

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**



# 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

# EAPs Then and Now



2018

Single-center  
EAPs began at  
MGH Healey  
Center

2021 - 2022\*

Multi-center  
companion EAPs  
at 9 sites of the  
HEALEY ALS  
Platform Trial

2022 - 2024

NIH funding

Trehalose: 25 sites  
Pridopidine: 45 sites  
RAPA-501: 10 sites  
CNM-Au8: 7 + 1 virtual site

2024 - 2025

New NIH-funded  
EAP announced

Ibudilast

*\*ACT for ALS signed into law on Dec 23, 2021*

# 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**

Home - Neurology - ALS - Research

## Expanding Access to Investigational Products

EAP Opportunities >    EAP News & Webinars >    Information for Patients >

### 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 >](#)

### Expanded Access

- [Overview >](#)
- [EAP Opportunities >](#)
- [For Patients >](#)
- [News & Webinars >](#)

#### 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

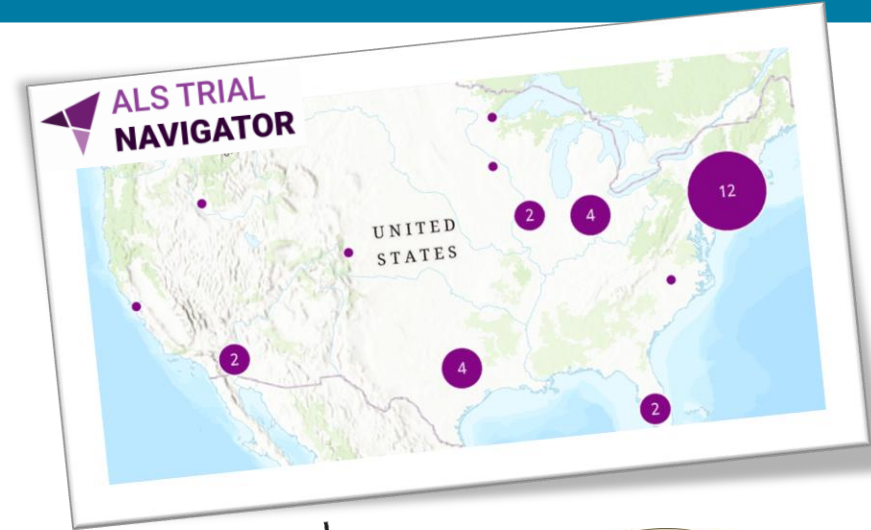
Common questions and helpful resources about expanded access

[Learn more >](#)

# 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



**Catherine Small**  
Patient Navigator

Phone: 833-425-8257 (HALT ALS)

E-mail: [healeyalsplatform@mgh.harvard.edu](mailto:healeyalsplatform@mgh.harvard.edu)

**EAP webinars  
second Thursday  
each month!**

**Register for  
webinars:**



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

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

**December 18** – HEALEY ALS Platform Trial Discussion

**January 8** – Expanded Access Discussion

**January 22** – Research Access Discussion

**February 12** – RAPA-501 EAP Update with Rapa Therapeutics

