

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 – February 15, 2024



Healey & AMG Center

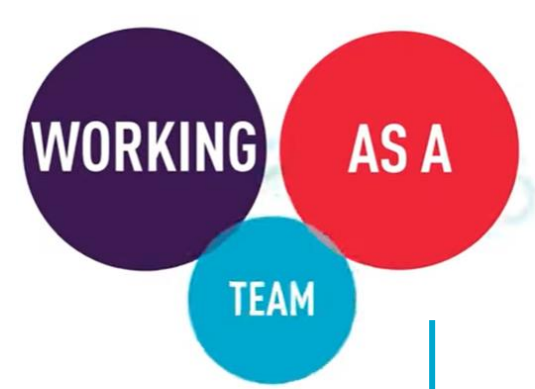
Sean M. Healey & AMG Center for ALS
at Massachusetts General Hospital



Calico



The AMG Foundation



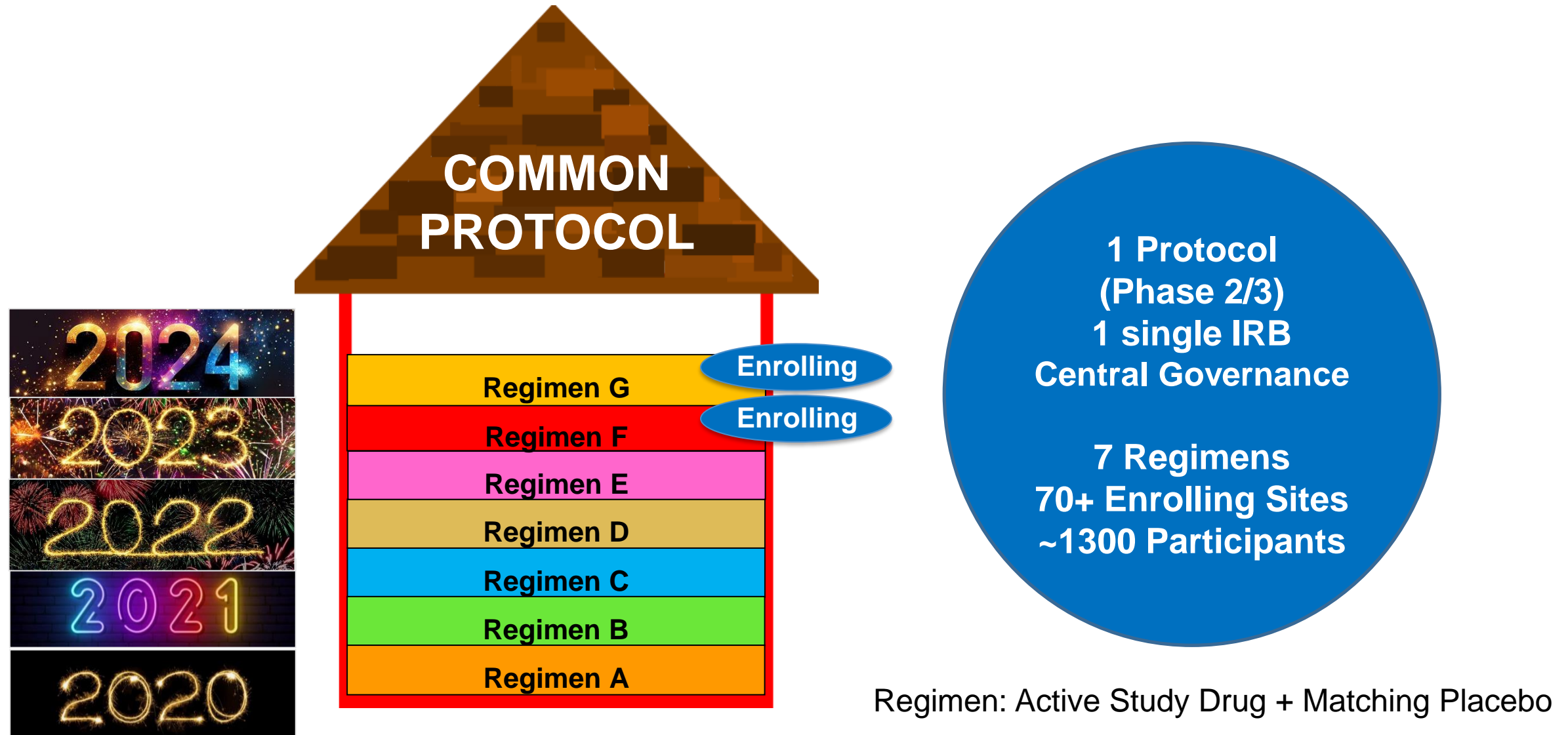
This trial is **dedicated to all people living with ALS**, their families, and friends. Your **partnership** in research is what keeps us filled with passion, dedication, and the commitment to develop new treatments for ALS

This breakthrough trial would not be possible without your participation.

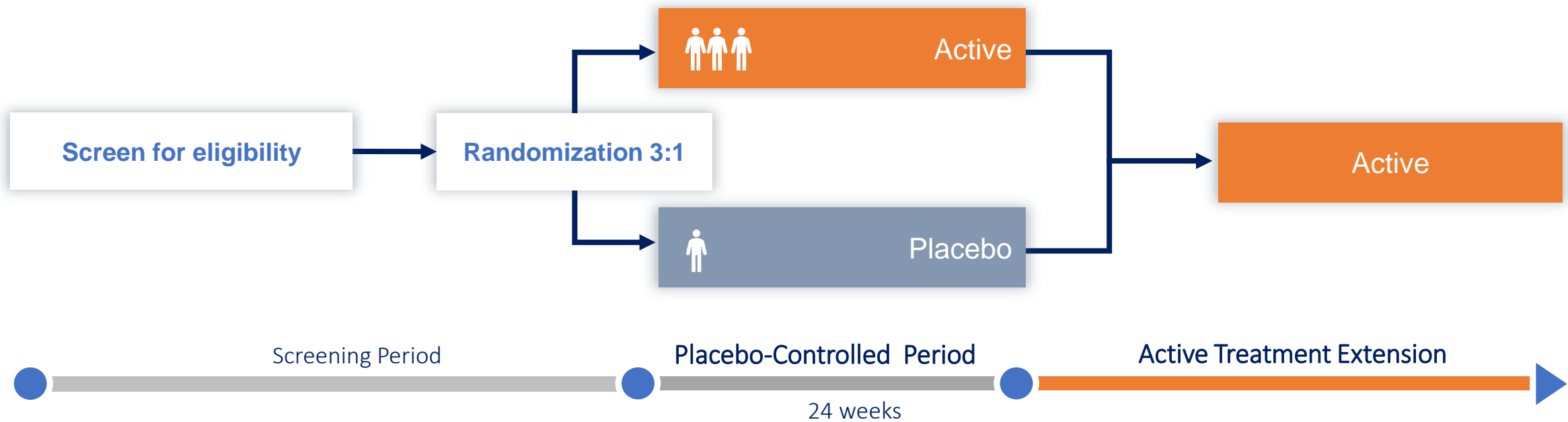
Every research participant, whether on the active drug or placebo, plays a critical role in making the hope of finding a cure for ALS a reality

Thank You

Common protocol and shared infrastructure allow for operational and scientific efficiencies



The objective of each regimen is to provide a go / no go decision to inform the clinical development program of the regimen study drug



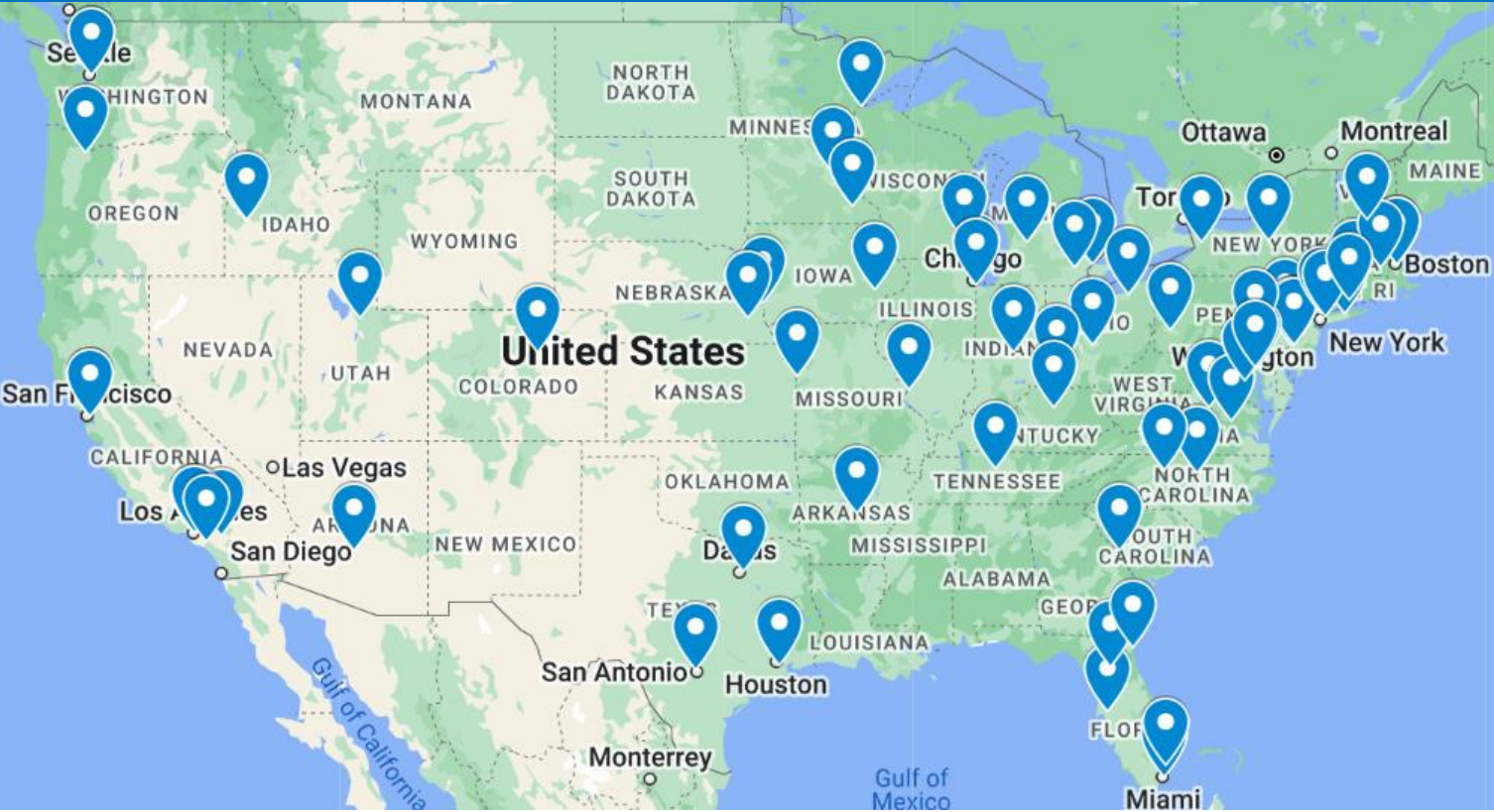
Primary Endpoint (Placebo-Controlled Period)

Change from baseline through week 24 in disease severity as measured by the ALSFRS-R total score and survival

Safety, Secondary, and Exploratory Endpoints

(respiratory function, muscle strength, survival, biomarkers + regimen-specific endpoints)

Providing research access across a diverse network of 70+ NEALS sites

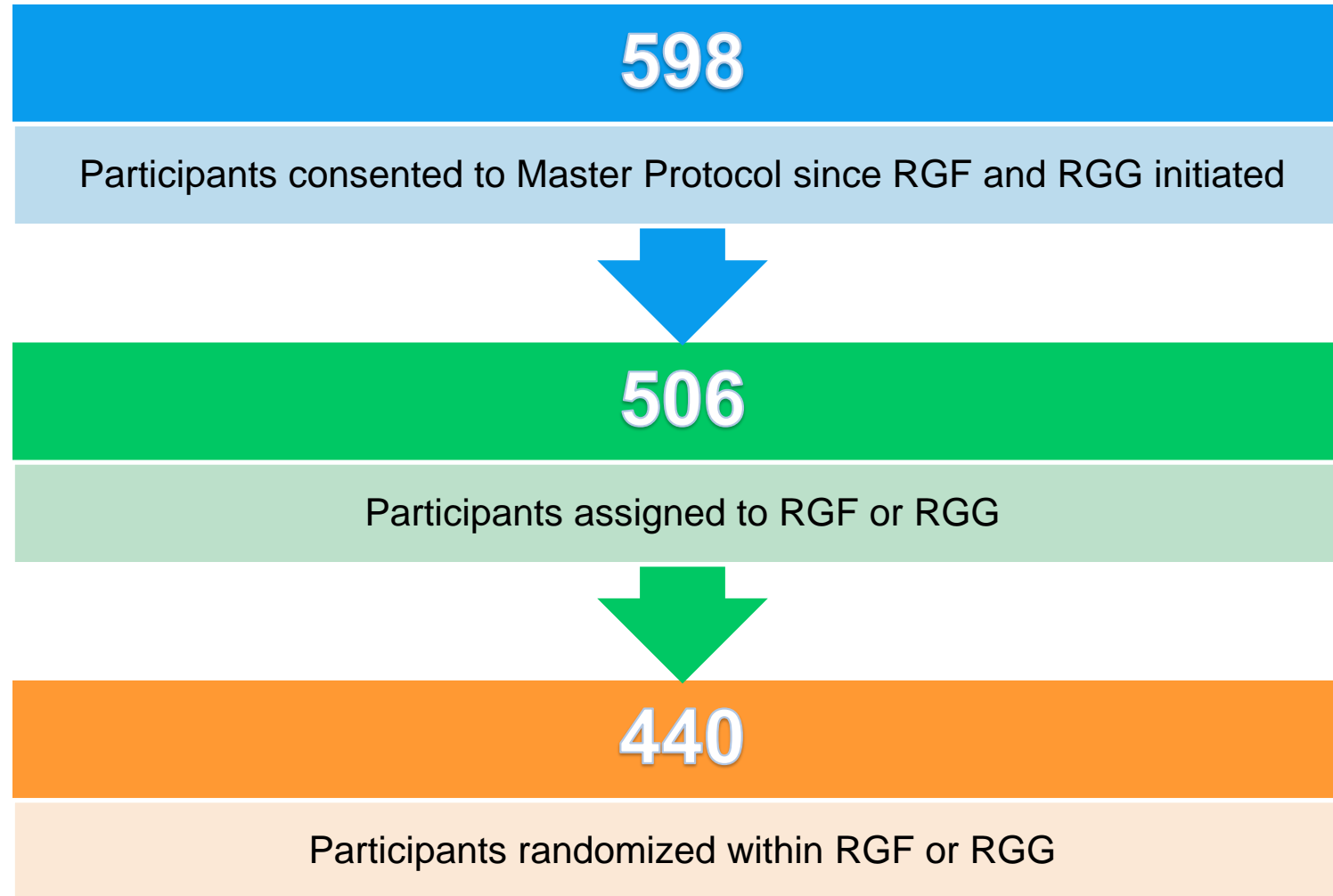


Contact a study team near you to discuss enrollment opportunities



<https://bit.ly/3g2NZr5>

Enrollment Update: Regimen F and Regimen G

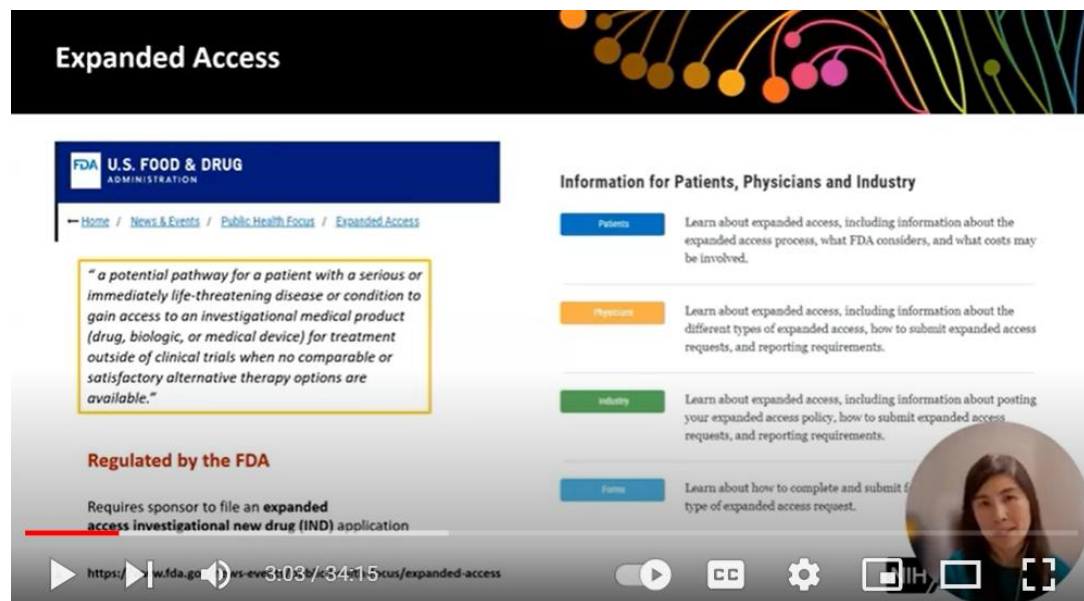


Thank You

for your partnership in ALS research

(as of 2/15/24)

Monthly Expanded Access Q&A Webinars



Expanded Access

U.S. FOOD & DRUG ADMINISTRATION

Home / News & Events / Public Health Focus / Expanded Access

"a potential pathway for a patient with a serious or immediately life-threatening disease or condition to gain access to an investigational medical product (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available."

Regulated by the FDA

Requires sponsor to file an **expanded access investigational new drug (IND)** application

Information for Patients, Physicians and Industry

- Patients** Learn about expanded access, including information about the expanded access process, what FDA considers, and what costs may be involved.
- Physicians** Learn about expanded access, including information about the different types of expanded access, how to submit expanded access requests, and reporting requirements.
- Industry** Learn about expanded access, including information about posting your expanded access policy, how to submit expanded access requests, and reporting requirements.
- Forms** Learn about how to complete and submit for a type of expanded access request.

https://www.fda.gov/oc/expanded-access

February 8, 2024 –

EAP Talk with Dr. Rick Bedlack and Gwen Petersen

Recording: <https://bit.ly/49wySQn>

January 11, 2024 –

EAP Discussion with the NIH

Recording: <https://bit.ly/3HvfJCz>

December 14, 2023 –

EAP Update with Dr. James Berry (MGH)

Recording: <https://bit.ly/3UfCAJU>



March 14, 2024 – Thurs 5:00-5:30 EST

EAP Update with Dr. Jinsy Andrews (Columbia)

Expanded Access Resources and Webinar Opportunities

Join us for updates about Expanded Access and EAPs **on the second Thursday of each month!**

(or view recordings online)



<https://bit.ly/3r6Nd2L>

Visit the NEALS website to explore upcoming educational webinar opportunities or view previous recordings



<https://bit.ly/3S4P0Sb>

Find more webinar opportunities and recordings through the **International Alliance of ALS/MND Associations**



<https://bit.ly/3Obj2m6>



Upcoming NEALS Educational Webinars

March 1, 2024

The Benefits of Macronutrients in People Living with ALS: What Should We Eat?

Dr. Ikjae Lee, from the Eleanor and Lou Gehrig ALS Center at Columbia University, will present a summary of literature on the intake of various macronutrients and their impact on the progression of ALS. Building on this foundation, Dr....

March 18, 2024

The Science Behind ALS Treatments: Riluzole, Radicava, and Relyvrio Description

Moderated by Dr. Senda Ajroud-Driss, this webinar for both clinicians and people living with ALS will review the 3 drugs that are currently FDA-approved in the United States for the general ALS population. Panelists, Dr. Lauren Elman,...

Register Here:



<https://bit.ly/3S4P0Sb>

*Recording will later be available under
“educational webinars” on neals.org*

Patient Navigation

Central resource for people living with ALS



Catherine Small

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:

February 22nd- Genetics Discussion with Dr. Mark Garret (MGH)

February 29th- Weekly Q&A Featuring University of Chicago Study Team

March 7th- Weekly Q&A



Allison Bulat