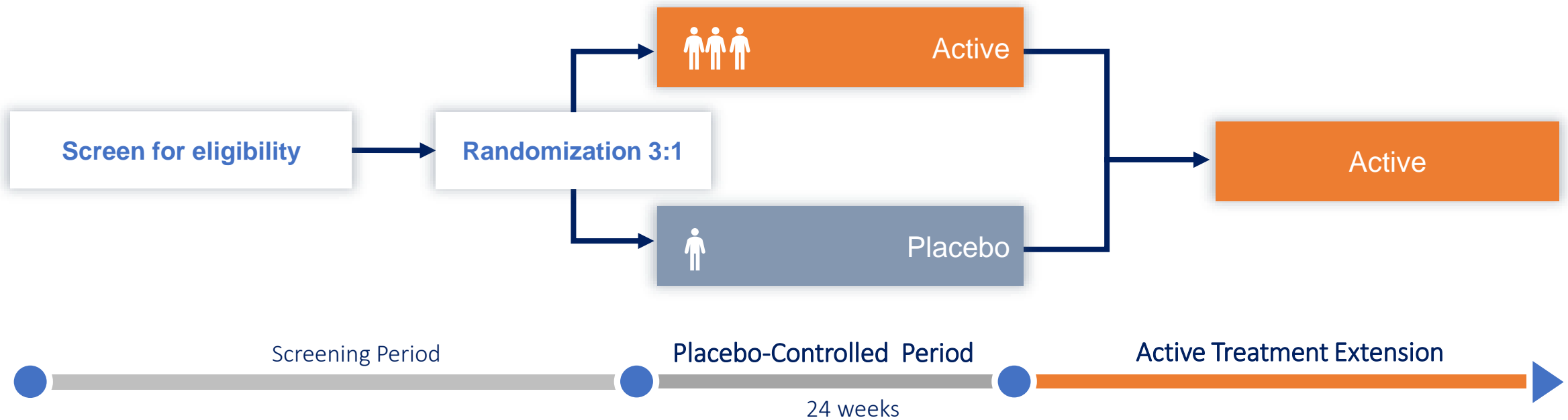
**7 Regimens
70+ Enrolling Sites
~1300 Participants**

Regimen: Active Study Drug + Matching Placebo

The HEALEY ALS Platform Trial is a perpetual trial to provide decisive answers and direction with efficient execution



The objective of each regimen is to provide a go / no go decision to inform the clinical development program of the regimen study drug



Primary Endpoint (Placebo-Controlled Period)

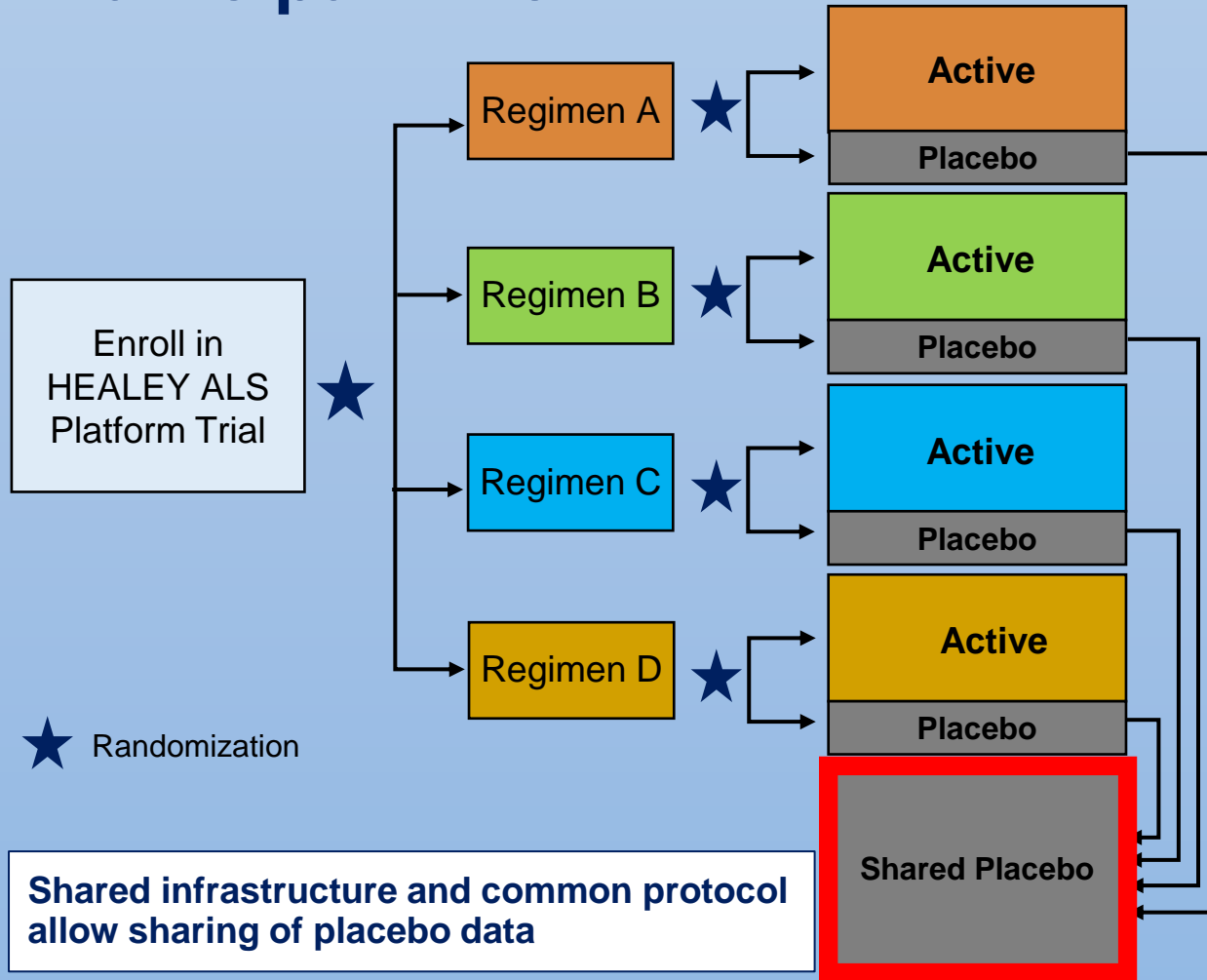
Change from baseline through week 24 in disease severity as measured by the ALSFRS-R total score and survival

Safety, Secondary, and Exploratory Endpoints

(respiratory function, muscle strength, survival, biomarkers + regimen-specific endpoints)

Each regimen is compared to the shared placebo dataset, which keeps growing as new regimens are added

Participant Flow



KEY ELIGIBILITY CRITERIA

Sporadic or familial ALS

(possible, probable, lab-supported probable, or definite by revised EEC)

Time since weakness onset ≤ 3 years

Slow vital capacity $\geq 50\%$ of predicted

Able to swallow

Either not take or be on stable dose of riluzole for ≥ 30 days

Either not take or have completed at least one cycle of edaravone

Either not take or have started Relyvrio/Albrioza ≥ 30 days prior to screening

The platform trial is a unique opportunity to move ALS biomarkers and new outcome measures forward



DNA – whole genome sequencing



Neurofilaments – for all regimens + regimen-specific biomarkers based on MOA



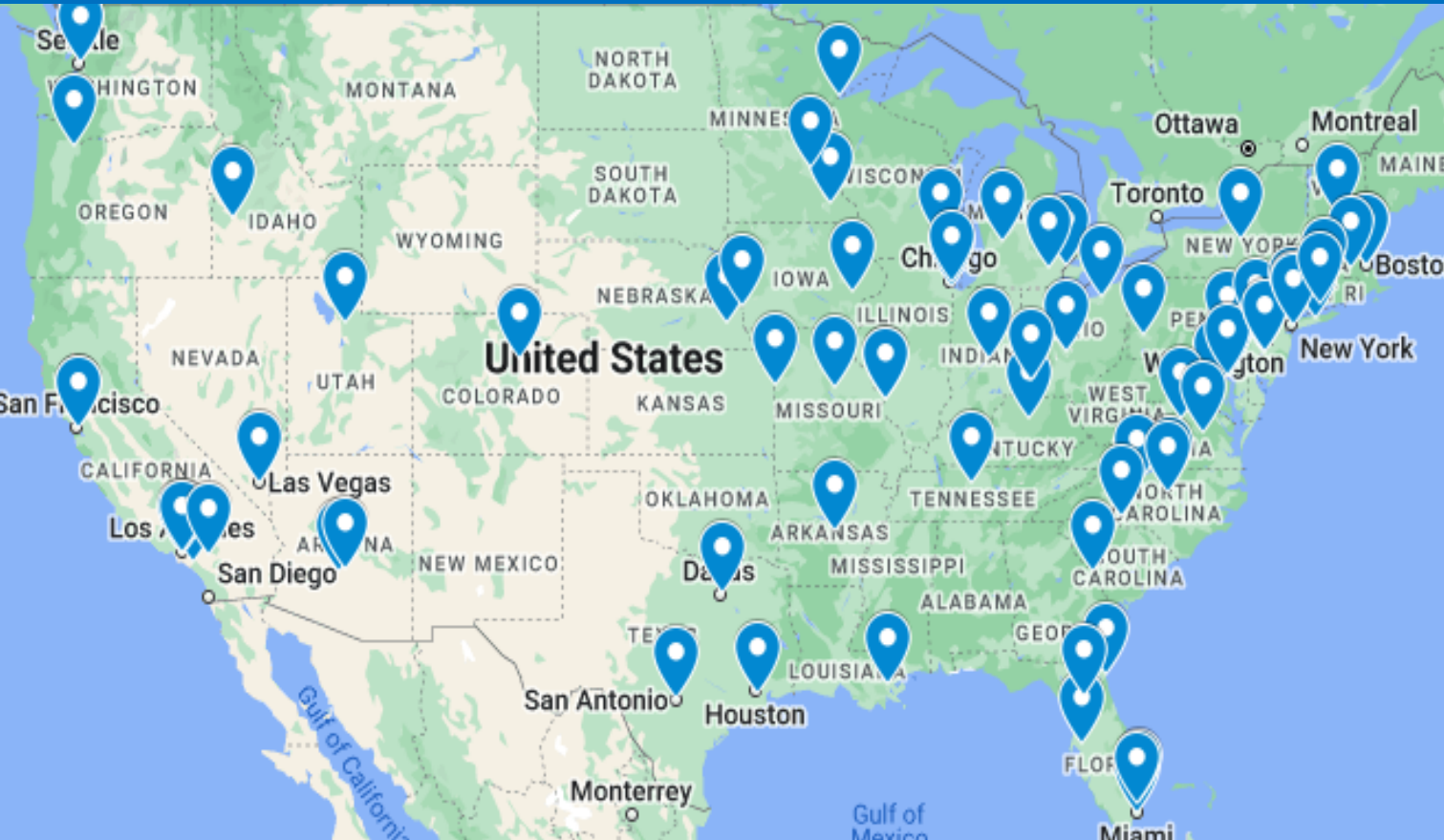
Home Spirometry – critical during the pandemic



Speech Analysis – emerging digital biomarker

Additional biomarkers/outcome measures considered for upcoming and future regimens (e.g., new patient-reported outcomes- ROADS; PBMCs for stem cell generation)

Providing research access across a diverse network of 70+ NEALS sites

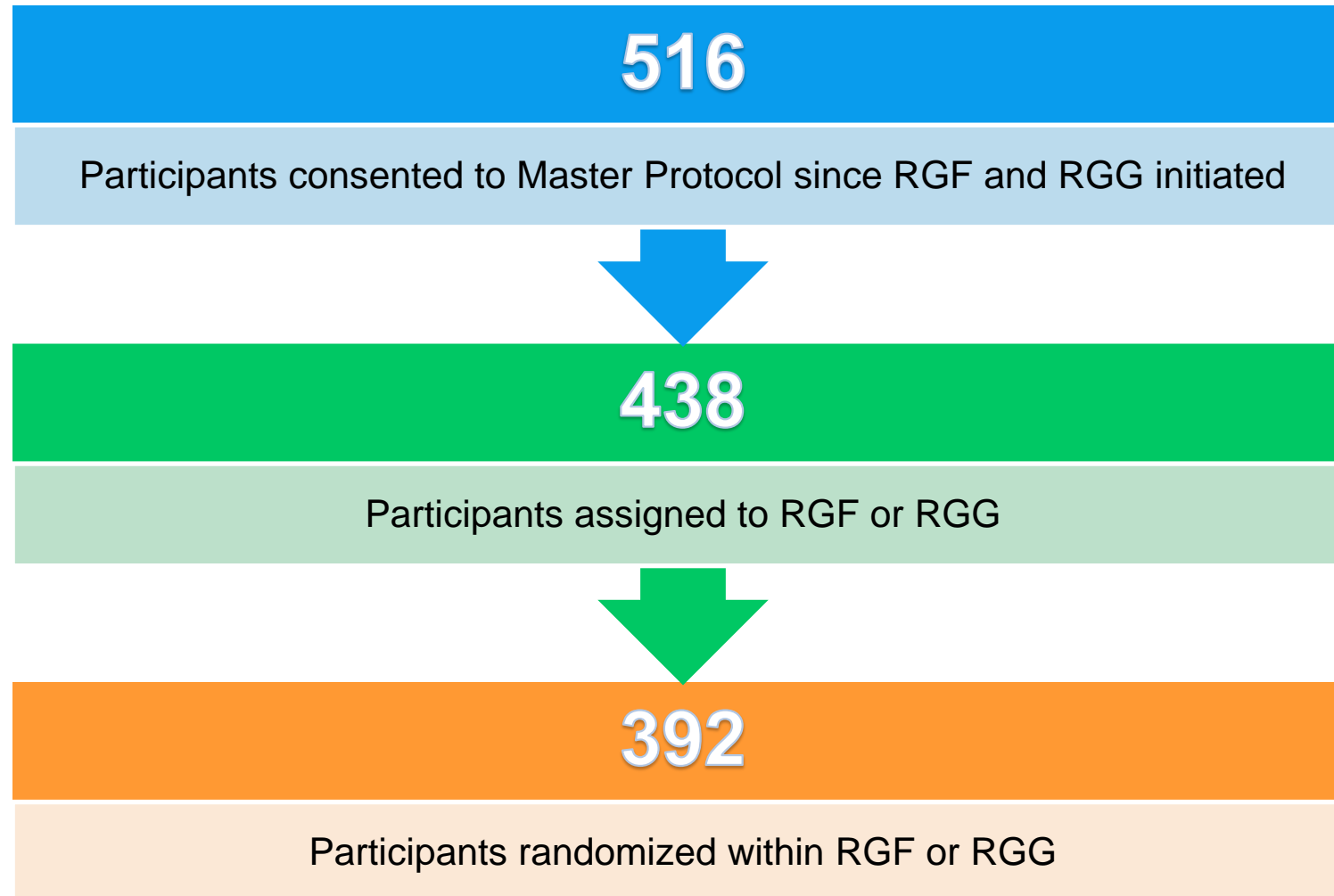


Contact a study team near you to discuss enrollment opportunities



<https://bit.ly/3g2NZr5>

Enrollment Update: Regimen F and Regimen G



Thank You

for your partnership in ALS research

(as of 1/4/24)

New Year, New Webinars, New Format!

Tune in for updates about Expanded Access and EAPs! **on the 2nd Thursday of each month** →



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

HEALEY ALS Platform Trial Q&A Webinars						
SUNDAY	MONDAY	TUESDAY	WEDNESDAY	THURSDAY	FRIDAY	SATURDAY
				Platform Trial Webinar		
				EAP Webinar		
				Platform Trial Webinar		
				Platform Trial Webinar		
				Platform Trial Webinar		

Patient Navigation

Central resource for people living with ALS



Catherine Small

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E-mail: healeyalsplatform@mgh.harvard.edu

Weekly webinar
registration:



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

ALS Link sign-up:



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

Upcoming Webinars:

January 11th- EAP discussion with guest speakers from the NIH

January 18th- Pridopidine (Regimen D) update with Prilenia Therapeutics

January 25th- CNM-Au8 (Regimen C) update with Clene Nanomedicine



Allison Bulat