

Thank you for joining the weekly webinar!

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 – November 30, 2023



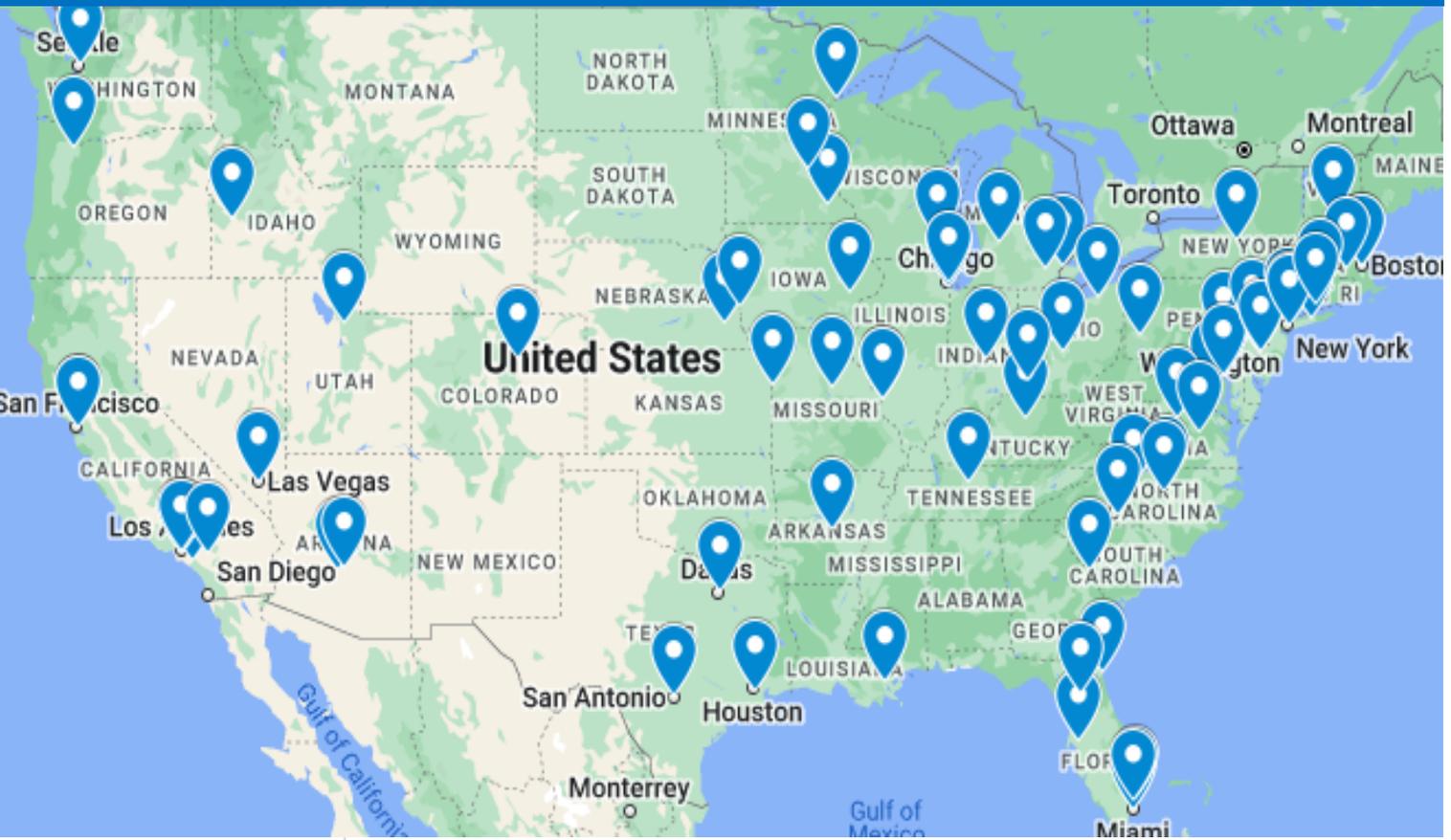
## Healey & AMG Center

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



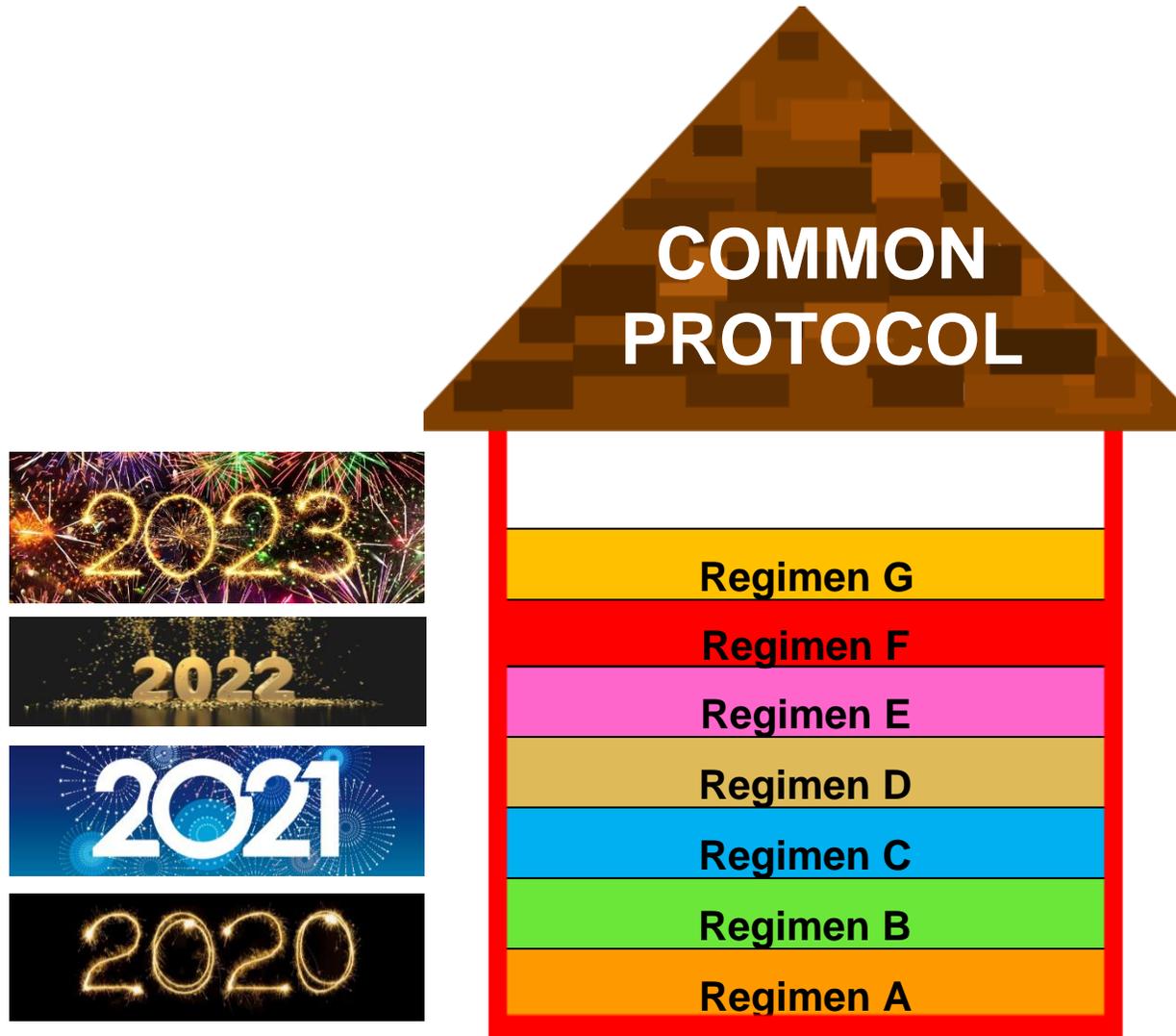
The AMG Foundation

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



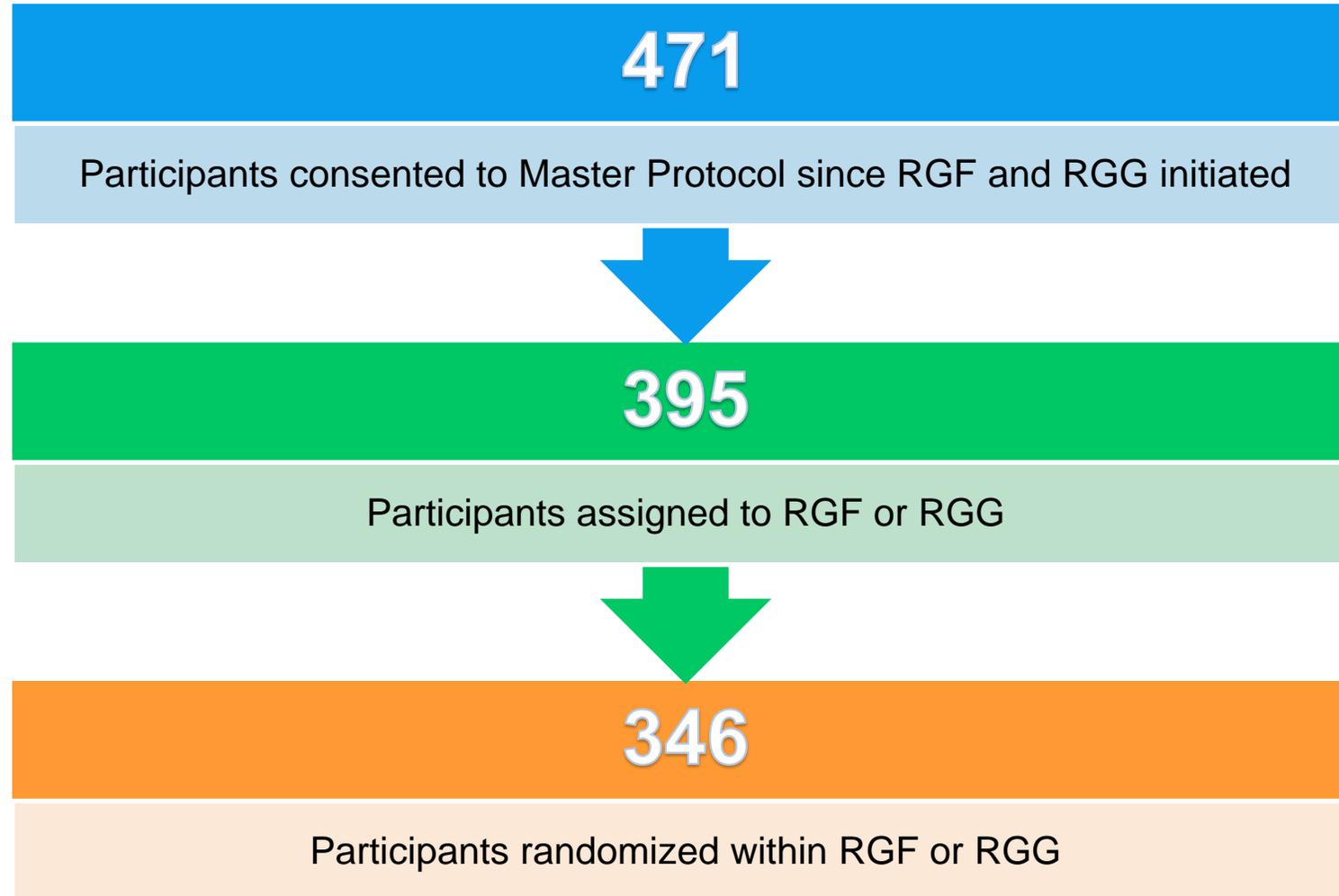
- Texas Neurology
- Mass General Hospital
- UTHSCSA
- Hospital for Special Care
- Holy Cross Hospital
- Thomas Jefferson
- Houston Methodist
- Henry Ford Health System
- Barrow Neurological Institute
- Ohio State University
- Northwestern University
- University of Chicago
- Wake Forest
- University of Nebraska
- Loma Linda University
- University of Washington
- University of Iowa
- Washington University
- University of Pennsylvania
- University of Michigan
- California Pacific Medical Center
- Penn State Hershey
- UMass Worcester
- University of Miami
- University of Colorado
- Cedars-Sinai
- University of Florida
- University of South Florida
- Columbia University
- University of Virginia
- University of Maryland
- SUNY Upstate
- Beth Israel Deaconess
- Temple University
- Dartmouth-Hitchcock
- Medical College of Wisconsin
- Spectrum Health
- University of Missouri
- University of Minnesota
- Johns Hopkins University
- University of CA Irvine
- University of Kansas
- Vanderbilt University
- University of Kentucky
- Mayo Rochester
- Duke University
- Neurology Associates
- Ochsner Health System
- Mayo Clinic Florida
- St. Louis University
- Providence Brain and Spine
- Georgetown University
- University of Southern California
- Cleveland Clinic
- George Washington University
- University of California, San Francisco
- Indiana University
- Stony Brook University
- University of Pittsburgh
- University of Utah
- Augusta University
- University of Cincinnati
- Virginia Commonwealth University
- Swedish Medical Center
- Las Vegas Clinic
- Kaiser, Los Angeles
- Lehigh Valley Health Network
- St. Alphonsus Regional Medical Center
- Hackensack University
- Essentia Health
- Nova Southeastern University
- Dent Neurologic Institute
- Mayo Clinic Scottsdale
- Mount Sinai Beth Israel
- Atrium Health
- Yale University
- University of Arkansas
- University of North Carolina

# Common Protocol and Shared Infrastructure Allow for Operational and Scientific Efficiencies



Regimen: Active Study Drug + Matching Placebo

# Enrollment Update: Regimen F and Regimen G

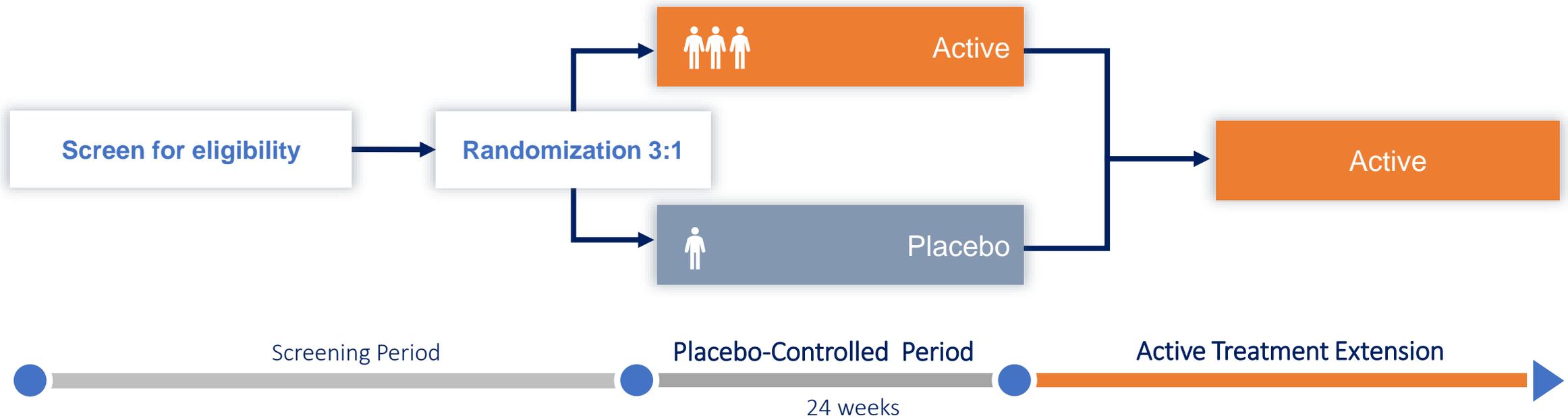


*Thank You*

for your partnership in ALS research

(as of 11/30/23)

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



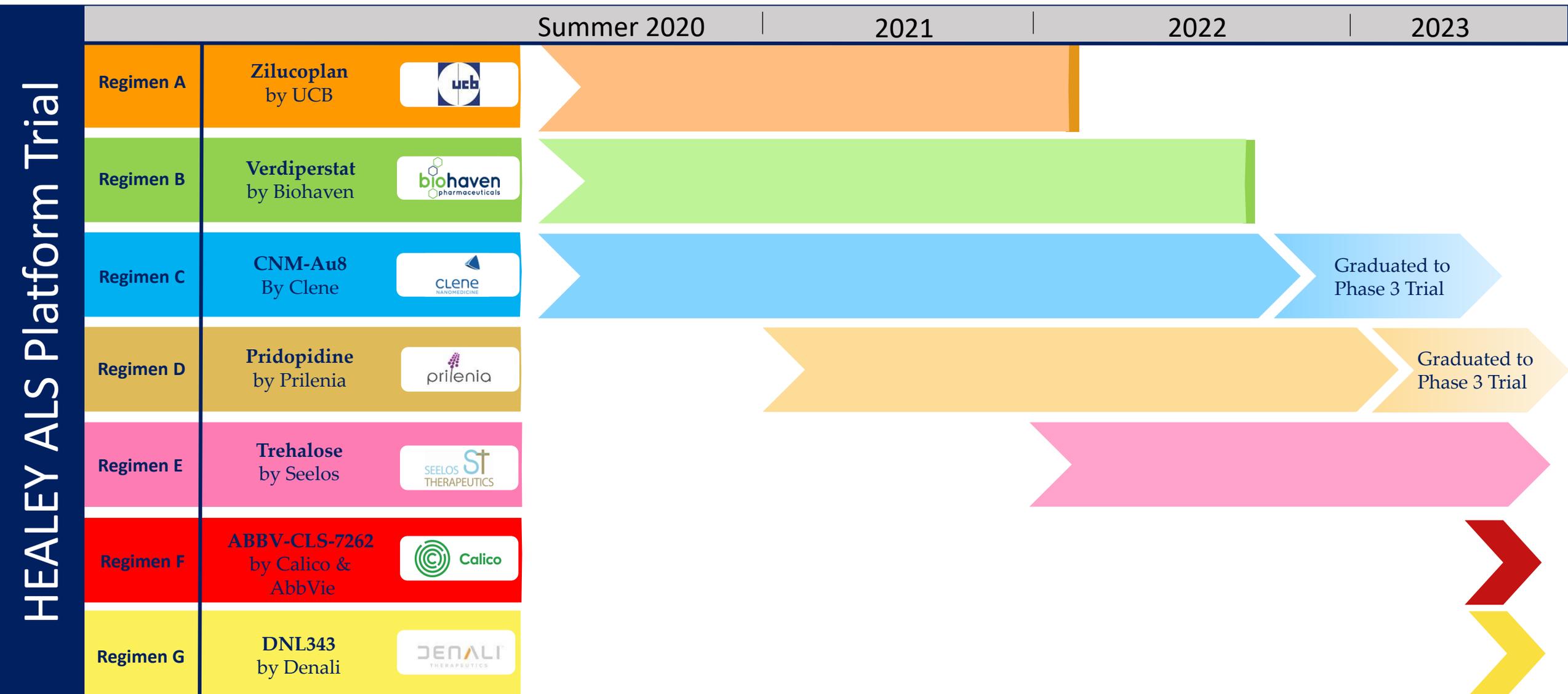
### Primary Endpoint (Placebo-Controlled Period)

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

### Safety, Secondary, and Exploratory Endpoints

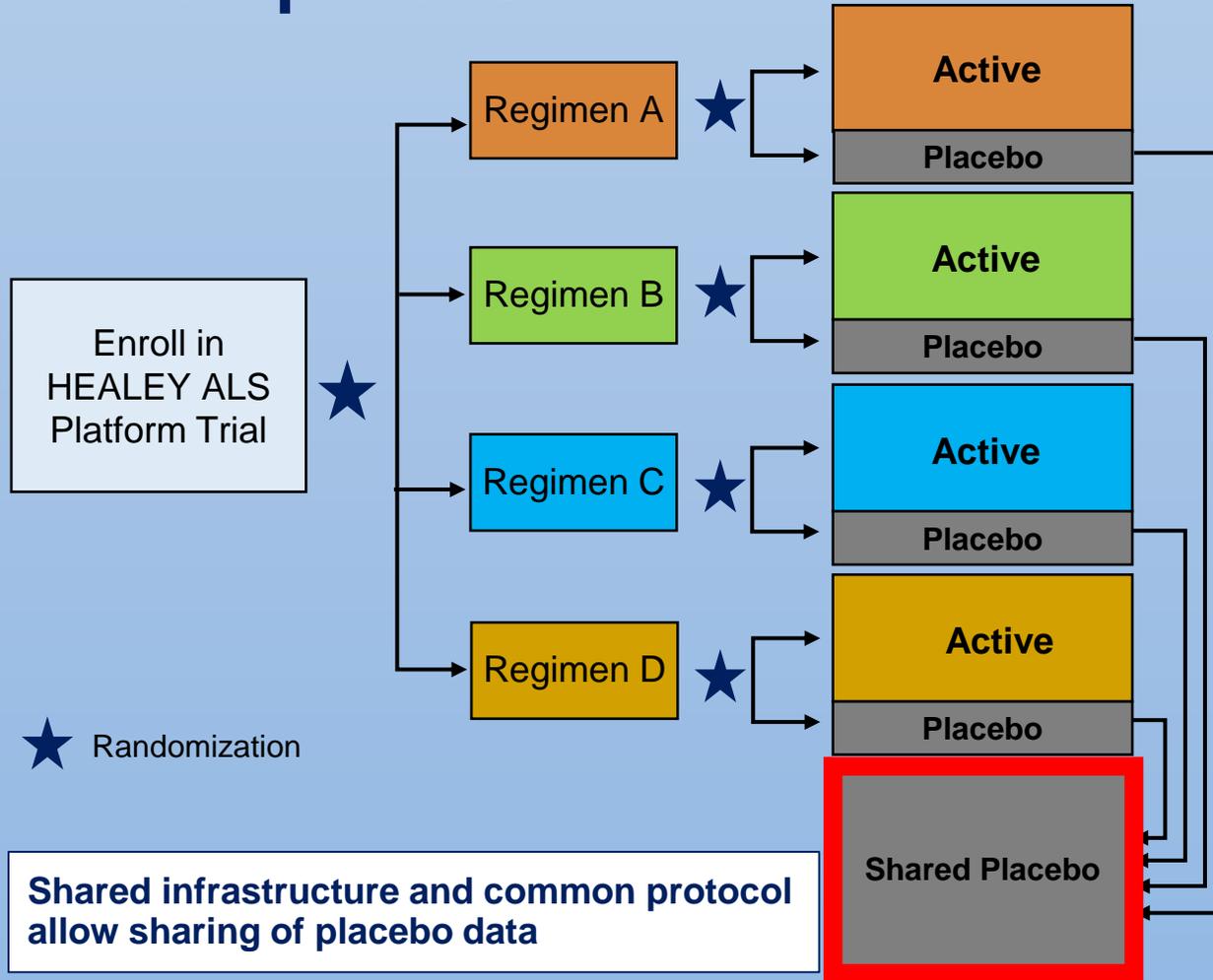
(respiratory function, muscle strength, survival, biomarkers + regimen-specific endpoints)

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



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  $\leq 3$  years

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

Able to swallow

Either not take or be on stable dose of riluzole for  $\geq 30$  days

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

Either not take or have started Relyvrio/Albrioza  $\geq 30$  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)



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*

# Patient Navigation

## Central resource for people living with ALS



Catherine Small

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E-mail: [healeyalsplatform@mgh.harvard.edu](mailto:healeyalsplatform@mgh.harvard.edu)

Weekly webinar  
registration:



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

ALS Link sign-up:



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

### Upcoming Webinars:

No webinar on December 7th- Cancelled for MND Conference

December 14th- EAP Discussion with Dr. James Berry of Mass General Hospital

No webinar on December 21st or 28th -Happy Holidays!

January 4th- First weekly Q&A webinar of 2024!



Allison Bulat