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.
Common Protocol and Shared Infrastructure Allow for Operational and Scientific Efficiencies

- 1 Protocol (Phase 2/3)
- 1 single IRB
- Central Governance
- 7 Regimens
- 70+ Enrolling Sites
- ~1300 Participants

Regimen: Active Study Drug + Matching Placebo
Providing research access across a diverse network of 70+ NEALS sites
The HEALEY ALS Platform Trial is a perpetual trial to provide decisive answers and direction with efficient execution.

<table>
<thead>
<tr>
<th>Regimen</th>
<th>Treatment</th>
<th>Time Period</th>
</tr>
</thead>
<tbody>
<tr>
<td>Regimen A</td>
<td>Zilucoplan by UCB</td>
<td>Summer 2020</td>
</tr>
<tr>
<td>Regimen B</td>
<td>Verdiperstat by Biohaven</td>
<td>2021 - 2022</td>
</tr>
<tr>
<td>Regimen C</td>
<td>CNM-Au8 by Clene</td>
<td>Consider Phase 3 Trial</td>
</tr>
<tr>
<td>Regimen D</td>
<td>Pridopidine by Prilenia</td>
<td>2021 - 2022</td>
</tr>
<tr>
<td>Regimen E</td>
<td>Trehalose by Seelos</td>
<td>Consider Phase 3 Trial</td>
</tr>
<tr>
<td>Regimen F</td>
<td>ABBV-CLS-7262 by Calico &amp; AbbVie</td>
<td>2021 - 2022</td>
</tr>
<tr>
<td>Regimen G</td>
<td>DNL343 by Denali</td>
<td>2021 - 2022</td>
</tr>
</tbody>
</table>
Many people living with ALS cannot participate in clinical trials

**Expanded Access**

**FDA Definition:**

- Sometimes called “compassionate use”
- Potential pathway for a patient with an immediately life-threatening or serious disease or condition
- Allows access to an investigational medical product outside of clinical trials
- Used when no comparable or satisfactory alternative therapy available

[https://www.fda.gov/news-events/public-health-focus/expanded-access](https://www.fda.gov/news-events/public-health-focus/expanded-access)
Categories of Expanded Access Protocols (EAPs)

- **Individual Patient**, including emergency use
- **Intermediate-size Patient Populations**
- **Treatment Protocol** (large patient population)

Requirements for All EAPs

- Patient has a **serious** or **immediately life-threatening** disease or condition; no comparable or satisfactory alternative therapy
- Potential benefit justifies potential risk; potential risk not unreasonable in context of disease or condition
- Providing drug will **not interfere with clinical trials** that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use
Community Support to EAPs

EAP COMPANION PROGRAM SUPPORTERS

- Healey Center for ALS
- Clearing Corporation Charitable Foundation
- Eddie and Jo Allison Smith Family Foundation
- Richard Stravitz Foundation
- I AM ALS
- Biohaven
- Cleene
- Prilenia
- Elliott & Frantz, Inc.

- Community Fundraisers
  - Tackle ALS – Team Change ALS
  - Ellen Corinidia’s Fundraiser
  - 2019 Olson Cornohole Tournament
  - 2019 Worthington Fore ALS
  - 2020 and 2021 Fishing for ALS Warriors
  - 2020 and 2021 tALSe For a Cure Pick Your Own Path Walk

- 2020 and 2021 Lori’s Shoes “Hope Is In the Bag”
- 2021 and 2022 MLB Lou Gehrig Day 4-ALS
- 2021 Russ Pallesen Fundraiser for EAP
- 2021 Voices for ALS Golf Tournament
- 2021 The Martha Olson-Fernandez Foundation Golf Tournament
- 2021 Gwendolyn Strong Walk
- TechVs ALS

• Big thanks to countless individual contributors
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 received the first grant
- Trehalose companion EAP to occur in parallel to Regimen E of the HEALEY ALS Platform Trial
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
  • Associate Professor of PM&R, Harvard Medical School
  • Co-Director, MGH Neurological Clinical Research Institute (NCRI)
Healey EAP Companion Program Growth

2018
Single-center EAPs began at MGH Healey Center

2021
Multi-center companion EAPs began at 9 sites across the US

2023
Trehalose EAP expanded to 25 planned sites across the US

Looking ahead
Launching new EAPs at more sites
Healey EAP Companion Program – 2018
Healey EAP Companion Program – 2021

Key
- MGH Healey Center EAPs
- Verdiprstat EAP
- CNM-Au8 EAP
- Pridopidine EAP
Healey EAP Companion Program – 2023

Key
- MGH Healey Center EAPs
- Verdiperstat EAP
- CNM-Au8 EAP
- Pridopidine EAP
- Trehalose EAP - Activated
- Trehalose EAP - In start up
Trehalose EAP Overview

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

Subsections:
- Central IRB (MGH)
- 25 Site IRBs
Trehalose EAP Overview

- **Startup**
  - Q4 2022 > Q1 2023

- **Enrollment**
  - Q1 2023 > Q1 2024

- **Treatment Follow-up**
  - Q1 2023 > Q3 2024

- **Closeout and Reporting**
  - Q4 2024 > Q1 2025

This EAP may be impacted by results of the Healey ALS Platform Trial RGE
Patient Navigation
Central resource for people living with ALS

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:
October 5th - canceled for NEALS conference
October 12th - weekly Q&A