

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.



HEALEY ALS Platform Trial

Community Q&A – Sept 26, 2024



Healey & AMG Center

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

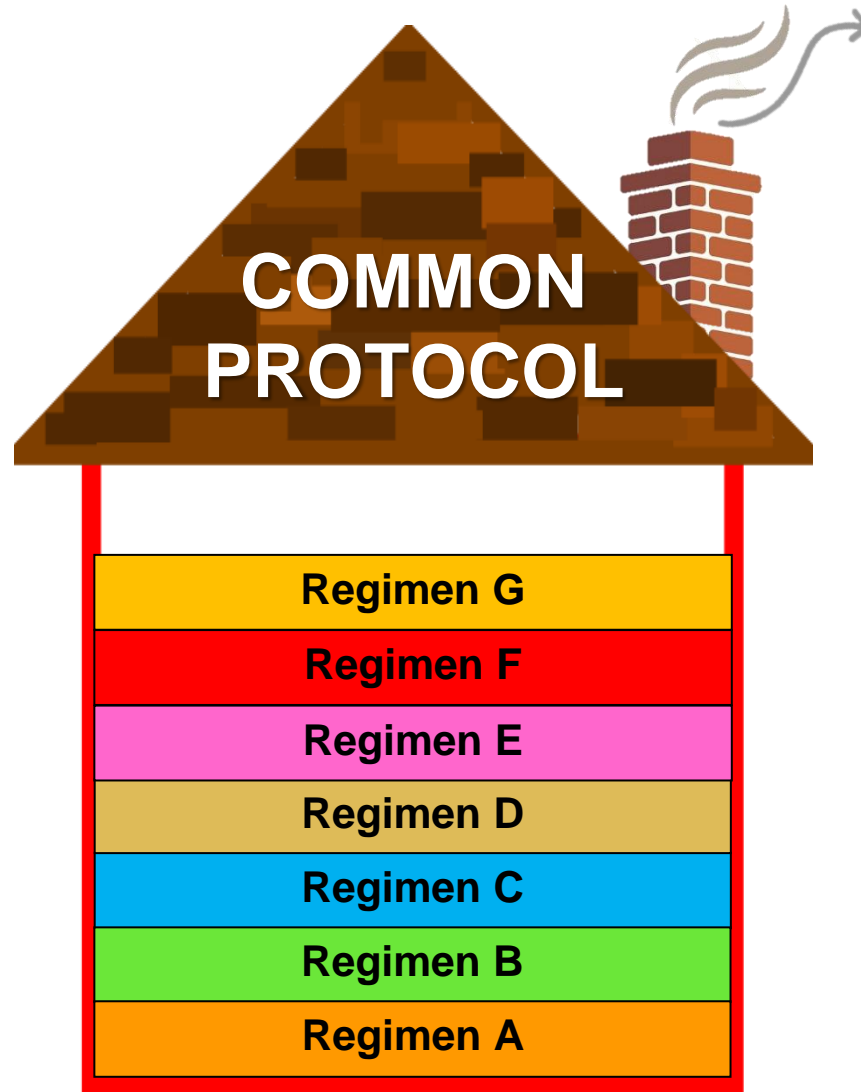
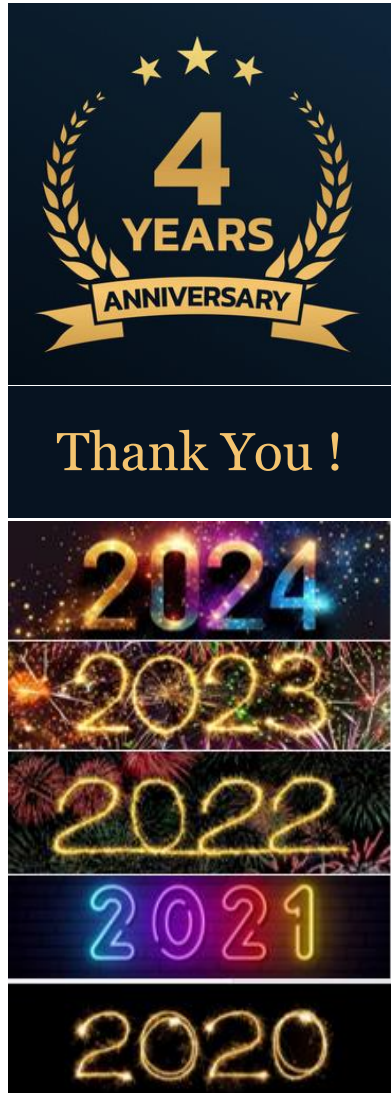


Calico



The AMG Foundation

Partnership, progress, and the pathway forward



Common protocol and shared infrastructure allow for operational and scientific efficiencies

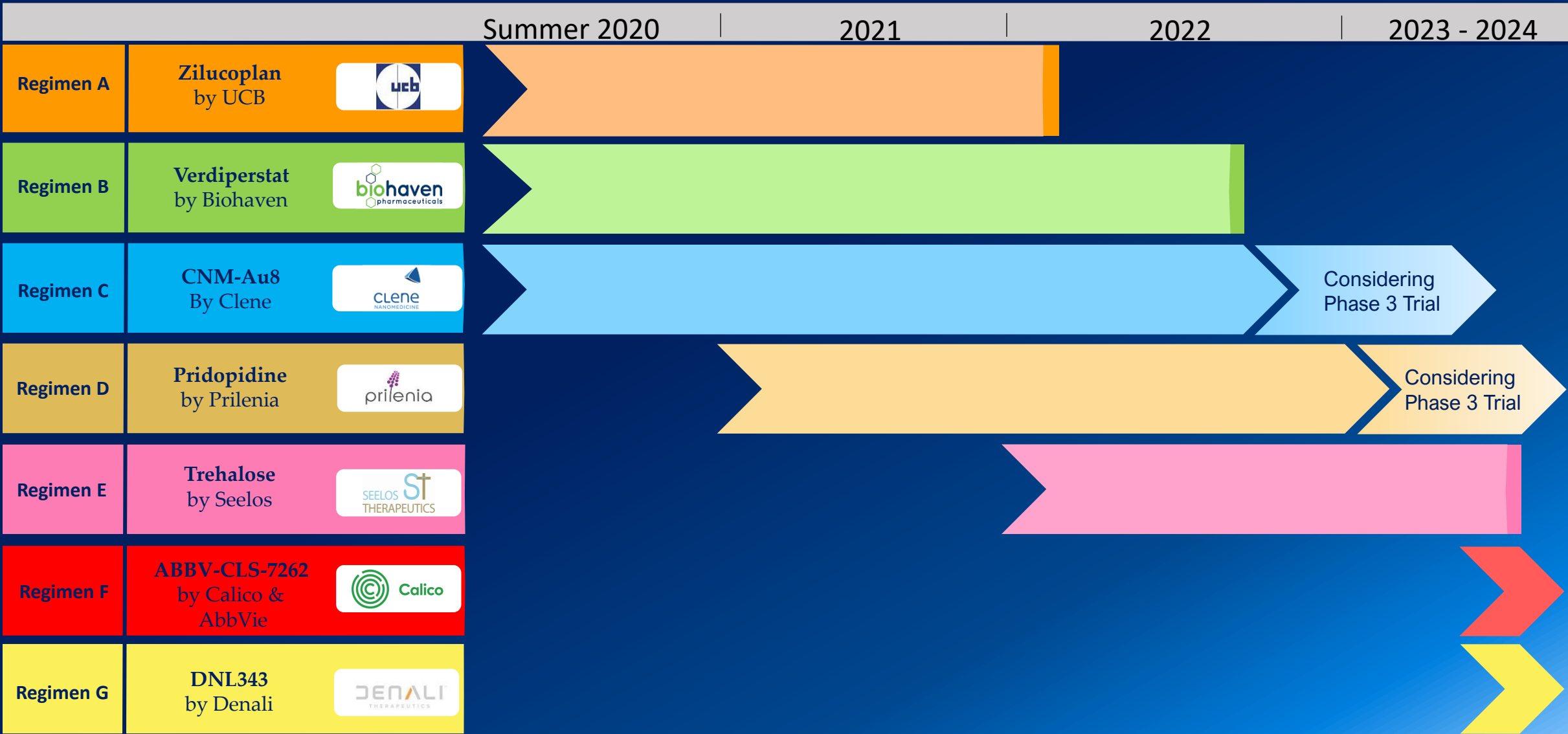
A large blue circle containing the following text in white:
1 Protocol (Phase 2/3)
1 single IRB
Central Governance

7 Regimens
70+ Enrolling Sites
~1400 Participants

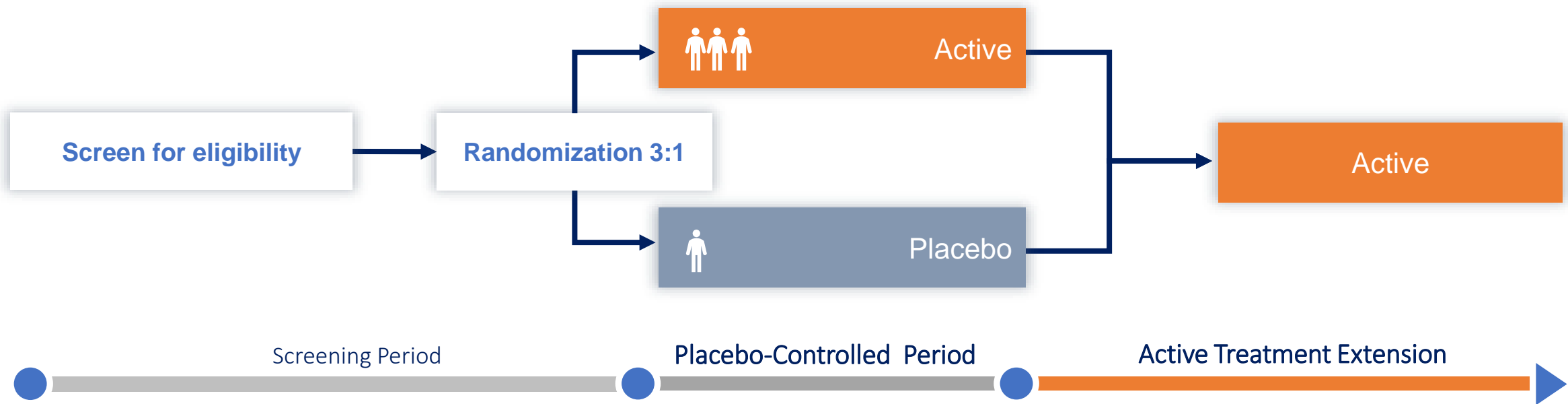
Regimen: Active Study Drug + Matching Placebo

The HEALEY ALS Platform Trial is designed to provide decisive answers and direction with efficient execution

HEALEY ALS Platform Trial



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 in disease severity as measured by the ALSFRS-R total score and survival

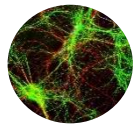
Safety, Secondary, and Exploratory Endpoints

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

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

**Endpoint
Development
Engine**

Additional biomarkers/outcome measures for upcoming regimens (new patient-reported outcomes; PBMCs for stem cell generation)

MDA awards \$500,000 grant to accelerate ALS research through the HEALEY ALS Platform Trial

View Press Release:



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

A blue rectangular graphic with white and light blue text. On the left is the MDA logo (Muscular Dystrophy Association). In the center, the text reads 'MDA Research Grant for the HEALEY ALS Platform Trial at Massachusetts General Hospital'. On the right is a circular portrait of Sabrina Paganoni, MD, PhD, with her name and title below it.

MDA Muscular Dystrophy Association

**MDA Research Grant
for the HEALEY ALS
Platform Trial**

at Massachusetts General Hospital

Sabrina Paganoni, MD, PhD
Co- Principal Investigator of the trial at
MGH and Co-Director, MGH Neurological
Clinical Research Institute (NCRI)

This grant will bolster the trial's capacity to explore new biomarkers, enhance patient outreach and recruitment, and engage with the FDA to adapt the trial based on previous experience.

The HEALEY ALS Platform Trial is a perpetual, adaptive trial testing multiple investigational products

NEWS · 5 MINUTE READ · JUL | 15 | 2024

Healey & AMG Center for ALS Announces New Investigational Drug Monepantel from PharmAust Limited will begin design for inclusion in HEALEY ALS Platform Trial

“We are thrilled to work with PharmAust leadership to design their regimen to investigate Monepantel for people with ALS.”

Merit Cudkowicz, MD, MSc

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

View Press Release:



<https://bit.ly/3zWwm9C>

- Drug candidates for the Platform Trial are chosen by a group of expert ALS scientists (the Therapy Evaluation Committee) and members of the Healey & AMG Center Science Advisory Committee
- Industry partners from selected companies work with the Healey Center Trial Design Team, the Northeast ALS Consortium (NEALS), Barrow Neurological Institute and Berry Consultants to tailor a new regimen to their investigational product

Acceleration Centers of Enrollment (ACE) Initiative

NEWS · 5 MINUTE READ · SEP | 4 | 2024

Sean M. Healey & AMG Center, Never Surrender, Inc. Establish Acceleration Center of Enrollment at University of Minnesota

View Press Release:



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

- The initiative will be led by a team of clinical investigators from **Mass General, Northwestern** and the **University of Minnesota**.
- Support for the ACE initiative comes from an Anonymous donor, ALS ONE, the Les Turner ALS Foundation, and Never Surrender.

NEWS · 5 MINUTE READ · AUG | 20 | 2024

Sean M. Healey & AMG Center for ALS and Les Turner ALS Center at Northwestern Medicine Establish Acceleration Centers of Enrollment Initiative

“By funding staff, training, and infrastructure, ACE is a vital step towards faster, more efficient research and ultimately, finding treatments and a cure for ALS.”

Jennifer DiMartino

Executive Director of ALS ONE

Navigating Clinical Trial Opportunities



- **ClinicalTrials.gov** – clinicaltrials.gov/

- **NEW ALS TDI Trial Navigator** – www.als.net/als-trial-navigator/

- **ALS Signal (I AM ALS)** – www.iamals.org/get-help/als-signal-clinical-research-dashboard/

- **NEALS** – neals.org/als-trials/neals-affiliated

- **MDA** – www.mda.org/research/clinical-trials

- **ALSA** – www.als.org/research/finding-als-research-opportunities

- **International Alliance of ALS/MND Associations** – www.als-mnd.org/support-for-pals-cals/research/understanding-als-mnd-research/



NEALS Consortium
@NEALSConsortium

Registration for our annual meeting has opened! People with lived ALS experience are welcome to join us virtually this year and be a part of this incredible event. Register here meeting.neals.org and select “Person with Lived ALS Experience (Virtual)”



meeting.neals.org
Home | NEALS Annual Meeting

➤ Registration deadline:
October 21st

3:37 PM · May 21, 2024 · 547 Views



2024 ANNUAL NEALS MEETING

OCTOBER 21 - 24, 2024

HYBRID MEETING
(Clearwater, FL and Online)



<https://meeting.neals.org/>

ABOUT THE MEETING

The Annual NEALS Meeting brings together researchers, clinicians, industry leaders, and individuals with lived ALS experience to discuss the latest advancements in ALS research and treatment.

With a focus on member initiatives and potential new therapies, the meeting prepares attendees for upcoming clinical trials and fosters collaboration among the ALS community. Join us as we continue to embrace inclusivity, facilitate knowledge exchange, and collectively shape the future of ALS research.



NEALS Educational Webinars

Visit the **NEALS website** to explore upcoming webinars or view previous recordings



<https://bit.ly/3S4P0Sb>



Access for ALL in ALS (ALL ALS) Consortium

Hosted May 16, 2024 / 11:00 am - 12:00 pm ET

Access for ALL with ALS Consortium

Funding started Oct 2023 by NINDS using ACT for ALS funds



Goals:

- Create a large, flexible ALS Research Consortium platform that can grow and be modified
- Provide opportunities for all individuals living with ALS in the United States to participate
- Run longitudinal natural history and biomarker studies
- Build a large openly shared data knowledge portal and biobank
- Provide clinical data and biosamples to better characterize ALS, identify biomarkers, and aid drug development
- Expand our goals to contribute ever more!

18 Apr 2024

7



[2024 SYMPOSIUM](#) [2023 SYMPOSIUM](#) [2022 SYMPOSIUM](#) [ALS ONE](#)

[REGISTER FOR FREE HERE](#)



7TH ANNUAL ALS RESEARCH SYMPOSIUM

November 13, 14 & 15th, 2024

Together from afar!

<https://www.alsoneresearchsymposium.org/>

Uniting researchers, clinicians and individuals living with ALS from around the world for three days of sharing important ALS research and hope.

Patient Navigation

Central resource for people living with ALS

Two webinars
per month!



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu

ALS Link sign-up:



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

Weekly webinar
registration:



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

Upcoming Webinars:

October 10th- EAP Discussion with Dr. Suma Babu and Prilenia Therapeutics

October 31st- Monthly Platform Trial Update

November 14th- EAP Discussion with Dr. David Walk and Never Surrender

November 21st- Monthly Platform Trial Update



Allison Bulat