Healey Center
Sean M. Healey & AMG Center for ALS at Mass General

HEALEY ALS Platform Trial

Regimen E

SLS-005 (trehalose)
Developed by Seelos Therapeutics

Regimen E is testing the investigational product SLS-005 (trehalose). The investigational products included in the HEALEY ALS Platform Trial were selected by a team of experts after careful review of the science supporting the potential of the product for ALS.

Please discuss the potential benefits and risks of this investigational product with your study physician.

Visit our website to learn more about what to expect in the trial process:
https://bit.ly/3ExRaI8

About Regimen E:

- Regimen E is enrolling approximately 160 participants to evaluate the safety and efficacy of trehalose as a potential treatment for ALS.
- **3:1 Active Drug to Placebo Ratio:** Participants who enroll in the trial have a 3 in 4 chance of being assigned to the active treatment group and a 1 in 4 chance of being assigned to the placebo group.
- **Open Label Extension (OLE):** Upon completion of the 24-week trial, participants will have the option to enroll in the OLE for trehalose. During OLE, all participants will receive active study drug.

To see if you may qualify, please review the list of eligibility criteria:
https://bit.ly/30ctynm

For general questions about the HEALEY ALS Platform Trial,
Contact the Patient Navigator

healeyalsplatform@mgh.harvard.edu

833-425-8257 (HALT ALS)

Q&A for Regimen E:

Q: How is this drug administered?
A: Trehalose is administered as weekly intravenous (IV) infusions. The first four infusions must be completed in-person at the study center, but there may be the option to transition to at-home infusions starting after week 4 of the study.

Q: Has this drug been studied before?
A: Yes. In previous studies using cell and mouse models of ALS, trehalose was shown to increase the clearance of TDP-43 (helping cells remove toxic proteins), decrease SOD1 and SQSM1/p62 aggregation (helping cells prevent the accumulation of toxic proteins), delay disease progression, and preserve motor neurons in the spinal cord. In prior Phase II trials involving patients with Oculopharyngeal Muscular Dystrophy and Spinocerebellar Ataxia Type 3, trehalose showed a favorable safety profile. The HEALEY ALS Platform Trial is the first trial studying trehalose in patients with ALS.

Q: What does this drug do?
A: Trehalose is a naturally occurring low molecular weight disaccharide (sugar). When administered through IV, trehalose crosses the blood-brain barrier, stabilizes proteins and, importantly, activates autophagy, which is the process that clears material from cells. Trehalose may delay the progression of ALS by reducing mutant protein aggregation and improving the clearance of toxic materials from motor neurons.

Additional questions?

Register to attend the Weekly Platform Trial Q&A Webinars:

https://bit.ly/3DvkJTa

Stay Connected to the Platform Trial

More investigational products are anticipated to be added to the HEALEY ALS Platform Trial through support by pharma, foundation partners, philanthropy, federal, and other fundraising initiatives.

Visit our website to learn more about current and future regimens:

https://bit.ly/31EKT98

View map and contact info for participating research centers:

https://bit.ly/3IICv9t

Sign up for the ALS Link to hear about ALS news and research:

https://bit.ly/3EH2eMT