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.

HEALEY ALS Platform Trial

Weekly Q&A – May 11, 2023

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

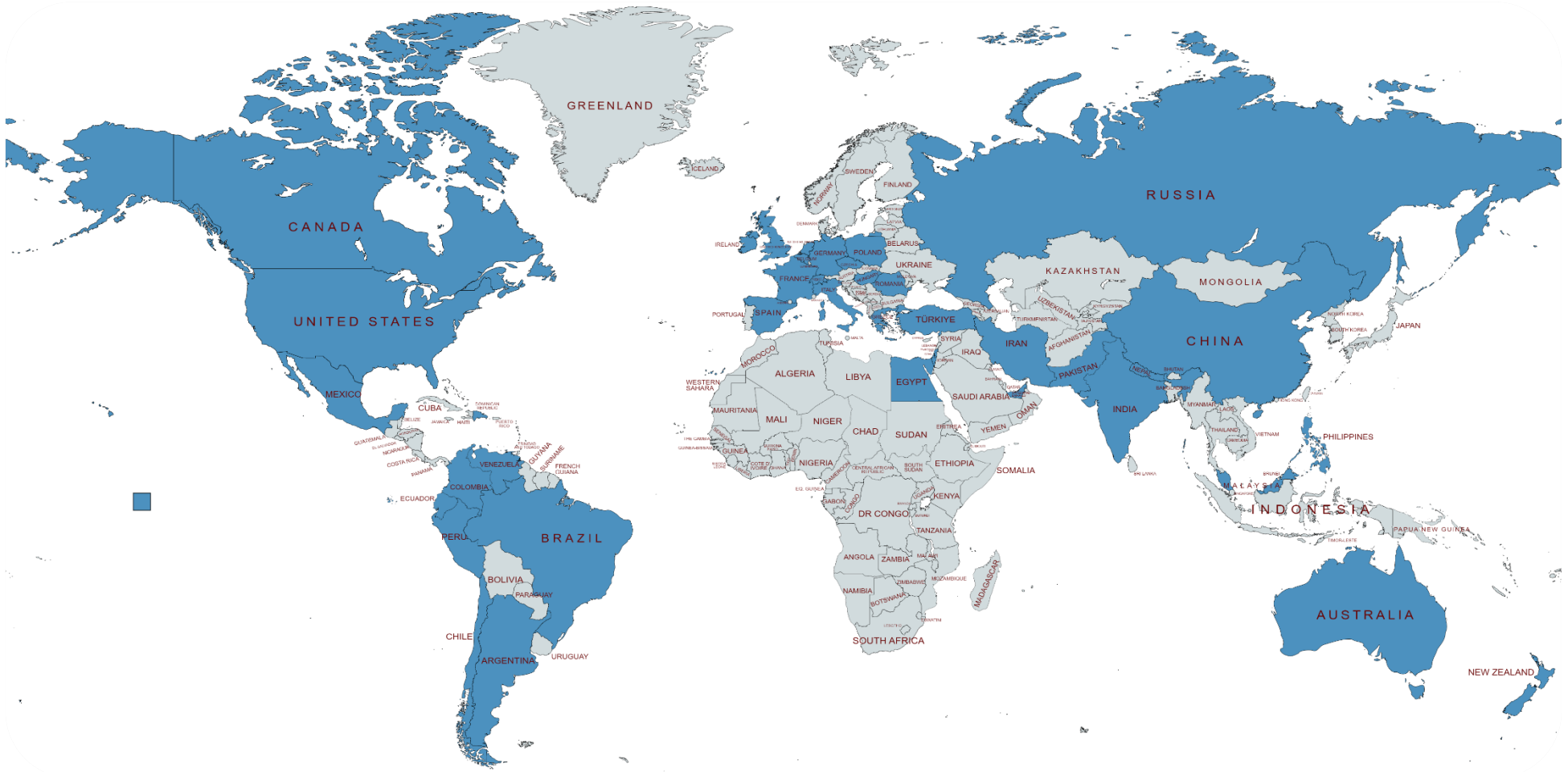
Drug Science Q&A Webinars

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

Argentina
Australia
Belgium
Brazil
Canada
Chile
China
Columbia
Czech Republic/ Czechia
Dominican Republic
Ecuador
Egypt
England/UK
France
Germany
Greece
Hungary
India
Iran
Ireland
Israel/Palestine
Italy
Malaysia
Mexico
Nepal
Netherlands
New Zealand
Pakistan
Peru
Philippines
Poland
Romania
Russia
Spain
Switzerland
Turkey
United Arab Emirates
United States
Venezuela



Online Eligibility Checking Tool

Find Out If You're Eligible

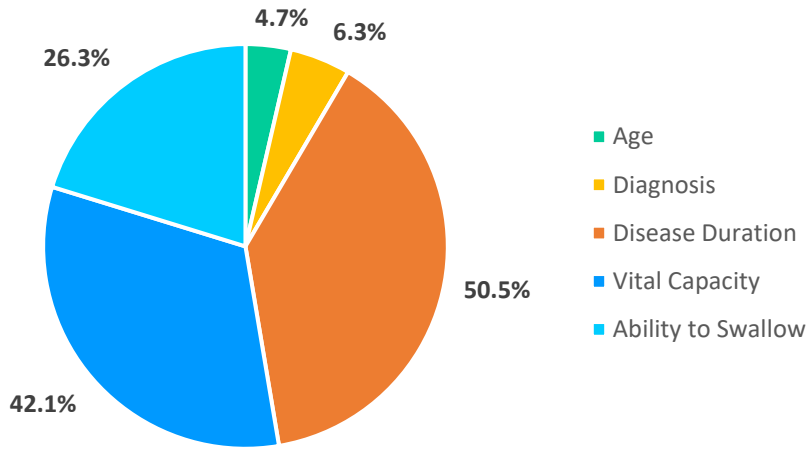
We've prepared a short list of questions to help you find out if you might be eligible to participate in the HEALEY ALS Platform Trial.

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

Reasons for "Not Eligible" Survey Responses



Total survey responses generated between 5/10/21 and 4/18/23	650
How many total completed survey responses?	644
How many people were eligible based on survey (all yes)?	454
How many were ineligible (one or more no)?	190
Reasons for "No"	
18 or older?	9
Dx with ALS?	12
Symptom onset within 36mos?	96
VC at or above 50%?	80
Ability to swallow pills and liquids?	50

Who are we?

Catherine Small

Patient Navigator

Allison Bulat

Community Engagement

Judi Carey, RN

Research Access Nurse

Contact the Patient Navigator

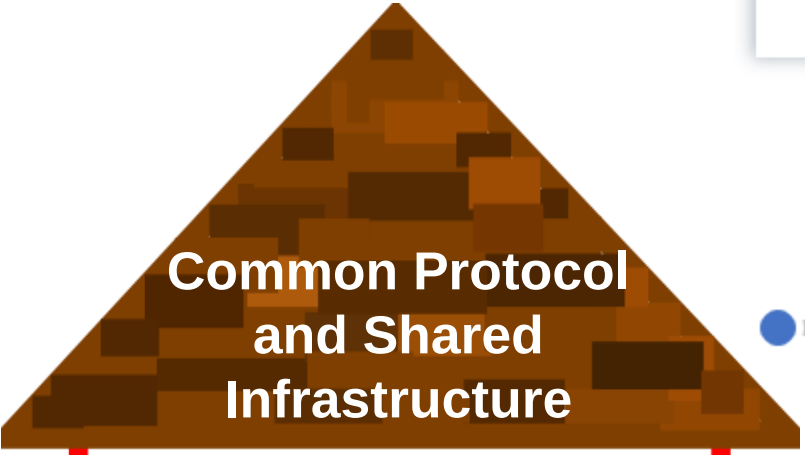
HEALEYALSPlatform@mgh.harvard.edu

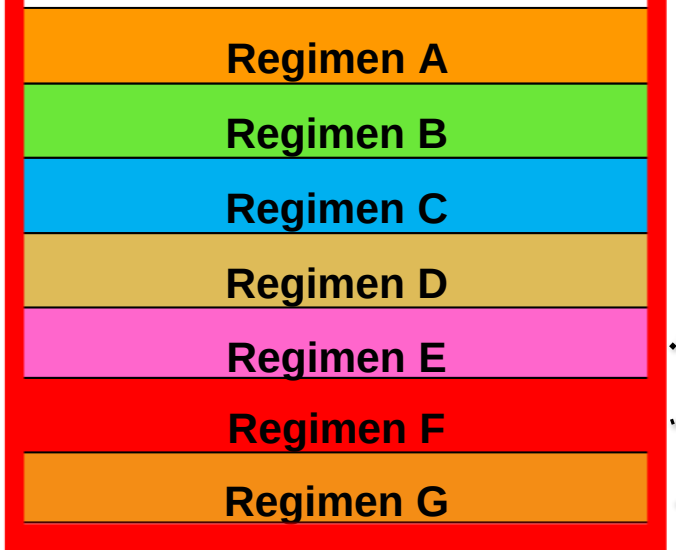
833-425-8257 (HALT ALS)

https://bit.ly/3UPTzR9

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

HEALEY ALS Platform Trial:



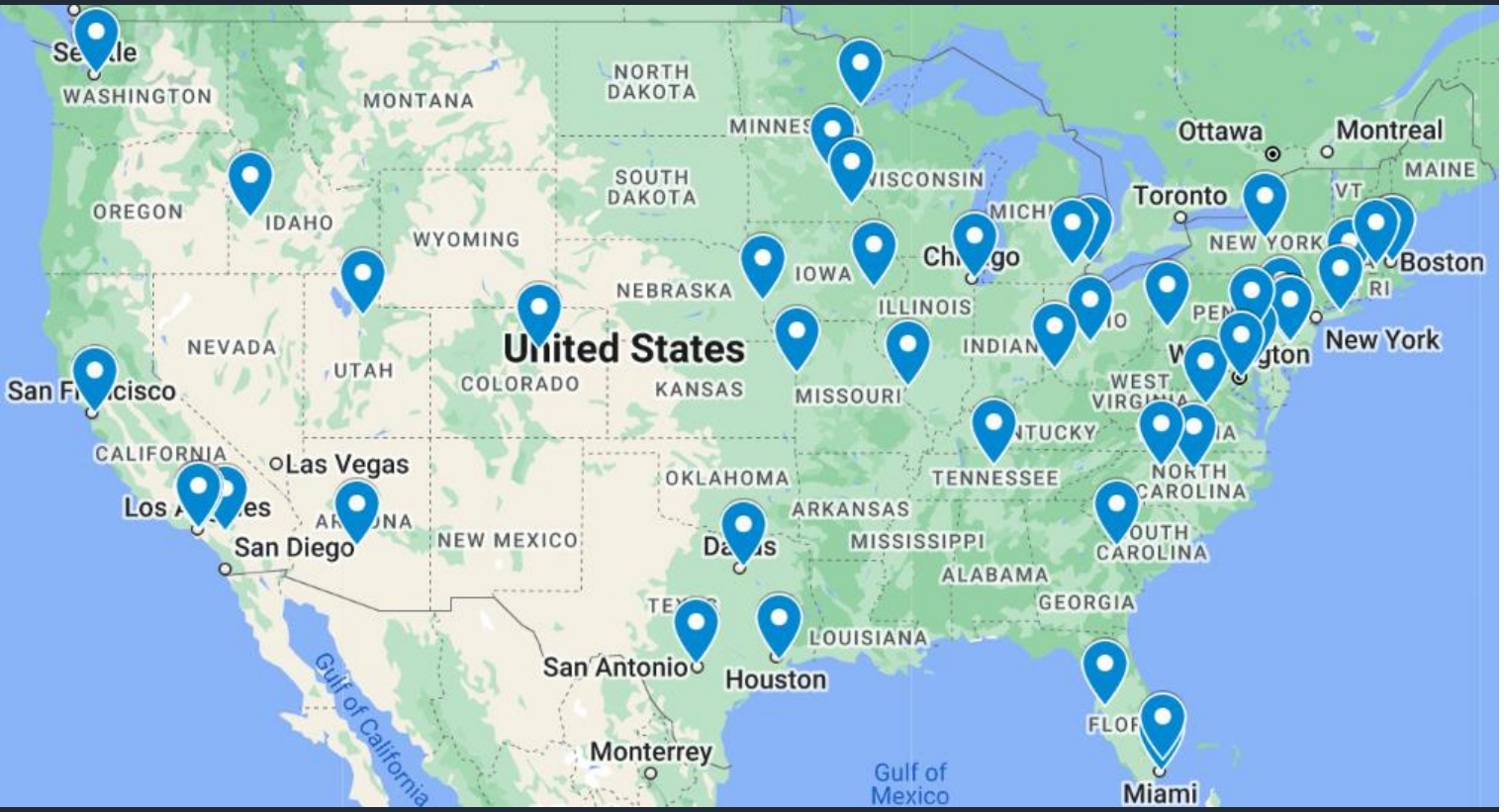


SEELOS THERAPEUTICS | ENROLLMENT COMPLETE

Calico | ENROLLING

DENALI THERAPEUTICS | 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
- University of Miami
- Temple University
- University of Virginia
- Johns Hopkins University
- University of Southern CA
- Holy Cross Hospital
- University of Washington
- University of Utah
- Penn State Hershey
- University of Michigan
- University of Kansas
- Stony Brook University
- University of Cincinnati
- Mayo Clinic Rochester
- Northwestern University
- Georgetown University
- Kaiser, Los Angeles
- UPMC

Site Map & Contacts:

https://bit.ly/3g2NZr5

Checking Site Status Online

Contact a study team near you to discuss enrollment opportunities

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.

Site	State	Enrollment Status	Trial Contact Information
Mayo Clinic Florida	FL	Active, Not recruiting	Jany Paulett
Nova Southeastern University	**FL**	**Recruiting**	**Donovan Mott**

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 eIF2B, which is a key regulator of the ISR. Binding of ABBV-CLS-7262 desensitizes eIF2B 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 eIF2B 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 HEALEY ALS Platform Trial.

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

Download brochure

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

NEALS Northeast Amyotrophic Lateral Sclerosis Consortium

HEALEY ALS Platform Trial

Regimen F

ABBV-CLS-7262

Developed by Calico Life Sciences LLC in collaboration with AbbVie Inc.

Investigational products included in the HEALEY ALS Platform Trial are selected by a team of experts after careful review of the study drug and the science supporting its treatment potential in Amyotrophic Lateral Sclerosis (ALS). Regimen F is testing an experimental medication called ABBV-CLS-7262, and the trial will involve in-person study visits every 4 to 8 weeks (about 6 visits total over the course of 24 weeks).

Please discuss the possible benefits and risks of this investigational product with your study team.

Visit our website to learn more about what to expect in the trial process:
https://bit.ly/3ExRal8

About Regimen F:

Regimen F is a Phase 2/3 trial enrolling approximately 240 participants to evaluate the safety and efficacy of ABBV-CLS-7262 as a potential treatment for ALS. This regimen involves biomarker analysis and cerebrospinal fluid collection via lumbar punctures to assess the effects of ABBV-CLS-7262.

3:1 Active Drug to Placebo Ratio: Participants who enroll in this trial have a 3 in 4 (75%) chance of being assigned to active study drug and a 1 in 4 (25%) chance of being assigned to placebo during the initial 24-week randomized controlled trial (RCT) period.

Active Treatment Extension (ATE): Participants have the option to enroll in the ATE for ABBV-CLS-7262 upon completion of the 24-week RCT. During ATE, all participants will receive the 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)

https://bit.ly/3SIwH4X

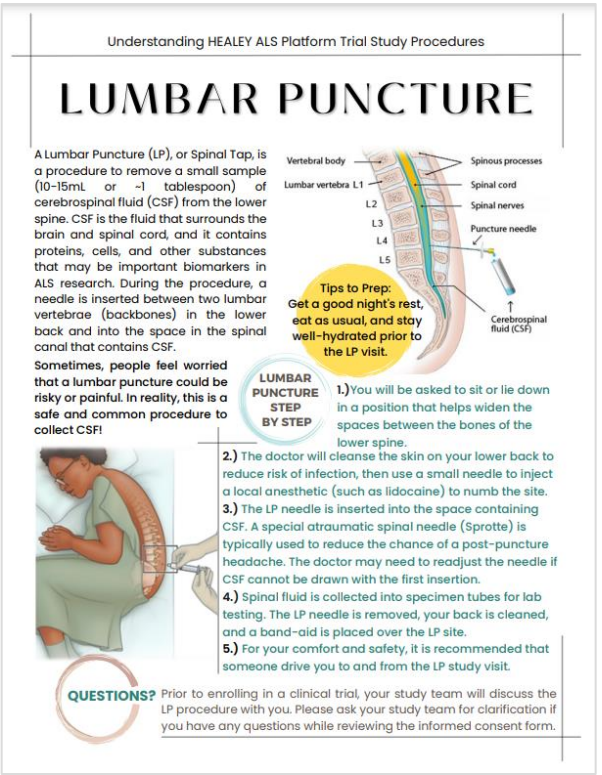
Printable Brochures!

↓

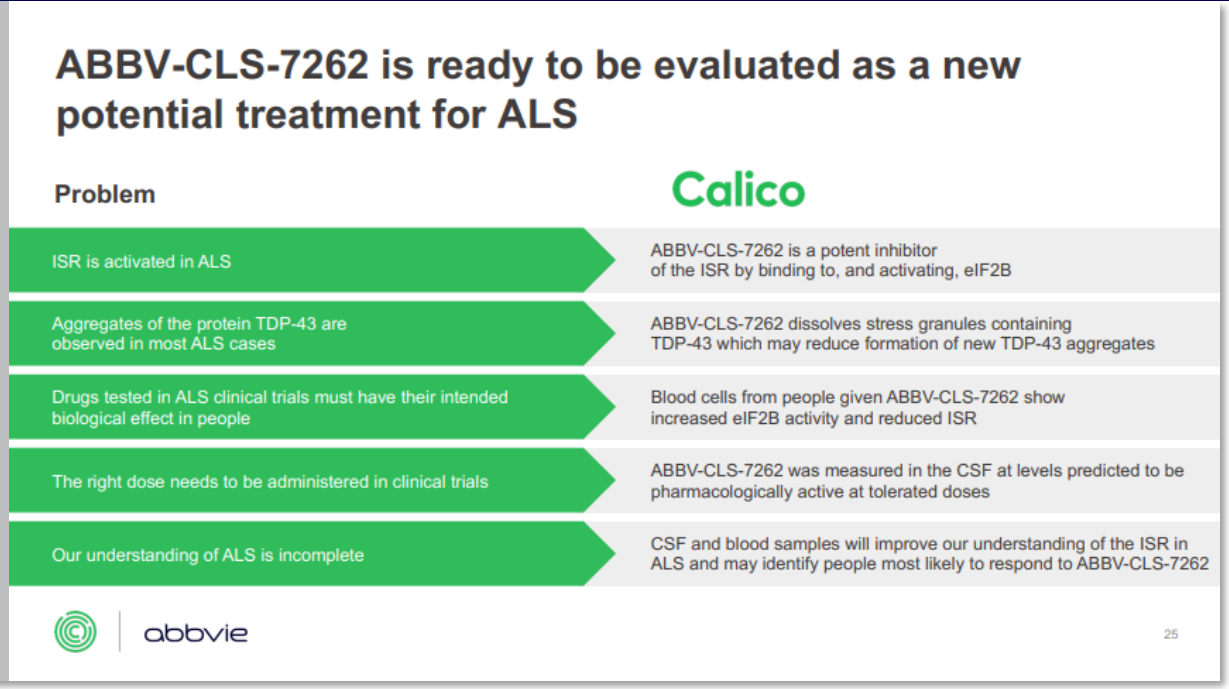
Regimen F Brochure

Lumbar Puncture Brochure

General Platform Trial Brochure



Regimen F Drug Science Q&A Webinar



Topic: Regimen F Drug Science and Mechanism of Action

Recording Available: https://bit.ly/3mQy5qQ

Calico

The ALS Association/Northeast ALS Consortium Educational Webinar

Why lumbar puncture and CSF biomarkers are important to ALS therapeutic development

Recording Available!

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

View Here:

https://bit.ly/3Mc1HZt

Recording are available under "educational webinars" on neals.org

Patient Navigation
Central resource for people living with ALS

Catherine Small

Allison Bulat

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:

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