RAPA-501
Expanded Access Protocol

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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.

Healey & AMG Center
Sean M. Healey & AMG Center for ALS
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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 ClinicalTrials.gov
https://rb.gy/be8e5p

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

Information About Expanded Access

What is Expanded Access (EA)?

EA is a pathway for people with a serious and life-threatening disease to access an investigational product (IP) that is not yet approved by the Federal Drug Administration (FDA). EA is an option for people who do not qualify for ongoing clinical trials. An IP offered through EA is experimental, so its effectiveness as a treatment for ALS is not yet known. At this time, EA protocols have limited availability due to funding and staffing constraints.

What are the benefits of participating in Expanded Access?

EA provides people living with ALS who are not eligible for clinical trials the opportunity to access an IP while the IP is being formally tested. Participation in EA may also contribute to research by providing safety and biomarker data (indicators of disease) that may benefit the greater ALS research community.

How is Expanded Access different from clinical trials?

The primary purposes of clinical trials are to formally evaluate IPs and gather data that may lead to FDA approval. Clinical trials have strict eligibility criteria, frequent in-person visits, and often include a placebo group. Participation in clinical trials is optional and separate from clinical care, and clinical trials may be conducted by a research specialist.

EA protocols have broader and more inclusive eligibility criteria, fewer in-person visits, and do not have a placebo group. Participation in EA is considered an extension of clinical care and requires oversight by the treating clinician in addition to FDA and ethics board compliance requirements.

General questions? Email: mghalsresearch@mgh.harvard.edu

More Info About Expanded Access

Visit the FDA website

https://bit.ly/3Jx32rj

Visit NEALS.org

https://bit.ly/402uapg

Register for Monthly EAP Webinars

https://bit.ly/43GuegQ