

Thank you for joining the weekly webinar!

We are admitting audience members from the waiting room.

Please allow a few moments for the webinar to begin.



HEALEY ALS Platform Trial

Weekly Q&A – April 18, 2024



Healey & AMG Center

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



The AMG Foundation



This trial is **dedicated to all people living with ALS**, their families, and friends. Your **partnership** in research is what keeps us filled with passion, dedication, and the commitment to develop new treatments for ALS

This breakthrough trial would not be possible without your participation.

Every research participant, whether on the active drug or placebo, plays a critical role in making the hope of finding a cure for ALS a reality

Thank You

Navigating Clinical Trial Opportunities



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



- **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/

Educational Webinar Opportunities



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



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

April 25, 2024 / 1:00 pm–2:00 pm

Constipation in People Living with ALS: Does Autonomic Dysfunction Play A Role?

Join us for an insightful session with Dr. Ghazala Hayat, MD, FAAN, FANA, FAANEM, as we delve into the prevalence and challenges of constipation in People Living with ALS. Dr. Hayat will discuss how constipation might be connected to...

May 8, 2024 / 4:00 pm–5:00 pm

An Overview of The ALS Better Care Act

Join Dr. Terry Heiman-Patterson, Dr. Kelly Gwathmey and Dr. Sabrina Paganoni, and as they discuss the importance of multidisciplinary care and the role of the ALS Better Care Act to ensure that quality care at Multidisciplinary Centers...

May 16, 2024 / 11:00 am–12:00 pm

Access for ALL in ALS (ALL ALS) Consortium

National Institutes of Health (NIH) funded “ALL ALS” consortium is a multi-institutional effort that aims to organize the ALS clinical research landscape in the US. ALL ALS is operating in partnership with several stakeholders...



EAP Webpages and Resources



Link to EAP Website:



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

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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.

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Common questions and helpful resources about expanded access

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EAP Webpages and Resources

RAPA-501 Expanded Access Protocol

This EAP is supported 100% by the NINDS of the National Institutes of Health under award number 1U01NS136020-01, led by the Healey & AMG Center at MGH and NEALS Consortium in collaboration with Rapa Therapeutics

The RAPA-501 Expanded Access Protocol (EAP) will be made available to 40 people with ALS at up to 10 US research centers. As per the ACT for ALS law, all EAP participants need to be ineligible for other ongoing clinical trials. This EAP is a unique opportunity for people with ALS to get access to an investigational product (IP) and contribute to clinical research.

RAPA-501 is simultaneously being studied in the RAPA-501 Phase 2/3 Expansion Cohort. The RAPA-501 EAP will provide real-world data to supplement the RAPA-501 clinical development program.

This EAP will be conducted with the same scientific rigor as a traditional clinical trial, and the data collected will be reported as per NIH and FDA requirements.

View the RAPA-501 EAP on ClinicalTrials.gov
<https://rb.gy/be8e5p>

About RAPA-501

What is RAPA-501?
RAPA-501 is a regulatory T-cell therapy being studied in ALS. RAPA-501 cells are made from a participant's own T (immune) cells. T-cells are filtered from the blood and modified in a laboratory to become RAPA-501 cells.

What impact could RAPA-501 have on ALS?
Since RAPA-501 is currently in a clinical trial for ALS, its effect is unknown. In ALS, certain inflammatory cells in the immune system may be overactive, leading to motor neuron damage and faster ALS progression. RAPA-501 cells are designed to balance an overactive immune system and slow ALS progression.

How is RAPA-501 administered?
RAPA-501 is administered as an intravenous (IV) infusion. There is one in-person infusion administered every 6 weeks. A participant may have up to 4 infusions.

How many in-person visits are required for the EAP?
There are up to 7 in-person visits followed by 2 months of remote monitoring. Planned participation is up to 8 months.

Who is eligible to participate in this EAP?
People with ALS who are not eligible for other trials may be eligible to participate. To learn if you maybe eligible, reach out to a participating research center.

View the RAPA-501 EAP on the MGH website
<https://rb.gy/hlfq31>

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Expanded Access Opportunities

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NIH-Funded Multicenter Expanded Access Protocols led by the Healey & AMG Center for ALS

RAPA-501 EAP, by Rapa Therapeutics

In people with ALS, the body's immune system becomes imbalanced and appears to hasten the loss of motor neurons in the brain and spinal cord. Regulatory T-cells help reduce inflammation and could lead to a more balanced immune system in people with ALS. The goal of this study is to reduce neuroinflammation, potentially slowing ALS progression, using specially prepared regulatory T-cells, called RAPA-501 cells. RAPA-501 cells are created through a series of steps by first taking the participant's own blood though a specialized IV (apheresis), then isolating regulatory T-cells from the blood. Next, these regulatory T-cells are grown under special conditions in a petri dish, becoming RAPA-501 cells. The RAPA-501 cells are then returned to the participant through an intravenous infusion. Prior to the IV infusion of RAPA-501 cells, a low dose of chemotherapy is given to reduce the body's immune response and potentially heighten the effects of the RAPA-501 cells.

[View the RAPA-501 EAP on ClinicalTrials.gov](#)

[Download the RAPA-501 EAP Brochure](#)

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Common questions and helpful resources about expanded access

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EAP News & Webinars

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EAP Webpages and Resources

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

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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.

Webinar Recordings

Featured EAP Webinars

Monthly EAP Updates

Press Releases

- October 2023:** [Sean M. Healey & AMG Center for ALS awarded NIH U01 Grant to support Expanded Access to Pridopidine in Collaboration with Prilenia Therapeutics](#)
- October 2023:** [Sean M. Healey & AMG Center for ALS awarded NIH U01 Grant to support Rapa Therapeutics' Expanded Access Protocol of Epigenetically Reprogrammed RAPA-501](#)

Publications

- [Expanded access protocol \(EAP\) program for access to investigational products for amyotrophic lateral sclerosis \(ALS\)](#)

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Patient Navigation

Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu

Weekly webinar
registration:



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

ALS Link sign-up:



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

Upcoming Webinars:

April 25th- Weekly Q&A webinar

May 2nd- Weekly Q&A webinar

May 9th- EAP Discussion with Shawn Sarbacker from Tech vs ALS



Allison Bulat