Platform Patient Webinar
Trehalose EAP
An Expanded Access Protocol of Intravenous Trehalose Injection 90.5 mg/mL
Treatment of Patients with Amyotrophic Lateral Sclerosis

July 27, 2023
5:00 – 5:30pm EST
ACT for ALS - A new opportunity to expand access and collect real world data in parallel to clinical trials via EAP

Signed into law on Dec 23, 2021
Grants for Research on Therapies via Intermediate-Size EAPs for ALS

- NIH U01 grant mechanism
- MGH Healey Center receives the first grant
- Trehalose companion EAP will occur in parallel to Regimen E of the HEALEY ALS Platform Trial
- PIs: Babu, Berry, Paganoni
- 25 sites
- 70 ALS participants
- FPFV in Q1 2023
Multi-PIs – Healey Center for ALS at MGH

➢ Suma Babu, MBBS, MPH
  • Assistant Professor of Neurology, Harvard Medical School

➢ James Berry, MD, MPH
  • Winthrop Family Scholar in ALS Sciences
  • Averill Healey Endowed Chair in ALS
  • Director, MGH Neurological Clinical Research Institute (NCRI)

➢ Sabrina Paganoni, MD, PhD
  • Co-Director, MGH Neurological Clinical Research Institute (NCRI)
Study Design

➢ Planned enrollment: 70 pALS at up to 25 sites
➢ Weekly IV infusions of trehalose, 90.5 mg/mL, at a dose of 0.75 g/kg
➢ Infusions may take place at the study center or at home

Cohort 1 (Trehalose Naïve)
➢ Patients who do not qualify for any reasonably accessible ongoing clinical trial.

Cohort 2 (RGE OLE Rollover)
➢ Patients who have completed Regimen E of the HEALEY ALS Platform Trial and are not eligible for enrollment in another treatment regimen of the platform study.
Inclusion Criteria

1. Sporadic or familial ALS.
2. Age 18 years or older.
3. Cohort 1: Patients who do not qualify for any reasonably accessible ongoing clinical trial.
4. Cohort 2: Patients who have completed Regimen E and the open label extension (OLE) period of the HEALEY ALS Platform Trial, or completed Regimen E of the HEALEY ALS Platform Trial if the OLE is not available at the site, and are not eligible for enrollment in another treatment regimen of the platform study.
5. Capable of providing informed consent and complying with study procedures, in the Site Investigator’s (SI’s) opinion.
6. Participants have established care with a physician at a specialized ALS center involved in the study and will maintain this clinical care throughout the duration of the EAP.
7. Participants must have a life expectancy of at least 6 months in SI’s opinion.
Exclusion Criteria

1. Current diagnosis or healthcare professional-recommended treatment (medication, exercise or diet) of diabetes mellitus.
2. Screening glucose $\geq 140$ mg/dl.
3. Known hypersensitivity to trehalose.
5. Inability for participant to return to site for weekly drug administration, until approved for home infusions.
7. Participant with a history of any clinically significant or unstable medical condition or lab abnormality based on the SI’s judgment that may interfere with assessment of the study objectives, with safety or full participation.
8. Females who are pregnant or nursing or who plan to get pregnant during the course of the EAP.
9. Females of child-bearing potential, or males, who are unwilling or unable to use highly-effective methods of birth control.
10. Use of investigational treatments for ALS (as part of participation in a clinical trial or another EAP) within 5 half-lives (if known) or 30 days (whichever is longer) prior to the Screening Visit.
11. Permanent assisted ventilation (PAV), defined as more than 22 hours per day of noninvasive or invasive mechanical ventilation for more than seven consecutive days. The date of onset of PAV is the first day of the seven days.
12. Active cancer or history of cancer, except for the following: basal cell carcinoma or successfully treated squamous cell carcinoma of the skin, cervical carcinoma in situ, prostatic carcinoma in situ, or other malignancies curatively treated and with no evidence of disease recurrence for at least 3 years.
13. Presence of unstable psychiatric disease, cognitive impairment, dementia, or substance abuse that would impair ability of the participant to provide informed consent, in the SI’s opinion.
14. Patients who chose to take experimental medications and/or supplements, and that is the only reason they are not eligible for trials, won’t be eligible for the EAP.
Workflow for sites

Cohort 1
(Trehalose Naïve participants)

First 4 weekly infusions must be in person
Rest can be remote

Cohort 2
(RGE OLE rollover participants)

All infusions (including baseline) may be remote
Brief in-person visits for screening, Week 3 and EOS for biomarker and clinical outcome collection
Project Overview

Trehalose EAP

MGH
IRB
Vendors
25 Study Sites
Patient Community
NIH/ NINDS
Seelos Therapeutics

Central IRB (MGH)

25 Site IRBs
MGH Overview

Data Management

Systems

Project Management

MGH

Grants Administration

Quality Assurance
Vendors Overview

- Central Safety Lab
- Central Biomarker Lab
- IP Shipment Courier Services
- Data and Safety Monitoring
- Study Drug Handling and Shipping
- Site Monitoring and Training
- Data Standardization and Management
- Biostatisticians
- Home Infusion Nursing Company
First patient first visit occurred at MGH in March 2023

Key elements for site activation:

- Completion of all local requirements (IRB, infusion center, pharmacy, etc.)
- Fully executed Clinical Site Agreement (CSA)
- sIRB approval
- Regulatory document collection
Study Sites

Activated Sites As of 7/25/23
- Mass General Hospital
- Texas Neuro
- St. Alphonsus
- Holy Cross Hospital
- Virginia Commonwealth University
- Nova Southeastern University
- University of Iowa
- Washington University
- University Colorado
- Hospital for Special Care
- George Washington University
- SUNY Upstate Medical University
- University of California, Irvine
- University of Minnesota

Trehalose EAP

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<th>Sites</th>
<th>Planned</th>
<th>Actual as of 7/17/23</th>
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<tbody>
<tr>
<td></td>
<td>25</td>
<td>14</td>
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<tr>
<td>Nearing activation: 2</td>
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<table>
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<th>Participants</th>
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<td>In screening/scheduled: 25</td>
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Site Enrollment Progress

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<th>Total Enrollment as of 7/25/23</th>
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Thank you!

Contact:

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