Trehalose Expanded Access Protocol

Healey & AMG Center
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First NIH-funded EAP by the ACT for ALS
Led by the Healey & AMG Center at MGH and
NEALS Consortium in collaboration
with Seelos Therapeutics

Expanded Access (EA) is designed to be a pathway for people living with a serious and life-threatening disease to access an investigational product (IP) if they do not qualify for clinical trials.

The **Trehalose Expanded Access Protocol (EAP)** will be made available to 70 people with ALS at up to 25 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 IP and contribute to clinical research.

Trehalose is simultaneously being studied in the HEALEY ALS Platform Trial Regimen E. The Trehalose EAP will provide real-world data to supplement the Trehalose clinical development program by: 1) evaluating the effects of the drug in a population that is broader than the one included in the Platform Trial, and 2) gathering longer-term safety and efficacy data. 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 Trehalose EAP on clinicaltrials.gov
https://bit.ly/3G09dU3

About Trehalose (SLS-005)

What impact could Trehalose have on ALS?
Trehalose is in a clinical trial for ALS now, so its effect is not known. Trehalose may delay symptoms and preserve motor neurons in the spinal cord. Trehalose is a naturally occurring disaccharide (simple sugar) that crosses the blood-brain barrier when administered by intravenous (IV) infusion. Go to the MGH website (link below) to learn more.

How is Trehalose administered?
Trehalose is administered as a weekly IV infusion. For many participants, the weekly infusions may be done at home with approval from the study team.

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

How many in-person visits are required, and what is the length of participation?
There are up to 5 required in-person visits for this EAP. Participation is designed to last up to 6 months.

View the Trehalose EAP on the MGH ALS website
https://bit.ly/401G3vA

Information About Expanded Access:

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

What is Expanded Access? Expanded Access (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? Expanded Access (EA) provides people living with ALS who are not eligible for clinical trials the opportunity to access an investigational product (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 investigational products (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.

The primary purpose of Expanded Access (EA) is to provide people who do not qualify for a clinical trial with access to an IP. 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.

More Info About Expanded Access:

Visit the FDA website

https://bit.ly/3Jx32rj

Visit NEALS.org

https://bit.ly/402uapg

Register for Weekly Platform Trial Webinars

https://bit.ly/3TwAt1D