

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

Expanded Access

FDA Definition:

- Sometimes called “compassionate use”
- Potential pathway for a patient with an immediately life-threatening or serious disease or condition
- Allows access to an investigational medical product outside of clinical trials
- Used when no comparable or satisfactory alternative therapy available

Requirements for All EAPs

- Patient has a **serious** or **immediately life-threatening** disease or condition; no comparable or satisfactory alternative therapy
- **Potential benefit justifies potential risk**; potential risk not unreasonable in context of disease or condition
- Providing drug **will not interfere with clinical trials** that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use
- For patients who **do not qualify** to participate in a clinical trial (the criteria for participation in an EAP are generally broad and inclusive)

Categories of EAPs

Individual Patient, including emergency use

Intermediate-size Patient Populations

Treatment Protocol (large patient population)

FDA encourages EAPs while developing drugs for ALS

Long term safety data:

“During development, sponsors should collect safety data, including data from open-label studies or expanded access programs, from patients across the spectrum of disease stages and severities, and whenever possible, data from patients who may not have been included in effectiveness studies but in whom, based on other data, the use of the drug following approval is likely.” [Page 4]

Generalizability of safety and efficacy data:

“There is a need to understand the safety and effectiveness of investigational drugs for ALS across disease stages..... An acceptable approach could include enrollment of a broad population with the conduct of the primary analysis in a study subset defined based on clinical characteristics and/or biomarkers, and analyses of the broader population being secondary and supportive” [Page 3]

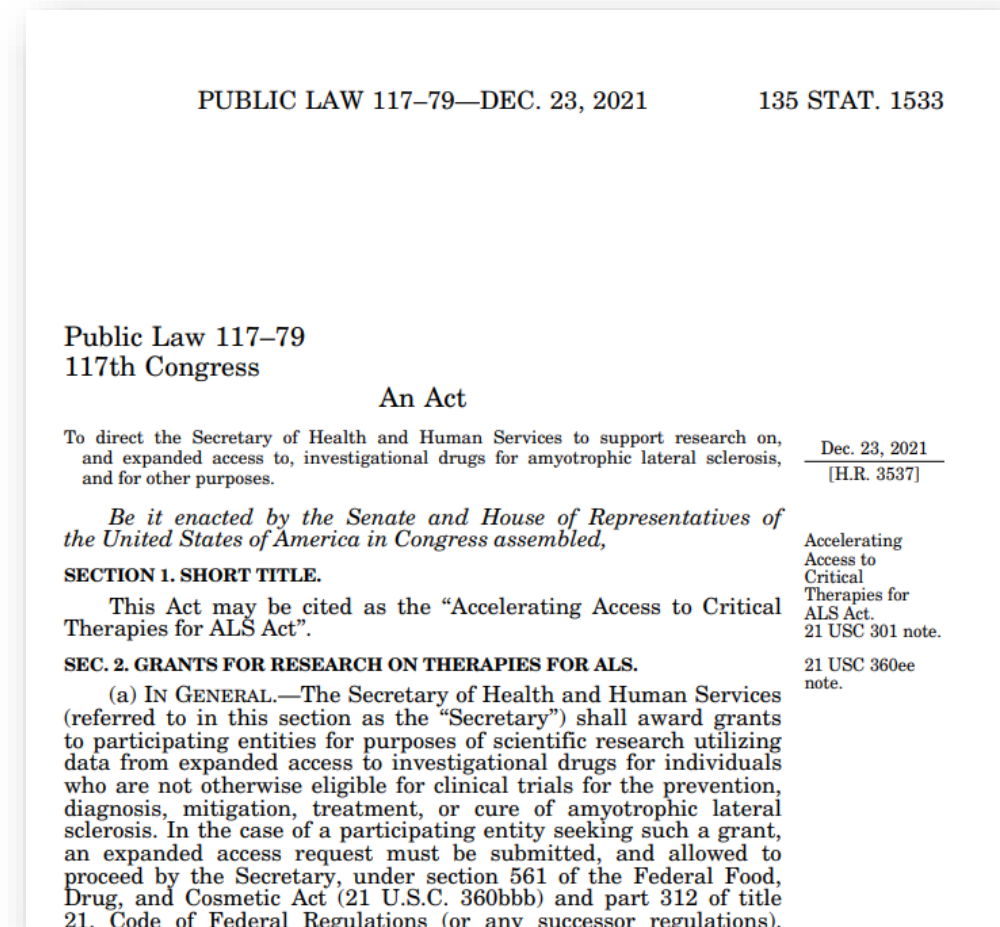
Amyotrophic Lateral Sclerosis: Developing Drugs for Treatment Guidance for Industry

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

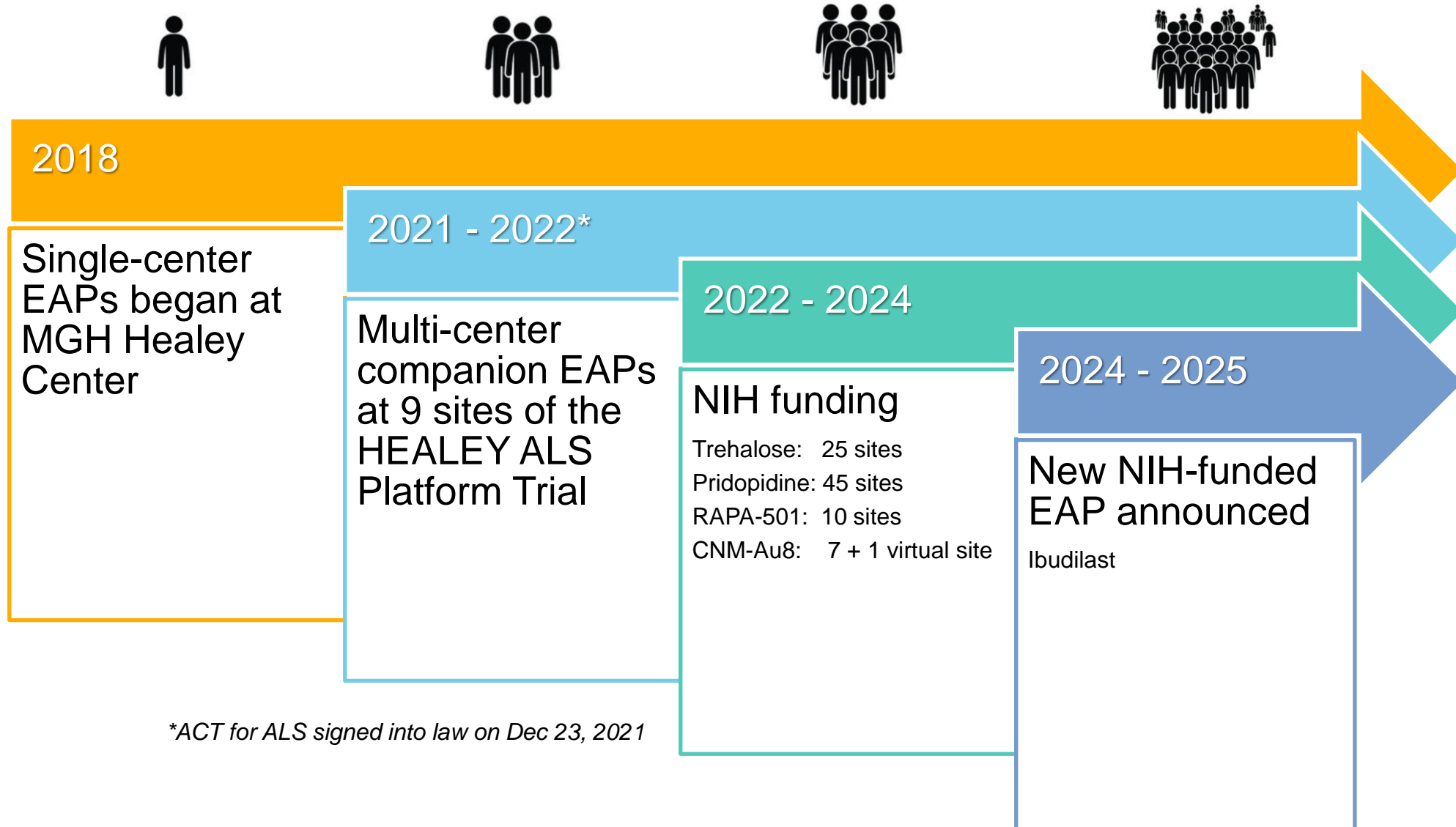
September 2019
Clinical/Medical

ACT for ALS- A new opportunity to expand access and collect real world data in parallel to clinical trials via EAP

- Signed into law on Dec 23, 2021
- Grants for Research on Therapies via Intermediate-Size EAPs for ALS
- NIH U01 grant mechanism



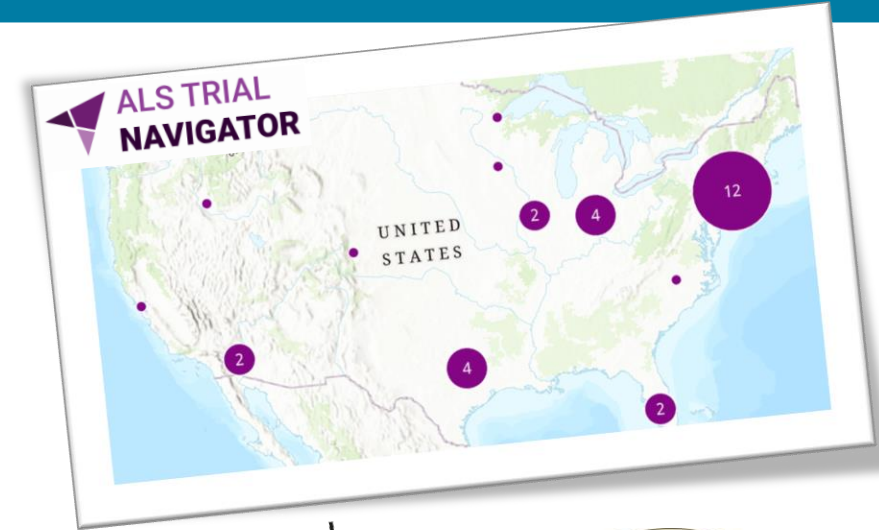
EAPs Then and Now



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



Pridopidine EAP 2

clinicaltrials.gov NCT06069934

[Status: No Longer Enrolling]

- 32 sites activated
- More than 200 participants enrolled



clinicaltrials.gov

CNM-Au8.EAP.04

clinicaltrials.gov NCT06408727

[Status: No Longer Enrolling]

- 8 sites activated
- About 180 participants enrolled



[clinicaltrials.gov](https://clinicaltrials.gov/ct2/show/study/NCT06408727)

RAPA-501 EAP

clinicaltrials.gov NCT06169176

[Status: Enrollment Ongoing]

- Epigenetically reprogrammed T stem cell therapy
- 9 sites activated (contact info on ct.gov)
- Target enrollment: 40 ALS individuals



clinicaltrials.gov

Ibudilast EAP

NINDS grant 1U01NS136022-01A1

[Status: Enrollment Ongoing]

- Scalable expanded access with analysis of neurofilament and other biomarkers for Ibudilast in ALS (SEA-NOBI-ALS)
- 16 sites selected (3 Mayo clinics)
- Target enrollment: 200 ALS individuals
- Webinar with Dr. Björn Oskarsson hosted August 14th (recording available)

<https://widetrial.com/>



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

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

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

Sharing Our Experience

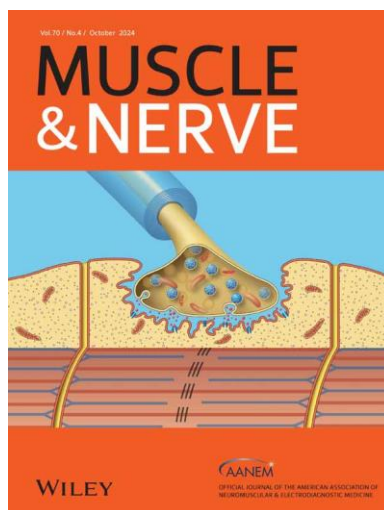


EAP publications available via open access

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

First published: 26 August 2025

Open Access



<https://onlinelibrary.wiley.com/doi/10.1002/mus.70011>

- [An expanded access protocol of RNS60 in amyotrophic lateral sclerosis](#) [March 2025]
- [Multicenter expanded access program for access to investigational products 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]

EAP Webpages and Resources



Link to EAP Website:



<https://bit.ly/3uni3lc>

About the Healey & AMG Center for ALS

Clinical Care & Resources

Research Opportunities

Platform Trial

Expanded Access

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Expanding Access to Investigational Products

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Overview

At the Sean M. Healey & AMG Center for ALS, we are dedicated to developing effective treatments for ALS and to providing people with ALS access to experimental therapies through Expanded Access Protocol (EAP) programs.

EAPs are a pathway for people with a life-threatening condition or serious disease to gain access to an investigational medical product when they are not eligible for a clinical trial. These investigational products – drugs, biologics, or medical devices – are currently being studied, but not yet approved by the US Food and Drug Administration (FDA). Alongside traditional data, EAPs can also provide data that may be useful in developing new therapies.

We have built a dedicated team at the Healey & AMG Center to rapidly implement EAPs for people with ALS at Mass General and are working with several other research centers across the US.

Supporting Expanded Access

Expanded Access Protocol programs at the Sean M. Healey and AMG Center for ALS are funded by multiple sources, including contributions from donors like you. Thank you for partnering with us in the quest to discover life-saving therapies for individuals affected by ALS.

Give Now >

Community Support >

EAP Opportunities

Current and upcoming expanded access protocols

Learn more >

EAP News & Webinars

Get the latest news and updates about expanded access protocols

Learn more >

Information for Patients

Common questions and helpful resources about expanded access

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EAP Webpages and Resources: FAQs

Common Questions:



<https://bit.ly/4aYRI3u>

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Expanded Access Questions & Answers

The best way to learn about expanded access opportunities is to have a discussion with your ALS doctor and clinical care team. Below are some common questions and resources that can help guide these discussions.

How is expanded access different from clinical trials?

What is an EAP?

Am I eligible to participate in EAPs?

Why would I participate in an EAP?

How are EAPs funded?

How can I learn more about EAPs?

Other Helpful Resources

- [NEALS | Expanded Access Programs](#)
- [Expanded Access | Information for Patients | FDA](#)
- [Expanded Access | Information for Physicians | FDA](#)
- View recordings of past [Healey & AMG Center Weekly webinars](#)
- [Watch an educational webinar about EAPs](#) hosted by the Northeast ALS Consortium (NEALS)

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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

Webinar Recordings

Featured EAP Webinars

Monthly EAP Updates

Register for
webinars:



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

Sign up for
ALS Link:



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

Educational Webinar Opportunities



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



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

Expanded Access Programs in ALS: What Have We Learned, Where Do We Go from Here?



Jinsy Andrews,
MD, MSc
Columbia
University



Suma Babu,
MMBS, MPH
Massachusetts
General Hospital



Richard Bedlack,
MD, PhD
Duke
University

Wednesday, January 15th 2025

1:00 PM - 2:00 PM ET

Educational Webinar Opportunities

- **NEALS** – neals.org/people-living-with-als/educational-webinars
- **Les Turner ALS Foundation** – lesturnerals.org/support-services/als-learning-series/
- **Everything ALS** – www.everythingals.org/events
- **ALL ALS** – www.all-als.org/webinars/

Missed a webinar?
View recordings
online!

