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# Trehalose EAP

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Co-Director, Neurological Clinical Research Institute, Massachusetts General Hospital

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

Associate Professor of PM&R, Harvard Medical School

# Principal Investigators



Suma Babu, MBBS, MPH



James Berry, MD, MPH



Sabrina Paganoni, MD, PhD

- NIH U01 grant mechanism awarded Sept 2022
- First EAP funded by NIH-NINDS under ACT for ALS
- Trehalose EAP to occur in parallel to efficacy trial of trehalose (Regimen E of the HEALEY ALS Platform Trial)
- Trehalose is delivered via weekly intravenous infusions that can be given in the participants' homes

## Overarching Study Goal:

To provide access to trehalose and gather research data to supplement findings from the efficacy trial

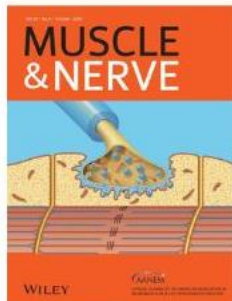
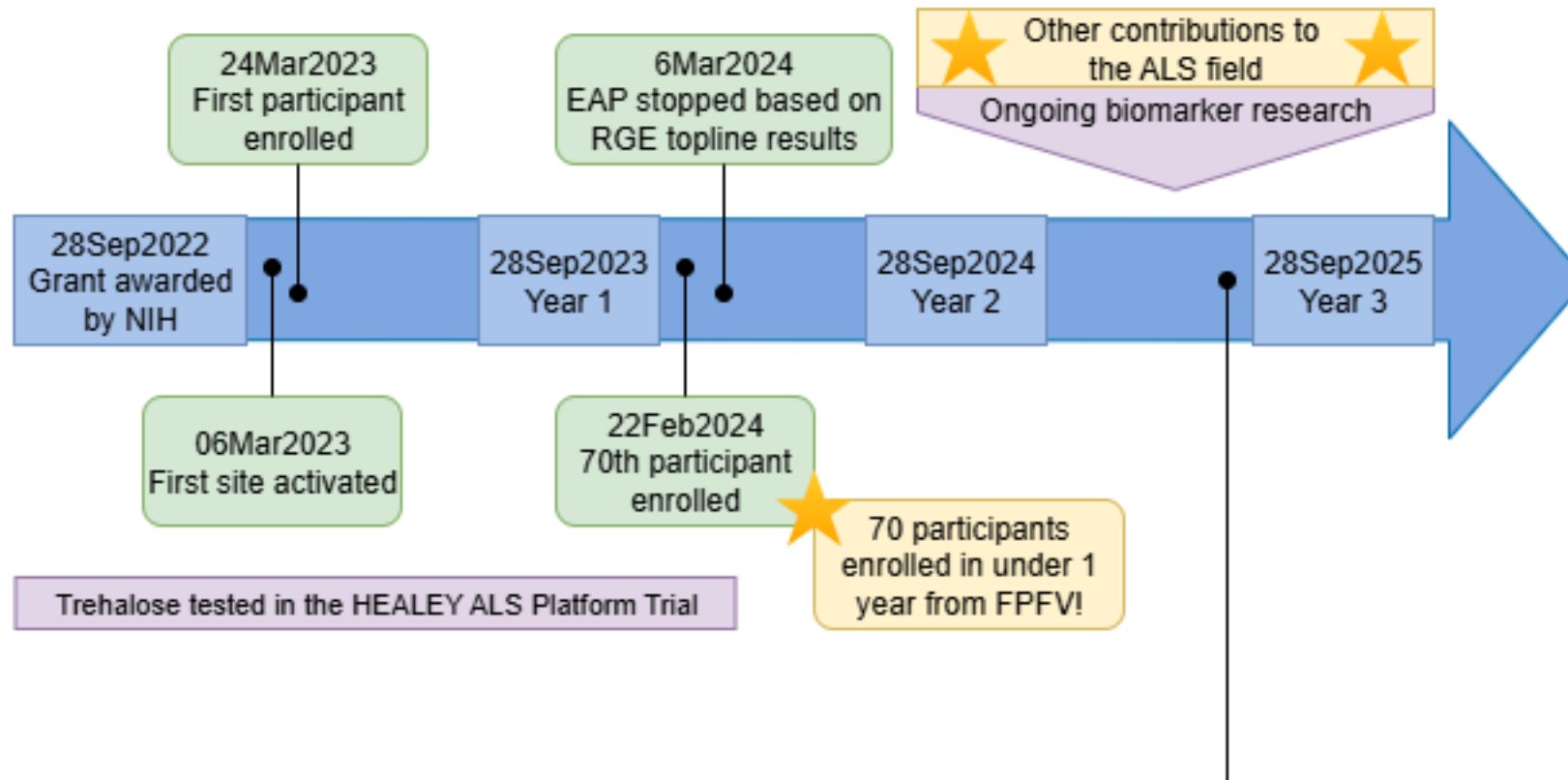
## Research Objectives:

- To generate safety data from a broad population not eligible for clinical trials
- To evaluate the biological impact of trehalose on motor neuron integrity (neurofilament)
- To evaluate the clinical effects of trehalose in this cohort and compared to external controls

70  
participants

25 sites  
across the  
US

# Study Timeline



CLINICAL RESEARCH ARTICLE

Open Access

**Multicenter Expanded Access Protocol for Research Through Access to Trehalose in People With Amyotrophic Lateral Sclerosis**

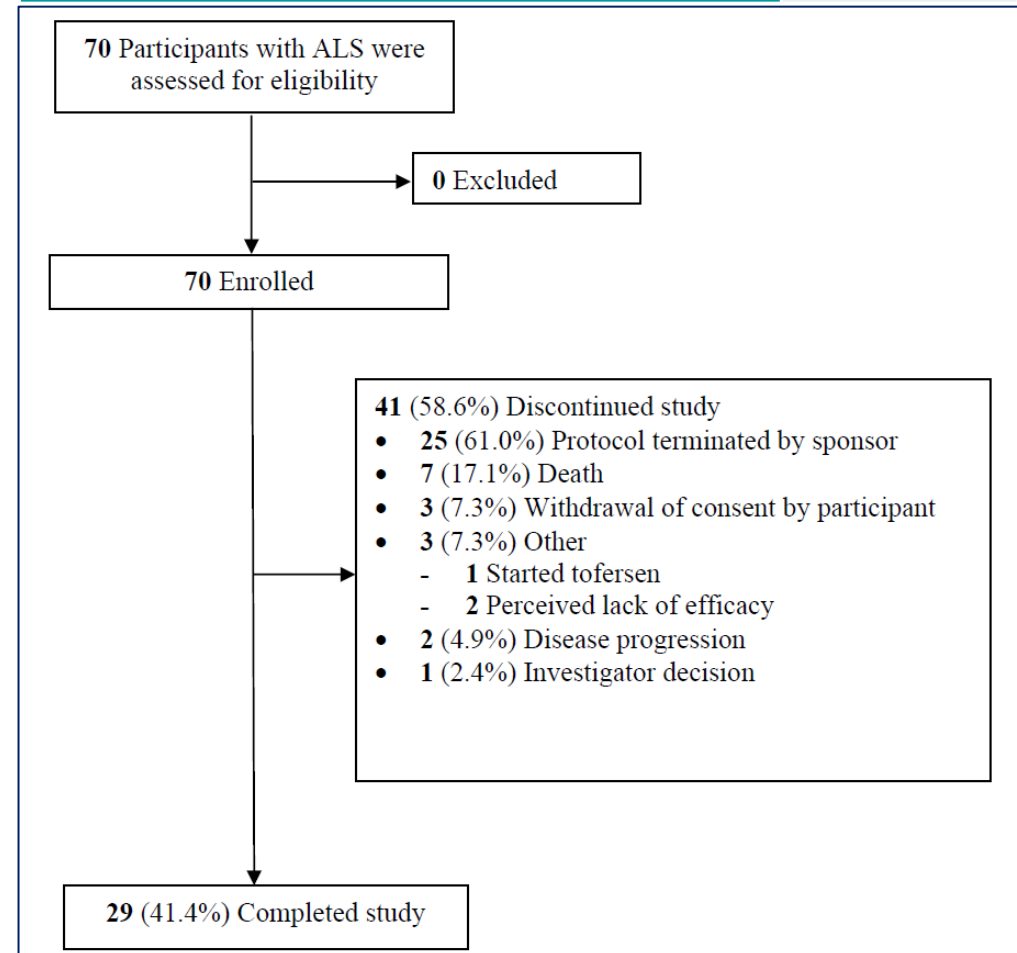


# Study Outcomes

## Clinical and NfL data in an EAP cohort

- Trehalose EAP enrolled participants not eligible for clinical trials including **participants with long disease duration and/or advanced stage**
- No new safety signals identified
- The NfL slope estimates per month were flat [ $-0.005$  (SE=0.0078; 95% CI ( $-0.021$ ,  $0.011$ ))]
- Propensity-score matching analysis (Answer ALS) showed no benefit of trehalose in ALSFRS-R total scores or survival over the study period

Baseline Data	Total (n=70)
Bulbar onset, No. (%)	29 (41.4)
Time since ALS symptom onset, mean (SD), mo	44.86 (28.91)
ALSFRS-R score, mean (SD)	27.9 (8.88)
Use of NIV, No. (%)	37 (52.9)



# Biomarker Research

840 serum aliquots collected  
(baseline and longitudinal)

108 serum aliquots used for NfL  
testing

461 serum aliquots used for  
biomarker projects

271 serum aliquots donated for  
future research

All Data Shared In:



# ALS Biomarker Analyses

Partnerships with biomarker expert groups foster cutting edge ALS biomarker discovery projects



ICON: Tau, pTau, miRNA



MIT & Broad: Metabolomics



Protavio: Olink (disease mechanistic protein pathway analyses)



SomaLogic: Proteomics



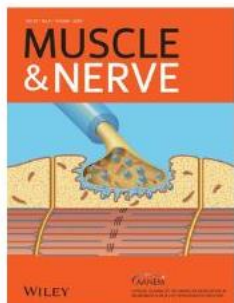
Johns Hopkins University: Cryptic Exons

Trehalose EAP samples + Answer ALS + ALL-ALS samples



# Conclusions

- The trehalose EAP was conducted in parallel to a phase 2/3 trial testing the efficacy of trehalose
- The trehalose EAP enrolled participants NOT eligible for the trial and collected safety, clinical and biological data in a population including patients with long disease duration and/or advanced disease
- All data have been deposited into PROACT and the ALS Knowledge Portal (AMP ALS, NIH)
- Samples are being used to generate new biomarker knowledge in collaboration with other large scale ALS projects



CLINICAL RESEARCH ARTICLE

 Open Access

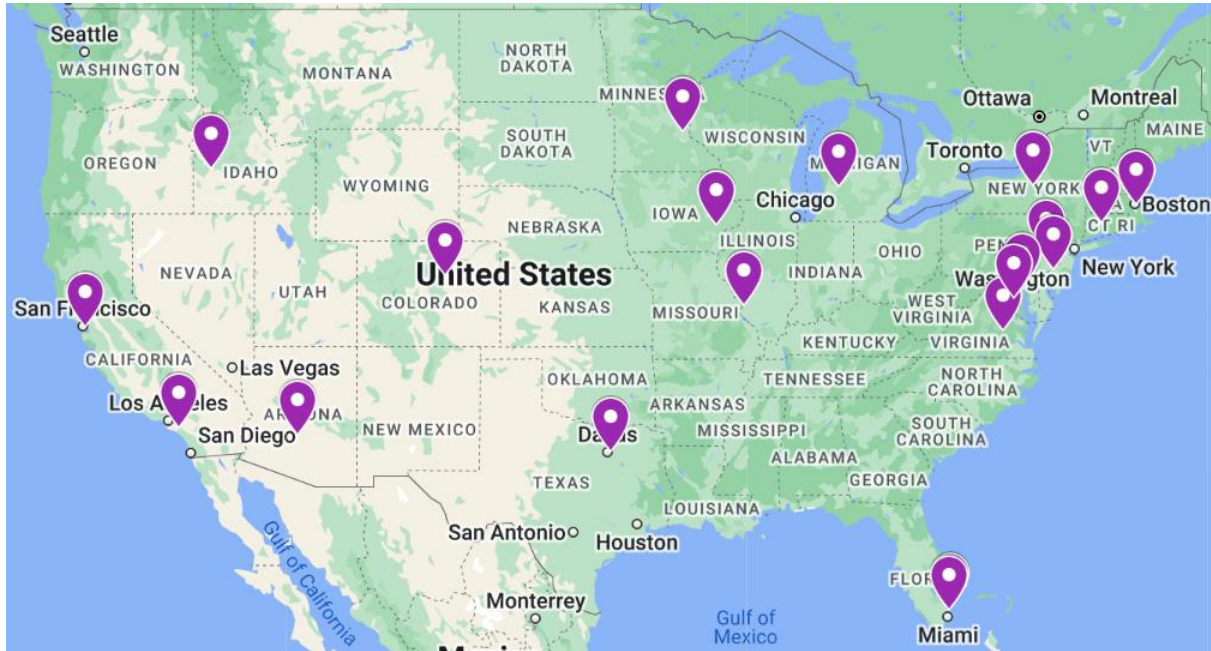


**Multicenter Expanded Access Protocol for Research Through Access to Trehalose in People With Amyotrophic Lateral Sclerosis**





# Thank you to our study sites and the Coordination Center (MGH and BNI)



Top Enrolling Sites
Mass General – 10 participants
Washington University – 9 participants
Texas Neurology – 6 participants
Virginia Commonwealth – 6 participants
Nova Southeastern – 6 participants

70 participants

21 sites

Site	Site Investigator
Massachusetts General Hospital	Sabrina Paganoni, MD, PhD
Texas Neurology	Daragh Heitzman, MD
Saint Alphonsus	Jackie Whitesell, MD
Holy Cross Hospital	Gustavo Alameda, MD
Virginia Commonwealth	Kelly Gwathmey, MD
Nova Southeastern University	Eduardo Locatelli, MD, MPH
University of Iowa	Andrea Swenson, MD
Washington University School of Medicine	Timothy Miller, MD, PhD
University of Colorado, Anschutz	Laura Foster, MD
Hospital for Special Care	Charles Whitaker, MD
George Washington University, MFA	Elham Bayat, MD
SUNY Upstate Medical University	Eufrosina Young, MD
University of California, Irvine	Namita Goyal, MD
University of Minnesota	David Walk, MD
University of Maryland School of Medicine Baltimore	Lindsay Zilliox, MD
Thomas Jefferson University	Hristelina Ilieva, MD, PhD
Beth Israel Deaconess Medical Center	Courtney McIluff, MD
Lehigh Valley Health Network	Alison Walsh, MD
Barrow Neurological Institute	Shafeeq Ladha, MD
Spectrum Health	Paul Twydell, DO
University of California, San Francisco	Laura Rosow, MD

# EAP Webpages and Resources: News & Webinars

## EAP News & Webinars:



<https://bit.ly/43GuegQ>

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Expanded Access News & Webinars

The Healey & AMG Center is committed to communicating news and updates about Expanded Access Protocols (EAPs).

For the most recent EAP updates, [read our latest EAP newsletter](#).

Upcoming EAP Webinars

The Healey & AMG Center hosts public Q&A webinars for the ALS community. Join us at 5:00-5:30 pm Eastern Time on the second Thursday of each month to discuss news and updates about Expanded Access, ask questions, and hear from doctors and industry professionals at MGH and beyond.

- **Thursday, September 11th**, 5:00 pm EDT: Expanded Access Discussion - [Register](#)
- **Thursday, October 9th**: Webinar Canceled
- **Thursday, November 13th**, 5:00 pm EDT: Expanded Access Discussion - [Register](#)

Register for upcoming webinars

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Monthly EAP Updates



# Patient Navigation

Central resource for people living with ALS



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Register for  
webinars:



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

EAP webinars  
second Thursday  
each month!

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

**November 20** – HEALEY ALS Platform Trial Discussion

**December 11** – Expanded Access Discussion