

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.



Monthly EAP community Q&A webinar

December 12, 2024

EAP Webpages and Resources



About the Healey & AMG Center for
ALS

Clinical Care &
Resources

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Expanded Access News & Webinars



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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 5:00-5:30pm 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, December 12th, 5:00 pm EST:** Monthly EAP Discussion - [Click here to register](#)

Register for Community Webinars >

Sign up for ALS Link >

Read Healey & AMG Quarterly
Newsletter >

EAP Opportunities

Current and upcoming expanded access protocols

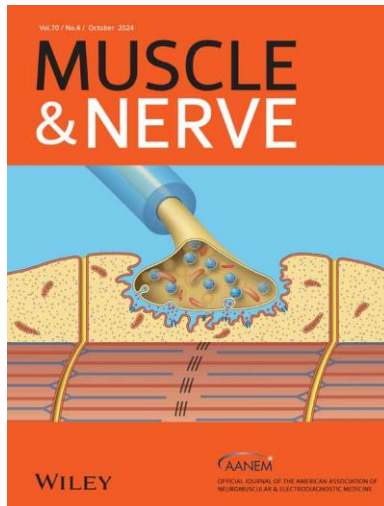
Sharing our experience

EAP publications available via open access

Multicenter expanded access program for access to investigational products for amyotrophic lateral sclerosis

First published: 06 June 2024

Open Access

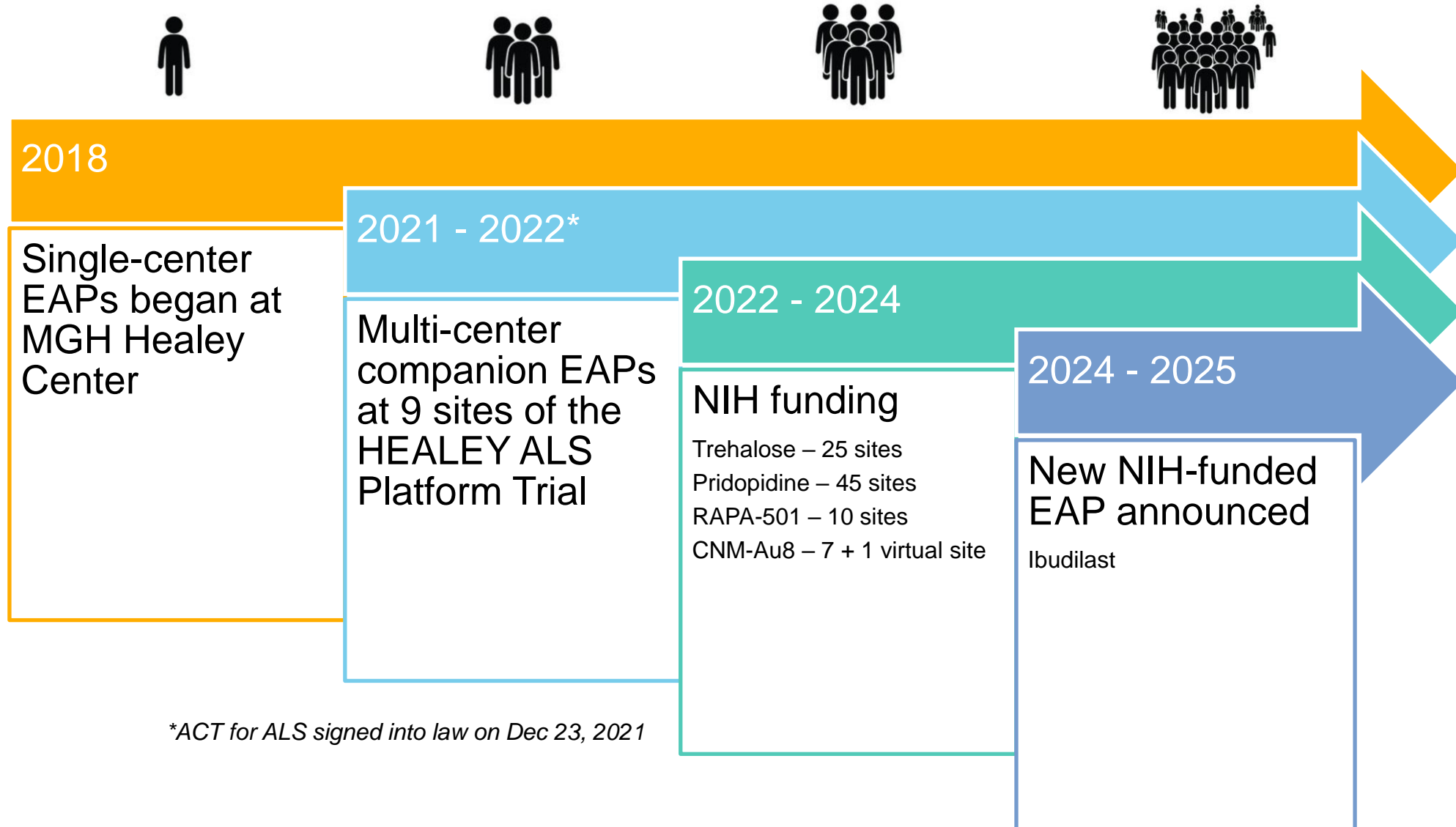


Publications

- [Multicenter expanded access program for access to investigational product for amyotrophic lateral sclerosis](#) [June 2024]
- [Expanded access protocol \(EAP\) program for access to investigational products for amyotrophic lateral sclerosis \(ALS\)](#) [April 2023]
- [Safety and activity of anti-CD14 antibody IC14 \(atibucimab\) in ALS: Experience with expanded access protocol](#) [December 2022]
- [An expanded access protocol of RT001 in amyotrophic lateral sclerosis- Initial experience with a lipid peroxidation inhibitor](#) [October 2022]

<https://onlinelibrary.wiley.com/doi/10.1002/mus.28169>

EAPs Then and Now





Pridopidine EAP 2

More info: [clinicaltrials.gov](https://clinicaltrials.gov/NCT06069934)
NCT06069934

- 32 sites activated
- **181** participants enrolled
- 19 screenings planned or in progress



CNM-Au8.EAP.04

More info: [clinicaltrials.gov](https://clinicaltrials.gov/NCT06408727)
NCT06408727

- All 8 sites activated
- ~**140** participants enrolled
- ~40 screens planned



RAPA-501 EAP

More info: [clinicaltrials.gov](https://clinicaltrials.gov/NCT06169176)
NCT06169176

- 6 sites activated
- **15** participants screened

Ibudilast EAP

- In Start Up
- Announced Sept 30, 2024
- Target enrollment: 200

Innovation and Research Through EAPs

INNOVATION:

- Flexible study designs with options for remote visits, testing feasibility of fully remote enrollment, remote collection of data
- Collection of digital outcome measures, longitudinal clinical data and biomarker samples
- Study data and biofluid samples from all EAPS go into NIH identified central repository after study completion for open science
- Use of virtual or 'synthetic' controls and application of historical controls

RESEARCH / SCIENTIFIC RIGOR:

- These EAPs have full data monitoring for data validity and reliability
- They are collecting patient reported outcomes (PROs), function, survival and clinical and digital biomarkers for efficacy analyses
- They employ regulatory standard clinical monitoring, DSMB, steering committees and safety reporting similar to traditional clinical trials

Building Landscape for Future Research

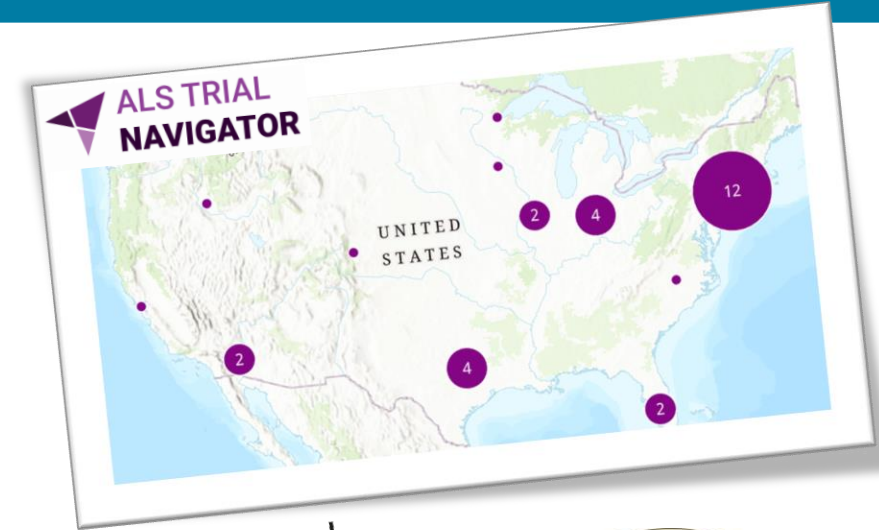
EAP programs are developing a more robust infrastructure for future ALS research

- **CNM-Au8 EAP** → first attempt at fully remote enrollment across the United States
- **Pridopidine EAP** → includes new sites including smaller community hospital and neurology private practice group to build research capacity for more centers to be involved in ALS research in the future
- **RAPA-501 EAP** → broadening the number of centers that can develop interdisciplinary expertise in ALS and cell therapy
- Learnings from the setup of these EAPs has led to efficiency for future EAP programs
- All EAPS are contributing to same sustainable central repository for ALS through the NIH that will be available and open for future research by any researcher

Navigating the EAP Landscape

Resources and Tools

- **ALS Doctor / Clinical Care Team**
 - Start local, ask your provider about EAP options
- **ClinicalTrials.gov:** <https://clinicaltrials.gov/>
 - Lists EAP opportunities, sites, and contact information once active
- **ALS TDI Trial Navigator Tool:** <https://www.als.net/als-trial-navigator/>
 - Worldwide map to search for opportunities based on *your* preferences
- **NEALS Website:** <https://neals.org/als-trials/expanded-access>
 - Scroll to the bottom of page to view list



Patient Navigation

Central resource for people living with ALS

Two webinars
per month



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ALS Link sign-up:



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

Weekly webinar
registration:



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

Upcoming Webinars:

December 19th- Platform Trial Update

January 9th- Monthly Expanded Access Update

January 23rd- Platform Trial Update



Allison Bulat