

Sign up for
ALS Link:



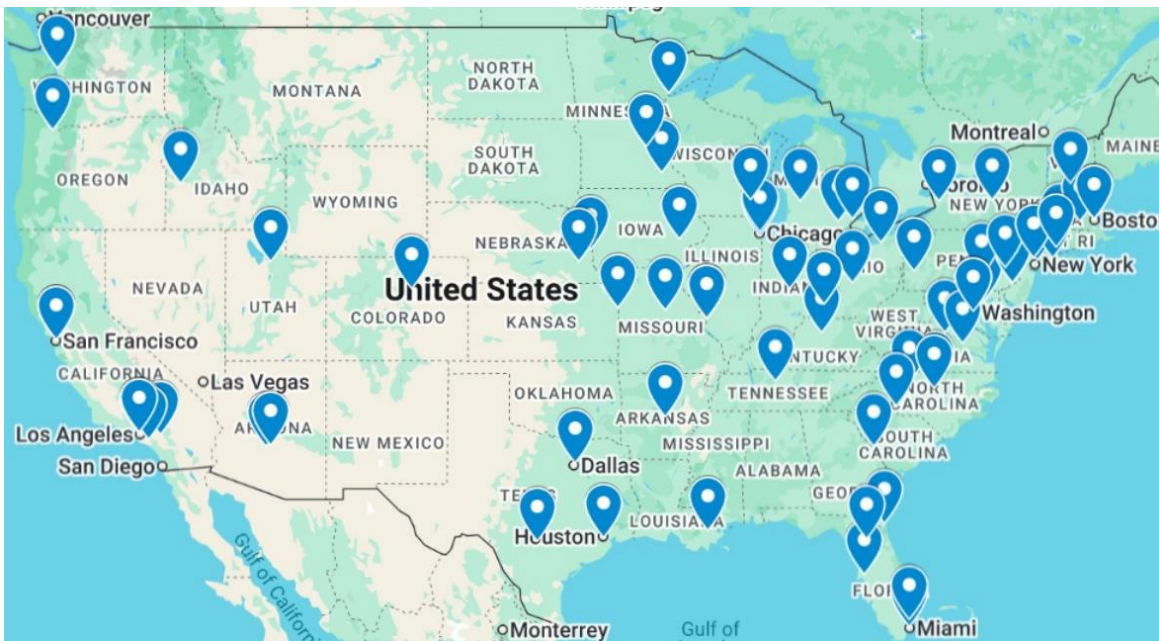
<https://bit.ly/3CqGbhb>

Thank you for joining the webinar!

We are admitting audience members from the waiting room
Please allow a few moments for the webinar to begin



Commemorating 5 Years of the Platform Trial



Study drugs tested: 7
Study drugs that offered Expanded Access: 3



Study participants: 1,373



IRB approved Platform Trial sites to date: 88



Study visits: 25,632



Current study staff nationwide: 946



Biosamples: 271,643
Includes plasma, serum, urine, whole blood, isolated DNA, and CSF



10 Publications

Published in the JAMA and Lancet Families of journals, Nature Aging, Muscle and Nerve and Annals of Neurology



Multiple Publications in the pipeline

Including Regimen F and G results, papers on biostatistics, biomarkers and digital outcomes



48 Poster presentations



78 Oral presentations



Sharing experience with 29 Disease-specific networks



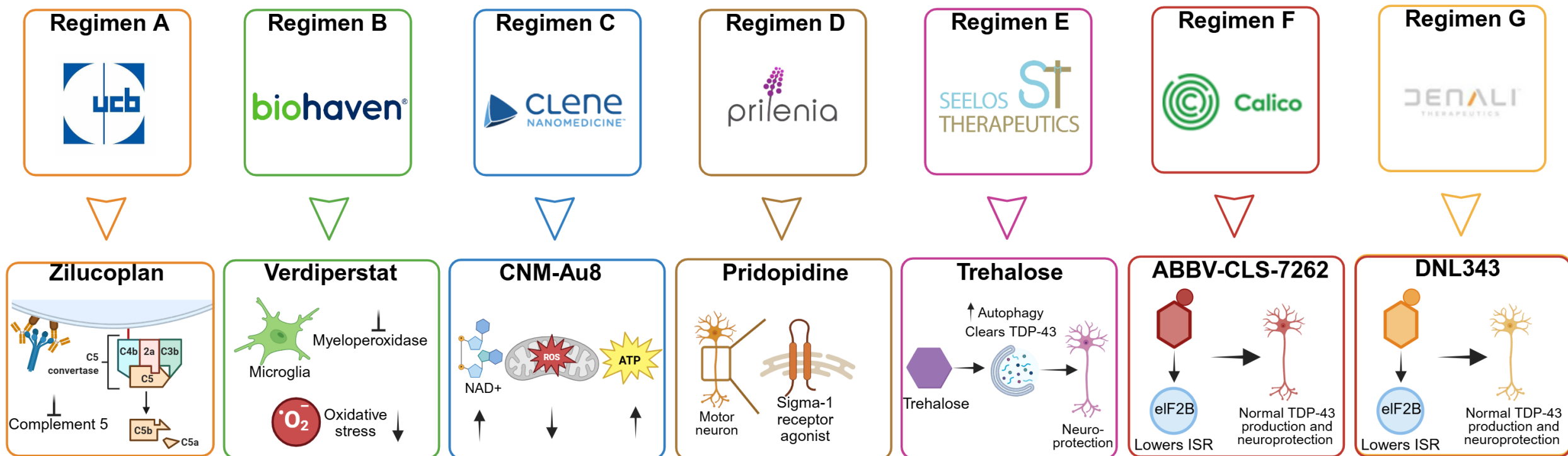
114 Companies we have engaged
52 Companies that applied



~180 Total webinars hosted
~60,000 Total views on YouTube

Mechanisms of Action across Platform Trial Regimens

Moving forward to Phase 3



JAMA family of journals

Lancet Neurology

Manuscripts in preparation

The Trial Learns from its Experience and Adapts

Revised the Master Protocol to:

- Increase statistical power
- Streamline operations
- Add even more patient-centered features

Study Duration

9 months RCT followed by ATE

Inclusion Criteria

2 years since symptoms onset

Visit Schedule

Increased remote visits (patient-centered)

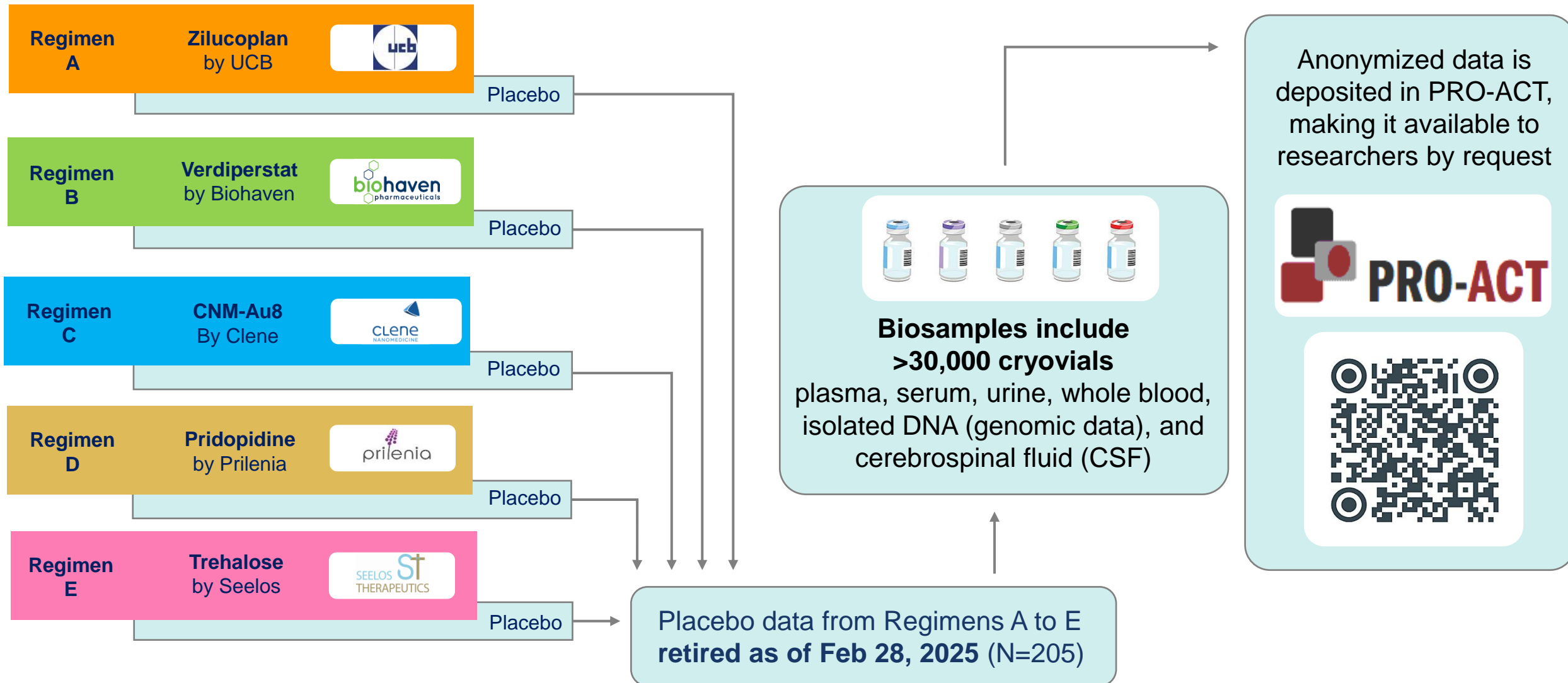
Biomarkers

New biomarkers and PBMCs for stem cells



New regimens will enroll under the revised Master Protocol

Broadening the Impact of Placebo Data from Regimens A-E



Operational Adaptations and Efficiencies – Value for Sites and Trial Participants

Nature Aging

Lessons from the HEALEY Adaptive Platform Trial in Amyotrophic Lateral Sclerosis

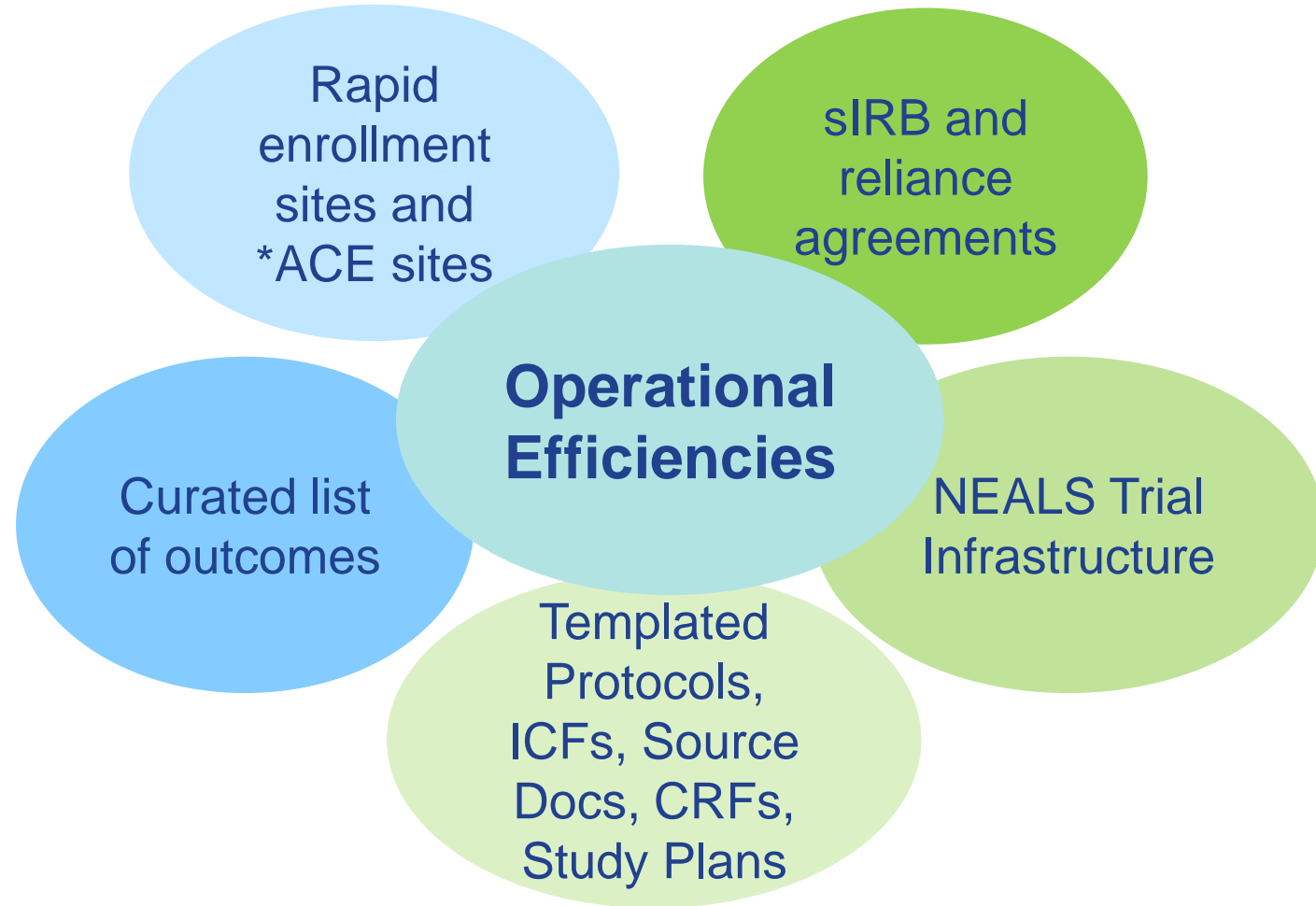
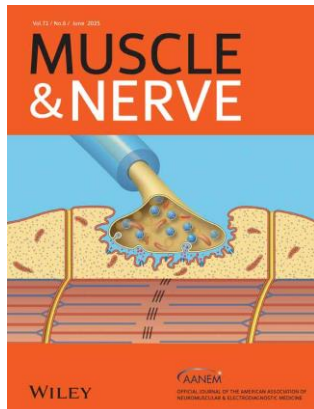
First published: 01 November 2024



Muscle & Nerve

Operational Development and Launch of an Adaptive Platform Trial in Amyotrophic Lateral Sclerosis: Processes and Learnings From the First Four Regimens of the HEALEY ALS Platform Trial

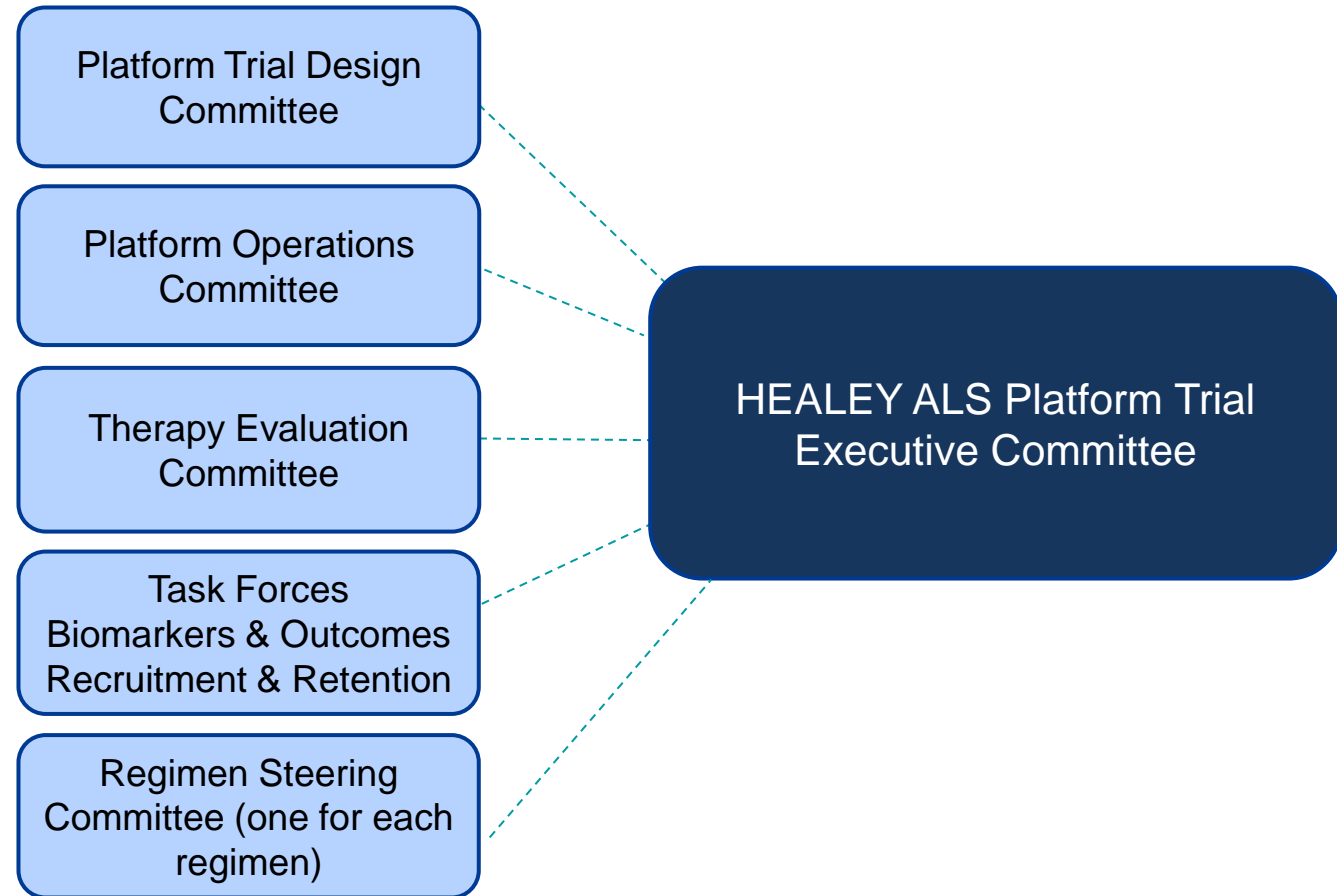
First published: 26 May 2025



*ACE: Acceleration Centers of Enrollment

How Academics can Partner with Us

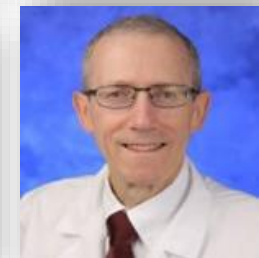
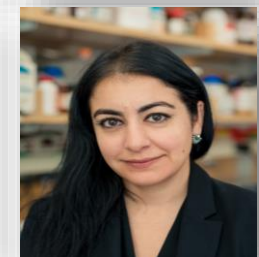
- **Leadership opportunities** are always available (e.g., to become a study co-lead)
- Opportunities to become a **member of a task force and/or a committee** (e.g., steering committee for a new regimen)
- We are always excited to develop **new research opportunities** through collaborative projects and grants!



How Companies can Partner with Us

- Submit a **Therapy Application Form**
- The **Therapy Evaluation Committee** meets monthly or ad hoc as needed for reviews and decision is based on science
- Therapy selection is determined in conjunction with the **Executive Committee** to include financial and operational considerations

Scan for Therapy
Application
Form



Who to Connect With and How

Cudkowicz.merit@mgh.harvard.edu



Merit Cudkowicz

Director, Sean M. Healey & AMG Center for ALS
Executive Director, MGB Neuroscience Institute
Julianne Dorn Professor Neurology, Harvard
Medical School

HEALEY ALS Platform Trial PI

spaganoni@mgh.harvard.edu



Sabrina Paganoni

Robert H. Brown, Jr., PhilD, MD
Endowed Chair in Neurology; Co-Director,
Neurological Clinical Research Institute
Associate Professor, Harvard Medical
School

HEALEY ALS Platform Trial Co-PI

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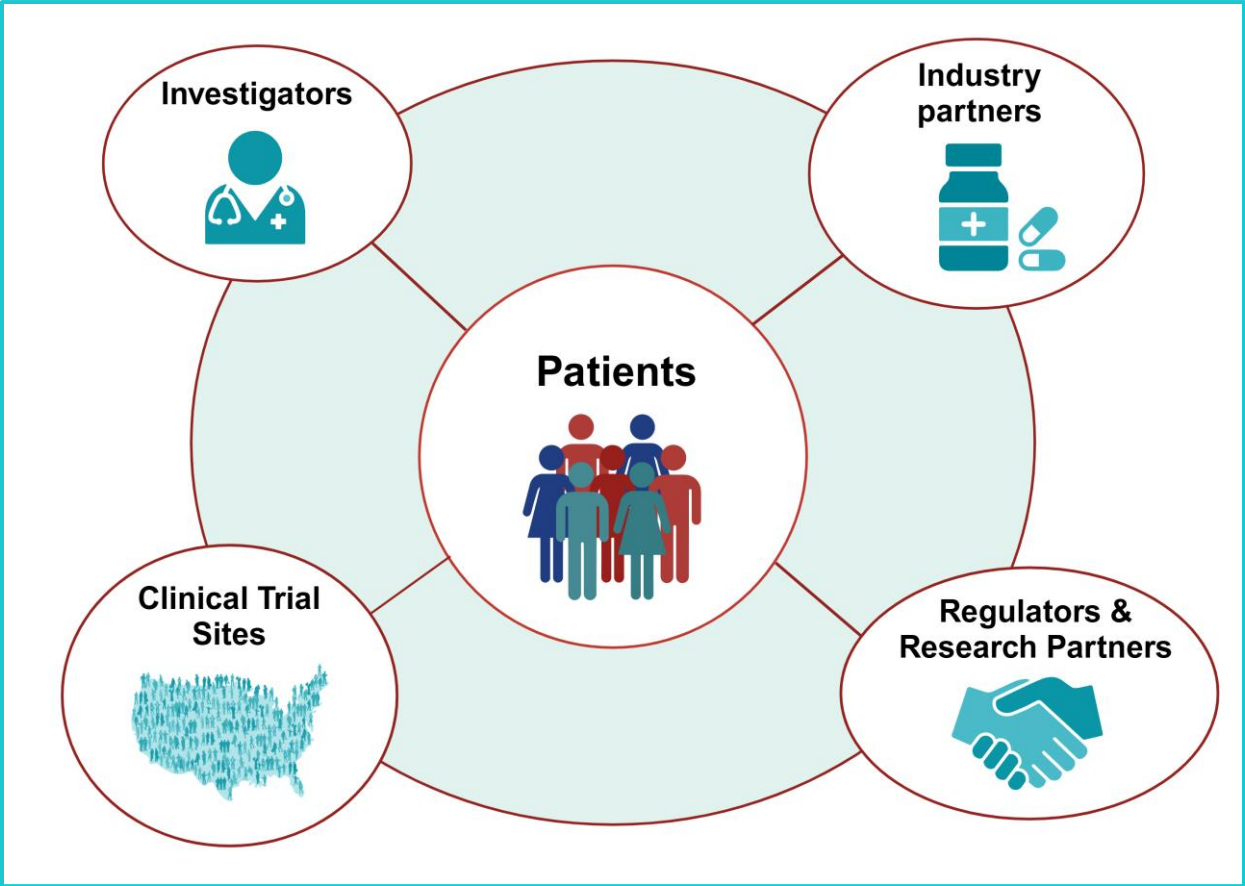


Kristin Drake

Senior Director, Sean M.
Healey & AMG Center for ALS
and Neurological Clinical
Research Institute

We accept applications on a rolling basis

Thankful to You All!



Thank you to all trial participants- progress in ALS would not be possible without your generous contributions

Patient Navigation – Central resource for people living with ALS



Catherine Small
Patient Navigator



Mimi Le
Admin. Assistant

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu

Register for Webinars:

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



205 Webinars to date

72 Attendees on avg.

2,991 Qs answered live

71,099 Total views on YT

Data captured Sept 2020 - Nov 2025

Upcoming Webinars (Thurs, 5:00- 5:30pm Eastern Time)

December 11 – Expanded Access Discussion

December 18 – Platform Trial Discussion

Stay Tuned in 2026!

January 8 – Expanded Access Discussion

January 22 – Research Access Discussion

February 12 – RAPA-501 EAP Update

Thank you for driving conversations about research!

