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.
Building Community & Partnership in ALS Research

**Patient Navigator: Central Resource**
- 2,614 Total emails/phone calls/zoom calls with ALS families
- 630 Uses of the Online Eligibility Checking Tool
- 39 Countries in contact about research

**Weekly Webinars: News & Updates**
- 115 Public Q&A webinars hosted to date
- 50+ Guest speakers featured
- 8,317 Total attendees, 71 Weekly average
- 40,553 Total views on YouTube

**Drug Science Q&A Webinars**
- 6 Webinars hosted (Regimens A,B,C,D,E,F)
- 8,481 Total views on YouTube
- 242 Questions answered live

(Data Collected Oct 2020-Mar 2023)
Global Reach of Patient Navigation: 39 Countries
Online Eligibility Checking Tool

Find Out If You're Eligible

We’ve prepared a short list of questions to help you find out if you might be eligible to participate in the HEALEY ALS Platform Trial.

Please note that the result of this survey does not serve as official confirmation of your eligibility, as many factors are considered prior to study participation (view a full list of inclusion / exclusion criteria here). Determining eligibility for the Platform Trial will depend on a thorough assessment of your clinical symptoms, review of past medical history, and lab work that can only be performed by an investigator (doctor or nurse practitioner) on the trial.

Eligibility questions

https://bit.ly/3ezu4Qx
Who are we?

Contact the Patient Navigator
HEALEYALSPlatform@mgh.harvard.edu
833-425-8257 (HALT ALS)

Catherine Small
Patient Navigator

Allison Bulat
Community Engagement

Judi Carey, RN
Research Access Nurse

HEALEY ALS Platform Trial:

Common Protocol and Shared Infrastructure

- Regimen A
- Regimen B
- Regimen C
- Regimen D
- Regimen E
- Regimen F
- Regimen G

Screen for eligibility → Randomization 3:1 → Active

Placebo → Active

Screening Period → Randomized Period (24 weeks) → Open-Label Extension Period (Active Treatment Extension)

ENROLLMENT COMPLETE

ENROLLING

IN START-UP
48 Sites Currently Active for Regimen F

(as of 5/11/23)

- Nova Southeastern University
- Essentia Health
- Texas Neurology
- Mass General Hospital
- University of Nebraska
- Hospital for Special Care
- Henry Ford Hospital
- Augusta University
- Beth Israel Deaconess
- University of Texas HSC
- University of Colorado
- Loma Linda University
- Ohio State University
- Cedars Sinai Medical Center
- Duke University
- Wake Forest University
- Saint Alphonsus
- UMass Worcester
- Lehigh Valley
- Thomas Jefferson
- University of South Florida
- University of Pennsylvania
- SUNY Upstate
- University of Iowa
- California Pacific Med Center
- Houston Methodist
- Vanderbilt University
- University of Minnesota
- Washington University
- Barrow Neurological Institute

Site Map & Contacts:

https://bit.ly/3g2NMr5
Checking Site Status Online

List of Participating Sites

Many sites are expected to start enrolling for Regimen F soon. Sites marked "Recruiting" are currently enrolling participants.

Sites marked "Active, Not recruiting" are active in the Platform Trial (for example, they are following participants in ongoing regimens that have already completed enrollment) but are not enrolling new participants at this time.

<table>
<thead>
<tr>
<th>Site</th>
<th>State</th>
<th>Enrollment Status</th>
<th>Trial Contact Information</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mayo Clinic Florida</td>
<td>FL</td>
<td>Active, Not recruiting</td>
<td>Jany Pauletti</td>
</tr>
<tr>
<td>Nova Southeastern University</td>
<td>FL</td>
<td>Recruiting</td>
<td>Donovan Mott</td>
</tr>
</tbody>
</table>

Contact a study team near you to discuss enrollment opportunities.

https://bit.ly/3g2NZr5
Regimen F Resources on MGH Website

Regimen F: ABBV-CLS-7262, by Calico and AbbVie - Now Recruiting

ABBV-CLS-7262 is an investigational drug developed by Calico Life Sciences LLC in collaboration with AbbVie Inc. ABBV-CLS-7262 aims to restore function in cells affected by ALS by normalizing protein synthesis and preventing further sequestration and aggregation of TDP-43, thereby protecting neurons, and possibly slowing ALS progression.

The integrated stress response (ISR) is a fundamental transient process that regulates cell function during various stressful conditions. Tissue studies suggest that the ISR is chronically induced in people with ALS. It is proposed that TDP-43 aggregates, a hallmark feature in the motor neurons of people with ALS, could be formed by a chronically induced ISR. ABBV-CLS-7262 activates the protein complex elf2B, which is a key regulator of the ISR. Binding of ABBV-CLS-7262 desensitizes elf2B to stress and decreases the ISR. Reduction of the ISR restores normal protein synthesis, reduces TDP-43 sequestration in stress granules, and may decrease TDP-43 aggregation.

A prior first-in-human study of ABBV-CLS-7262 showed that this drug was well-tolerated by participants, demonstrated target engagement by increasing elf2B enzymatic activity, and suppressed the ISR in blood cells. ABBV-CLS-7262 crossed the blood brain barrier at concentrations predicted to be efficacious in ALS. ABBV-CLS-7262 is currently being investigated in a Phase 1b study in people with ALS (NCT04948645), and will be studied further as part of the HEALY ALS Platform Trial.

Watch this video for more information on the mechanism of action behind ABBV-CLS-7262.

Download brochure

Regimen F Drug Science Q&A Webinar

Topic: Regimen F Drug Science and Mechanism of Action

Recording Available: https://bit.ly/3mQy5qQ
Why lumbar puncture and CSF biomarkers are important to ALS therapeutic development

Presenter: Nicholas J. Maragakis, M.D., Johns Hopkins University

View Here:
https://bit.ly/3Mc1HZt

Recording are available under “educational webinars” on neals.org
Upcoming Webinars:

May 18th - Weekly Q&A and overview of FDA approved medications for ALS
May 25th - Weekly Q&A and overview of Platform Trial progress to date
June 1st - Weekly Q&A

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