

LES
TURNER
ALS
FOUNDATION

ALS
LEARNING
SERIES

Inclusion Support & Education: ALS Clinical Research



Allison Bulat
Strategic Advisor of Community Engagement
Sean M Healey & AMG Center for ALS at MGH
NEALS



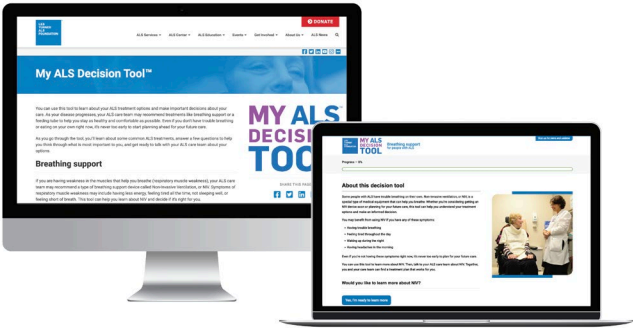
Judi Carey, RN
Research Access Nurse Manager
Sean M Healey & AMG Center for ALS at MGH
NEALS

**The ALS Learning Series is made possible because of the
Gilbert & Jacqueline Fern Foundation and our industry partners.**



Mitsubishi Tanabe Pharma
America

Online Education Tools



My ALS Decision Tool™ can help you choose ALS care that's in line with your needs and values. This interactive tool explains ALS treatment options and includes reflection questions to help you decide what's right for you.



Looking for in-depth information about ALS symptoms and care options? Les Turner ALS Foundation has you covered. We've created guides about key topics like nutrition, communication, mobility, and more.

ALS LEARNING SERIES

Our online ALS Learning Series aims to empower the ALS community through the latest information and insights. Educational webinars and interactive Q&A's covering a diverse array of topics, from nutrition to respiratory care, are offered monthly.

Les Turner Symposium on ALS

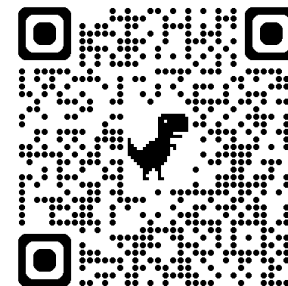
Monday, November 4, 2024



Join virtually or in person as leading ALS researchers, clinicians, advocates, and people living with ALS gather to discuss the latest research and perspectives on ALS.

It's an opportunity to ask questions, share insights, and engage with people striving to deliver the best possible ALS care – and it's a window into the future of treatment of the disease.

lesturnerals.org/symposium



Angela Genge, MD, FRCP(C)

“ We want to do whatever we can to facilitate the research and help find a cure for this disease so that others may live. ”

- Perry, a person with ALS, and his family



NATIONAL ALS REGISTRY

Learn how your participation can make a difference at cdc.gov/als



INCLUSION,
SUPPORT AND
EDUCATION:
ALS CLINICAL
RESEARCH



Judi Carey, RN
Research Access Nurse



Healey & AMG Center

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



NEALS

Northeast Amyotrophic
Lateral Sclerosis
Consortium®

Allison Bulat
Strategic Advisor -
Community Engagement

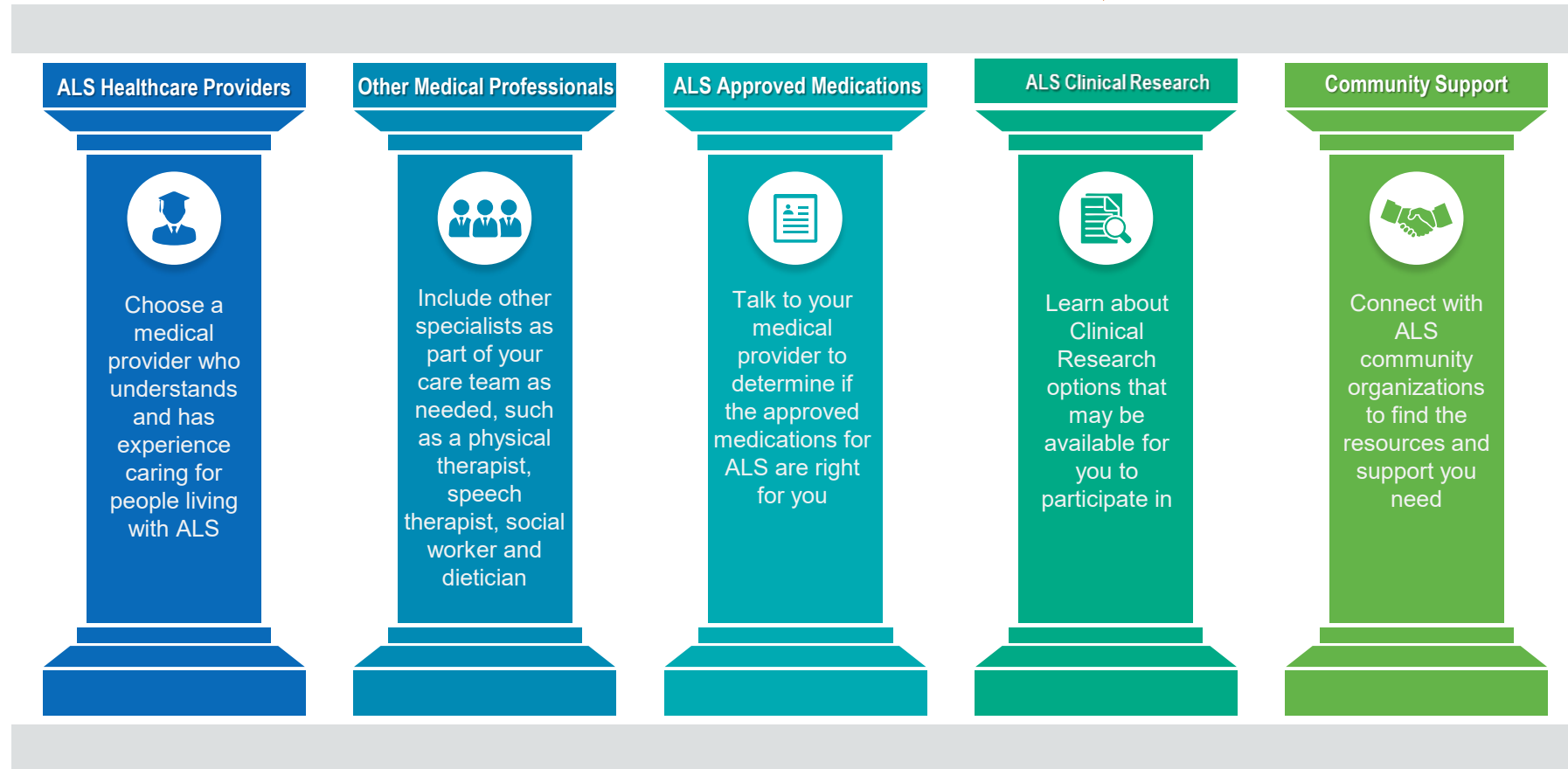


The Sean M. Healey and AMG Center for ALS at Mass General Hospital



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

Pillars of ALS Care



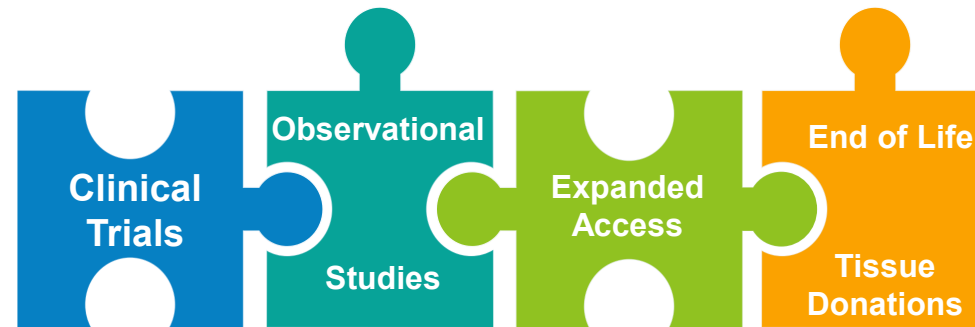
What is Clinical
Research, and why
is it important?

UNDERSTANDING CLINICAL RESEARCH

ALS Clinical Research

What is ALS Clinical Research?

ALS clinical research involves the participation of human volunteers in medical experiments and programs. Clinical research includes: 1) Clinical Trials 2) Observational Studies 3) Expanded Access 4) End of Life Tissue Donation Programs.



Why is ALS Clinical Research Important?

Clinical Research is the best way to get answers to questions about ALS including: What causes ALS? How can ALS be diagnosed sooner? How can progression of symptoms be slowed down? Can ALS symptoms be reversed? Can ALS be cured?

Observational Studies

This type of experiment is also called a biomarker study. In these experiments, volunteers may fill out surveys, provide samples of their blood or spinal fluid, or get an imaging test done of their brain. These studies are used to identify trends in people living with ALS and discover new possibilities for clinical trials.



Expanded Access

Expanded Access (EA), also referred to as Compassionate Use, is a pathway for people with ALS who do not qualify for a clinical trial, to access an investigational product (IP) that is not yet approved for everyone's use. An IP offered through EA is experimental, so its effectiveness as a treatment for ALS is not yet known.



Clinical Trials

A "clinical trial" also known as an "interventional trial" is a medical experiment performed on human volunteers. These experiments help researchers identify information about study drugs, special diets, or medical devices.

End of Life Tissue Donations

A donation of tissue (also referred to as brain and spinal cord) is a significant and impactful gift to ALS research. Each donation could lead to new discoveries and potential treatments for ALS.

What are the Phases of Clinical Trials, and what happens in each Phase?

CLINICAL TRIALS

The Phases of Clinical Trials



Phase I

A **Phase 1 trial** is the first test of an investigational product in a small group of human volunteers. The purpose of a phase 1 trial is to determine safety, tolerability and appropriate dosing for humans.



Phase II

A **Phase 2 trial** is the second test of an investigational product in a group of human volunteers. The purpose of a phase 2 trial is to continue to monitor safety and appropriate dosing for humans, and to begin to understand the potential biological effects of the investigational product.



Phase III

A **Phase 3 trial** is the third test of an investigational product, performed on a larger group of human volunteers than in previous phases. The purpose of a phase 3 trial is to continue to monitor safety, appropriate dosing for humans, and confirm the beneficial responses or outcomes in the prevention, treatment or progression of a disease (therapeutic efficacy) observed in the phase 2 trial. Confirmation of therapeutic efficacy can result in a request for drug approval being submitted to the FDA (Federal Drug Administration).



Phase IV

A **Phase 4 trial** occurs after approval of the investigational product by the FDA (Federal Drug Administration). The purpose of a phase 4 trial is to determine long-term safety and effectiveness and to identify adverse effects that may not have been apparent in prior trials. This phase occurs between pharmaceutical companies and the FDA and does not involve human volunteers and researchers.

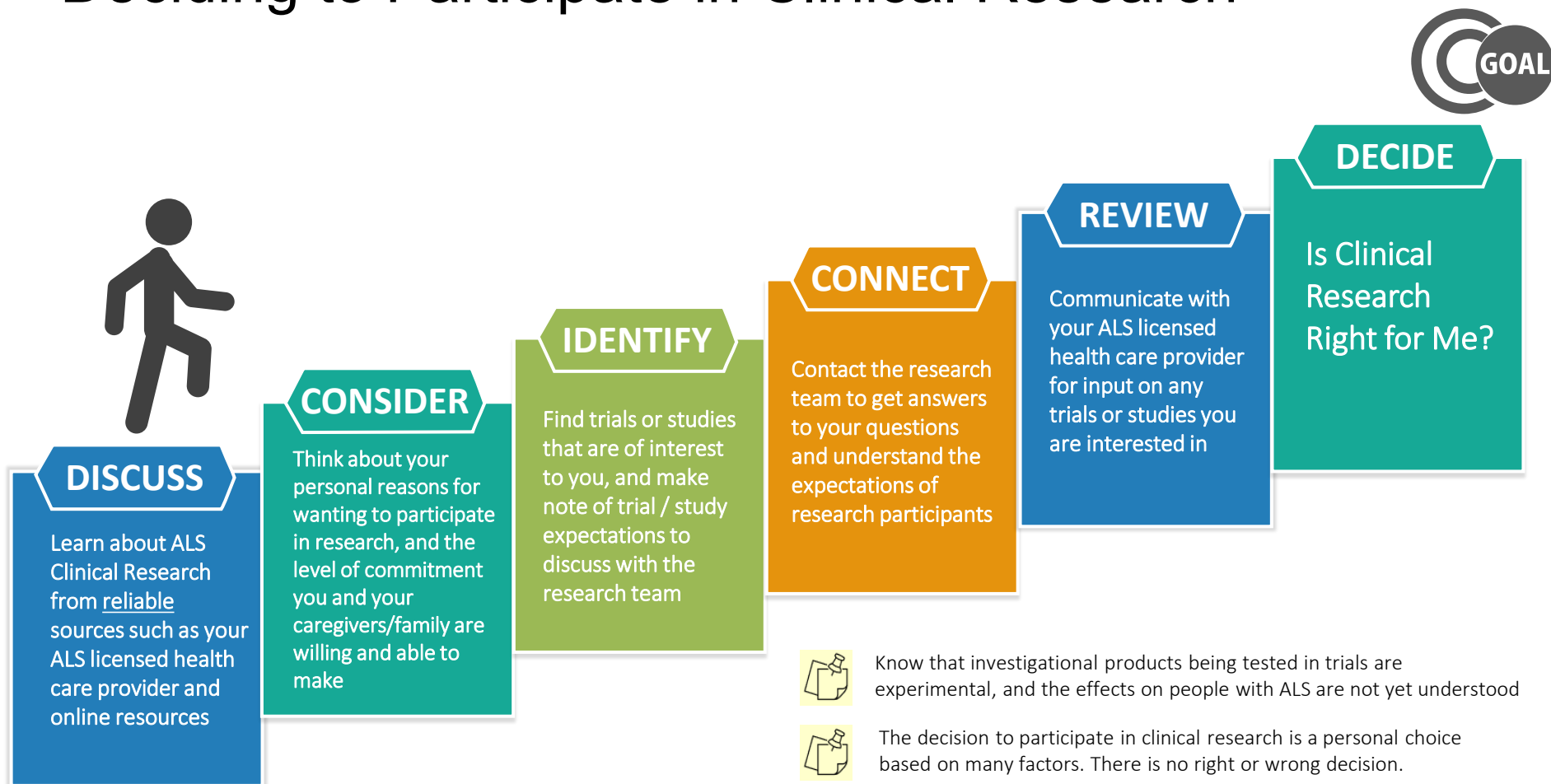
Outcomes



How do I decide if
Clinical Research is
right for me?

PARTICIPATING IN CLINICAL RESEARCH

Deciding to Participate in Clinical Research



What resources are available to learn more about current clinical research?

LEARN MORE ABOUT
CLINICAL RESEARCH

Community Resources



ALLISON BULAT
Community Engagement

Bridging communications about ALS research with families impacted by ALS, researchers conducting trials, and drug companies developing new ALS treatments

abulat@neals.org



CHRISTINA SMITH
Communications Coordinator

Assisting individuals searching for clinical research opportunities in their area, answering general questions about enrolling in clinical trials and connecting families with ALS specialists.

alstrials@neals.org



JUDITH CAREY, RN
Research Access Nurse

Communicating with families impacted by ALS about clinical trials enrolling at MGH and beyond, and answering general research questions from the patient community and medical professionals

mghalsresearch@mgh.harvard.edu
617-724-8995



CATHERINE SMALL
Patient Navigation

Connecting with potential research participants, medical professionals, and research centers across the country to share information about the HEALEY ALS Platform Trial

healeyalsplatform@mgh.harvard.edu
833-425-8257

Online Research Resources

Clinicaltrials.gov



<http://bit.ly/3PQHASA>

Neals.org



<https://bit.ly/3LF34P>

ALSuntangled.com



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



“The Patient Navigator will lead communication efforts to disseminate information and educate ALS patients regarding the Platform Trial. The Patient Navigator will act as an intermediary between patients, caregivers, families and study teams to address questions related to trial participation and enrollment. The Patient Navigator will be a member of various committees as a patient representative.”

Navigating Patient Navigation



Assist with patient education and communications.



Connect potential research participants with local sites.



Provide personalized site support on a case-by-case basis.



Organize weekly Platform Trial Q&A webinars.

Upcoming Webinars

Join us Thursdays at 5:00-5:30pm Eastern Time for weekly updates on trial progress and answers to audience questions.

- **Thursday, April 4th:** *Canceled*
- **Thursday, April 11th:** Weekly Q&A - [Click here to register](#)
- **Thursday, April 18th:** Weekly Q&A - [Click here to register](#)

Webinar Recordings

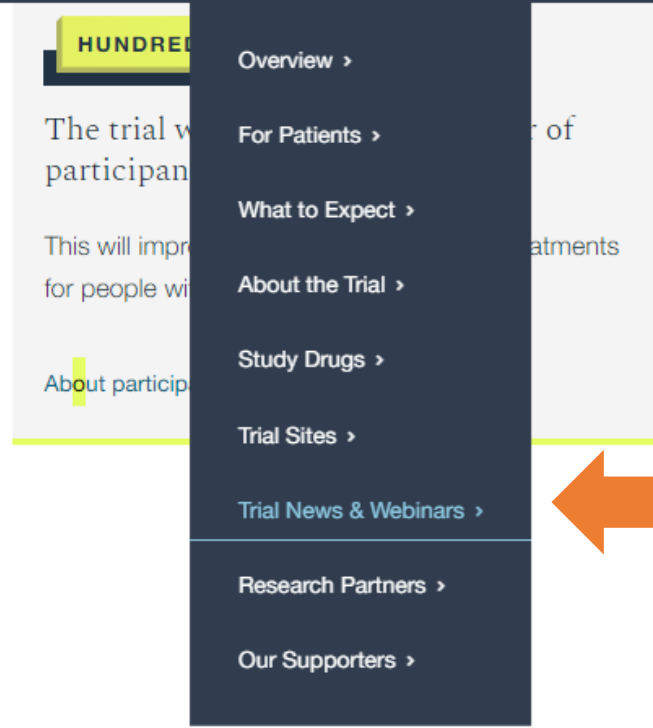
[Drug Science Webinar Series](#) +

[Weekly & Monthly Updates: 2024](#) +

[Weekly & Monthly Updates: 2023](#) +

[Weekly & Monthly Updates: 2022](#) +

**Platform Trial Q&A Webinars
Open to Everyone!**



Webpage Link:



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

Every Thursday!
5:00-5:30pm
Eastern Time

Digital Brochures – The Healey Platform Trial

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

NEALS
Northeast Amyotrophic Lateral Sclerosis Consortium

HEALEY ALS Platform Trial

A nationwide network of research centers in the Northeast ALS (NEALS) Consortium are partnering with the Sean M. Healey & AMG Center to conduct this first-of-its-kind platform trial for people living with ALS. The mission is to find answers as quickly as possible by testing multiple drugs using a shared platform.

Trial Highlights:

Multicenter Trial
More than 70 Platform Trial sites across the US are working together to enroll about 160-240 participants per regimen, depending on the regimen.

3:1 Active Drug to Placebo Ratio
Participants have a 75% chance of receiving active study drug, and a 25% chance of receiving placebo during the 24-week randomized controlled trial (RCT).

Active Treatment Extension (ATE)
Participants will continue into the ATE for their regimen upon completion of the RCT. During ATE, participants know that they are receiving the active study drug. ATE is also called Open Label Extension (OLE).

View map and contact info for participating research centers:
<https://bit.ly/3lICv2t>

For general questions about the HEALEY ALS Platform Trial, Contact the Patient Navigator:
healeyalsplatform@mgh.harvard.edu
833-425-8257 (HALT ALS)

Visit our website to learn more about current and future regimens:
<https://bit.ly/31EK198>

<https://bit.ly/3ExRai8> <https://bit.ly/3xCG6RXK> <https://bit.ly/3DvYkTa>

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

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/3ExRai8>

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 will continue into the ATE for ABBV-CLS-7262 after completing 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/30ctvnm>

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/31EK198> <https://bit.ly/3lICv9t> <https://bit.ly/3EHZaMT>

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

NEALS
Northeast Amyotrophic Lateral Sclerosis Consortium

HEALEY ALS Platform Trial

Regimen G

DNL343
Developed by Denali Therapeutics 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 G is testing an experimental medication called DNL343, 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/3ExRai8>

About Regimen G:

Regimen G is a Phase 2/3 trial enrolling approximately 240 participants to evaluate the safety and efficacy of DNL343 as a potential treatment for ALS. This regimen involves biomarker analysis and optional cerebrospinal fluid (CSF) collection to assess the effects of DNL343.

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 will continue into the ATE for DNL343 after completing 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/30ctvnm>

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/31EK198> <https://bit.ly/3lICv9t> <https://bit.ly/3EHZaMT>

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

Massachusetts General Hospital
Founding Member, Mass General Brigham

HEALEY ALS Platform Trial

Innovation through Biomarkers

Learn how your participation makes a difference

JULY 2023

What is a Biomarker?
A biomarker is an indicator of what is happening inside of your body. Biomarkers include everything from vital signs to laboratory tests of blood and other tissues. A biomarker may be used to: (1) learn more about a disease, (2) follow disease progression, (3) monitor how a disease responds to a study drug, or (4) serve as a target for treatment.

How do your biomarkers contribute to ALS research?
In the HEALEY ALS Platform Trial, biomarkers are studied in your blood, urine, and cerebrospinal fluid (CSF), or assessed via digital technology.

Neurofilaments (NF) are an example of a biomarker found in blood and CSF that has shown great potential in monitoring disease progression and drug effectiveness. NF levels are higher in the blood and CSF of people with ALS. This can be attributed to the breaking down of motor neurons, which spill contents such as neurofilaments into the spinal fluid.

By analyzing your biofluids, the research team is able to gather information on changes in important biomarkers, including NF levels. These analyses enable the team to have a better-informed view of how the study drug works, which may influence the course of drug development and result in a long-lasting impact on ALS research.

Biofluid Biomarkers
Digital Biomarkers

Analysis

Accelerate Drug Development

Progress Toward Better Treatments!

Blood Samples
Blood carries nutrients, drugs, and wastes throughout the body. Blood samples allow researchers to measure how the study drug is broken down and used in your body. In the HEALEY ALS Platform Trial, monitoring changes in various protein, hormone, and NF levels over time may help determine the effectiveness of an investigational drug.

Urine Samples
Urine samples enable researchers to collect valuable biomarkers as they are flushed out of your system. For example, p75 is a protein which increases in concentration with ALS progression. Tracking this protein may help determine drug efficacy and disease progression in certain trials.

Patients can visit HEALEYALSPlatform@mgh.harvard.edu or 833-425-8257 (HALT ALS)

<https://www.massgeneral.org/als> <https://www.massgeneral.org/als/als-research/plateform-trial>

Educational LP Resources

Short Video LP Demo:



<https://bit.ly/3OzI0wK>



Understanding HEALEY ALS Platform Trial Study Procedures

LUMBAR PUNCTURE

A Lumbar Puncture (LP), or Spinal Tap, is a procedure to remove a small sample (10-15ml or ~1 tablespoon) of cerebrospinal fluid (CSF) from the lower spine. CSF is the fluid that surrounds the brain and spinal cord, and it contains proteins, cells, and other substances that may be important biomarkers in ALS research. During the procedure, a needle is inserted between two lumbar vertebrae (backbones) in the lower back and into the space in the spinal canal that contains CSF.

Sometimes, people feel worried that a lumbar puncture could be risky or painful. In reality, this is a safe and common procedure to collect CSF!

Tips to Prep: Get a good night's rest, eat as usual, and stay well-hydrated prior to the LP visit.

LUMBAR PUNCTURE STEP BY STEP

- 1.) You will be asked to sit or lie down in a position that helps widen the spaces between the bones of the lower spine.
- 2.) The doctor will cleanse the skin on your lower back to reduce risk of infection, then use a small needle to inject a local anesthetic (such as lidocaine) to numb the site.
- 3.) The LP needle is inserted into the space containing CSF. A special atraumatic spinal needle (Sprotte) is typically used to reduce the chance of a post-puncture headache. The doctor may need to readjust the needle if CSF cannot be drawn with the first insertion.
- 4.) Spinal fluid is collected into specimen tubes for lab testing. The LP needle is removed, your back is cleaned, and a band-aid is placed over the LP site.
- 5.) For your comfort and safety, it is recommended that someone drive you to and from the LP study visit.

QUESTIONS? Prior to enrolling in a clinical trial, your study team will discuss the LP procedure with you. Please ask your study team for clarification if you have any questions while reviewing the informed consent form.

eat

your visit and head remember a few things and safety.

Drink plenty of fluid in the 12 hours prior to help your body move during the procedure. This is the likelihood of a headache.

strenuous physical activity the day of the LP, you may feel tired and shower as soon as you can. Your team can answer questions about timing, location, and you are encouraged to bring someone to drive you to your next day.



a mild headache, you may feel (in addition to the above instructions)

- If your headache becomes more than mild or persists longer than 24 hours, and is not relieved by the above interventions OR if you develop a fever at any time following the LP, please contact your study team right away.
- Back Discomfort:**
- If you experience back discomfort, try applying ice wrapped in a towel to the affected area for 20 minutes, 3-4 times over the course of the day.

Short Video LP Demo:



Understanding HEALEY ALS Platform Trial Study Procedures

LUMBAR PUNCTURE

Dr. Latha's Q&A

From an accredited US hospital

Learn how experts define health sources in a journal of the National Academy of Medicine

Webinar Recordings:
 March 30th with James Berry
 June 22nd with Shafeeq Ladha
 Aug 10th feat. participant story

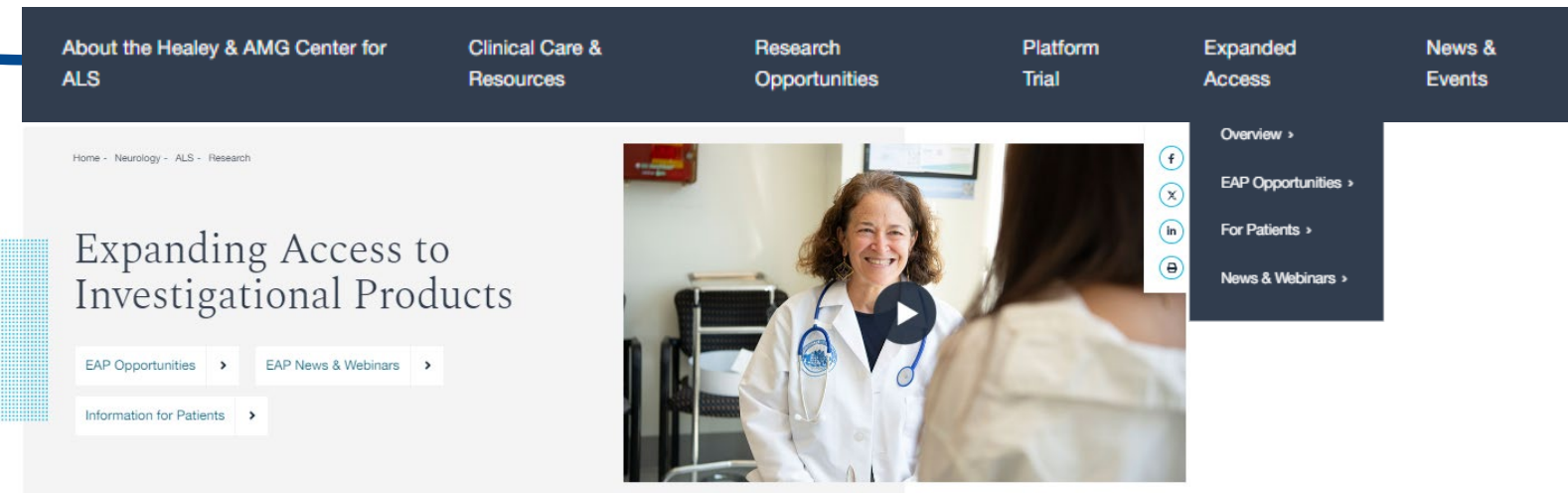


<https://bit.ly/3UMDqOD>

HEALEY ALS Platform Trial Weekly Q&A Webinar: August 10, 2023 | Massachusetts General Hospital



EAP Webpages and Resources



The best way to learn about expanded access opportunities is to have a discussion with your ALS doctor and clinical care team. Below are some common questions and resources that can help guide these discussions.

Upcoming Opportunities: Enrollment Anticipated Spring 2024

- Pridopidine EAP2, by Prilenia Therapeutics** +
- RAPA-501 EAP, by Rapa Therapeutics** +

Other Multicenter Expanded Access Protocols

- CNM-Au8 EAP2, by Clene Nanomedicine** +

No Longer Enrolling

- Trehalose (SLS-005) EAP, by Seelos Therapeutics** +

EAP Opportunities
Current and upcoming expanded access protocols
[Learn more >](#)

EAP News & Webinars
Get the latest news and updates about expanded access protocols
[Learn more >](#)

Information for Patients
Common questions and helpful resources about expanded access
[Learn more >](#)

Link to EAP Website:



<https://bit.ly/3uni3lc>

What online resources are available to learn more about a specific trial or study?

WHERE TO FIND CLINICAL RESEARCH ONLINE

ClinicalTrials.gov is a place to learn about clinical studies from around the world.



The U.S. government does not review or approve the safety and science of all studies listed on this website.



Read our full [disclaimer](#) for details.

Focus Your Search (all filters optional)

Condition/disease ⓘ

ALS \ (Amyotrophic Lateral Sclerosis\)

Other terms ⓘ

Intervention/treatment ⓘ

Location

Search by address, city, state, or country and select from the dropdown list

Search



NEALS HOMEPAGE

Search for a Trial

Please select any of the boxes below. All fields are optional; making a selection in each search field is **not** required for a search and may limit your search results. To apply your filters click on the Search button. To start over, click on the Reset button.

Keyword Search:

Trial Status

- Not yet recruiting
- Recruiting
- Recruiting healthy volunteers
- Not recruiting

Type Of Study

- Interventional
- Observational

Phase

- Phase 1
- Phase 2
- Phase 3
- Phase 4

Placebo

- active agents/drugs only
- active agents/drugs & a placebo

Genetic Target

- FUS
- C9orf72
- SOD1
- TDP-43
- Ataxin-2
- Other Genes

Country

State

NEALS Affiliated

Yes

Show entries

Showing 1 to 25 of 903 entries

Trial Name:

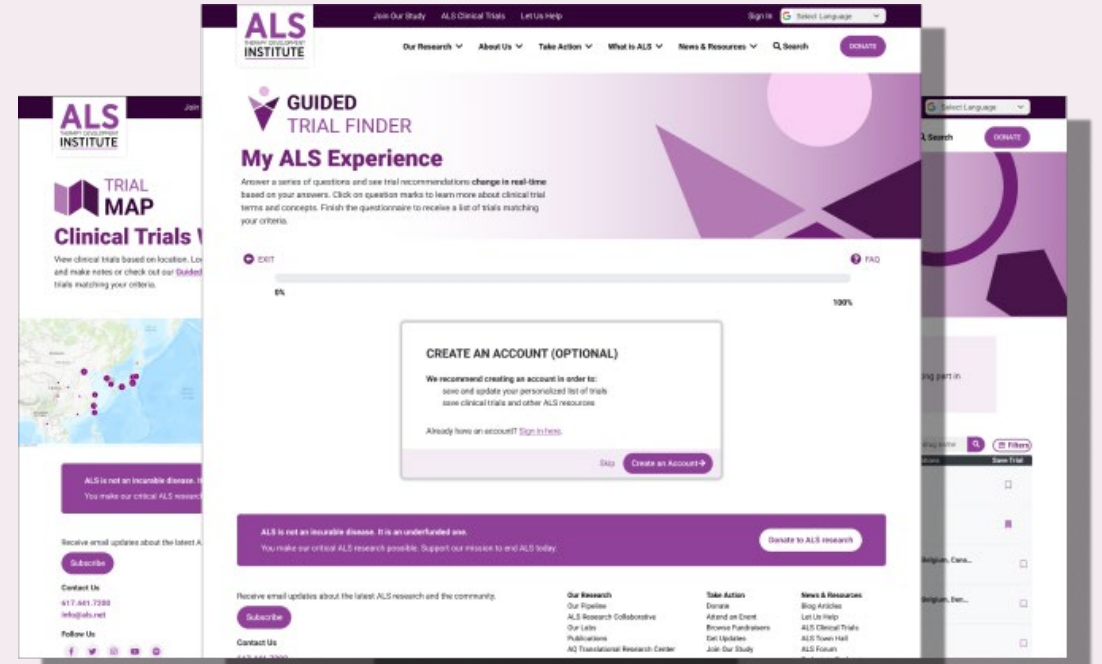
Study Status: **Study Type:**

'sPATIALS3' - National Multicenter Randomized Controlled Interventional Study, Comparing an Active Symbiotic and a Passive Symbiotic Aimed at Evaluating the Effect on the Intestinal Microbiota and on the State of Health and Well-being of Various Types of Chronically Frail Patients United by Alterations of Intestinal Function	Not recruiting	Interventional
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ALS TRIAL NAVIGATOR

A new innovative set of tools that match you to trials and help you to make informed decisions!

- **Easily Explore Global Opportunities:** Access in-depth information on research opportunities around the world.
- **Get Tailored Recommendations:** Answer questions to unlock a personalized list of trials tailored to your criteria.
- **Access a Personalized Portal:** Create an account to save, annotate, and bookmark trials within your personalized portal.
- **Empower Your Decision-Making:** Learn important terms and concepts to make informed decisions.



Scan the QR Code to check out the ALS Trial Navigator



ALS Signal: Canada & US Trials

Clinical Research

Canada & US Trials

Genetic ALS
Observational Studies

[Click Here for More Detailed Definitions](#)

Expand Trial Finder Table:

Country	State/Province	Drug/Treatment	Remote Trial	Trial Phase	Genetic Target	Recruitment Status
Search <input type="checkbox"/> Select All <input type="checkbox"/> Canada <input type="checkbox"/> US	Search <input type="checkbox"/> Select All <input type="checkbox"/> Alabama <input type="checkbox"/> Arizona <input type="checkbox"/> California <input type="checkbox"/> Colorado	Search <input type="checkbox"/> Select All <input type="checkbox"/> 4 Treatments <input type="checkbox"/> AMT-162 <input type="checkbox"/> ANX005 <input type="checkbox"/> Baricitinib <input type="checkbox"/> BUN067 (Tofersen)	Search <input type="checkbox"/> Select All <input type="checkbox"/> No	Search <input type="checkbox"/> Select All <input type="checkbox"/> 1 <input type="checkbox"/> 1/2 <input type="checkbox"/> 2 <input type="checkbox"/> 2/3 <input type="checkbox"/> 3	Search <input type="checkbox"/> Select All <input type="checkbox"/> FUS <input type="checkbox"/> Healthy Volunteers <input type="checkbox"/> SOD1 <input type="checkbox"/> Sporadic or Famil... <input type="checkbox"/> Sporadic/Mutated	Search <input type="checkbox"/> Select All <input type="checkbox"/> COMPLETED <input type="checkbox"/> Enrolling by invita... <input type="checkbox"/> Not Yet Recruiting <input type="checkbox"/> Not yet recruiting

Expanded Access Studies				Studies by Stage		Studies by Location	
Drug / Treatment	Sponsor	Drug Company	Learn More	Pie Chart		Map	
CNM-Au8	Clene Nanomedecine	Clene Nanomedecine	↗	1		3	
CNM-Au8	Clene Nanomedecine	Clene Nanomedecine	↗	2/3		1/2	
CNM-Au8	Clene Nanomedecine	Clene Nanomedecine	↗	N/A		2	
Pridopidine	Prilenia	Prilenia	↗				
RAPA-501	Rapa Therapeutics LLC	Rapa Therapeutics LLC	↗				

Drug/Treatment	Trial Phase	State/Province	Eligibility Timeframe	Recruitment Status	Target Enrollment	Location	Contact	Phone	Email	Trial Link
Masitinib	3	Alabama	< 24 months from diagnosis	Recruiting	495	University of Alabama at Birmingham	Clinical Study Coordinator	==+33(0)147200014	✉	↗
Masitinib	3	California	< 24 months from diagnosis	Recruiting	495	University of Southern California	Clinical Study Coordinator	==+33(0)147200014	✉	↗
Masitinib	3	Kentucky	< 24 months from diagnosis	Recruiting	495	University of Kentucky	Clinical Study Coordinator	==+33(0)147200014	✉	↗
Masitinib	3	Maryland	< 24 months from diagnosis	Recruiting	495	Johns Hopkins Medicine Brain Science Institute	Clinical Study Coordinator	==+33(0)147200014	✉	↗

<https://www.iamals.org/get-help/als-signal-clinical-research-dashboard/>

**NORTHEAST ALS
CONSORTIUM
(NEALS)**





ABOUT US

Governed by a seasoned Leadership Team, NEALS has developed into an active network of 155+ trial-ready sites with extensive training on conducting clinical research and robust experience in designing and managing FDA-regulated clinical trials for ALS.

NEALS has successfully partnered with industry and academic researchers to conduct high-quality ALS studies for over 25 years and has grown to be the largest ALS research consortium in the world.



NEALS Members

Our 155+ member sites are academic research centers equipped to perform clinical trials and research in ALS and MND.



NEALS Scientific Advisory Board (SAB)

The SAB provides a forum for investigators and industry to vet new ideas for drugs, technologies, & trials.



NEALS Coordinating Centers

Our strong infrastructure facilitates rapid institution and support of trials sponsored by industry, foundations, and federal granting agencies.



NEALS Biorepository

NEALS has extensive shared clinical data and biofluid samples available to researchers to further the understanding of ALS and for developing disease biomarkers

NEALS **MISSION**

Our mission is to accelerate the development of new treatments through innovative research and working collaboratively with people living with ALS and all stakeholders in the ALS research community.



OUR VALUES

At NEALS, our values underscore our commitment to the integrity of ALS clinical research and to the ALS community.

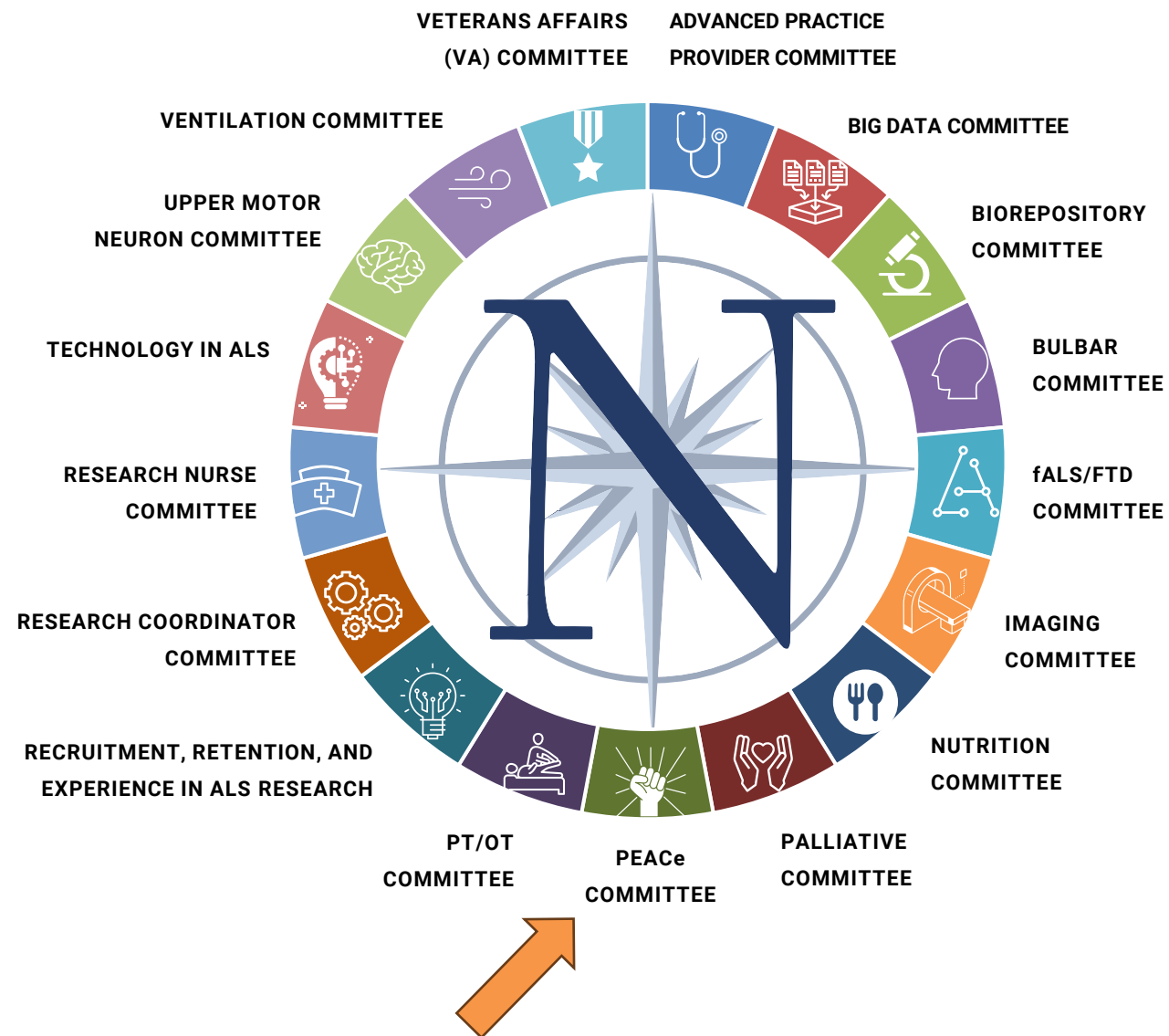
- **Transparency:** We believe in open scientific communication and strive to share information openly with our partners and stakeholders.
- **Integrating the Lived ALS Experience:** We are dedicated to building strong partnerships with people living with ALS, to ensure research initiatives align with community priorities.
- **Excellence:** Our approach to scientific research relies on rigorous peer review and ongoing quality improvement.
- **Democratic Governance:** We value inclusive decision-making and diverse perspectives to ensure that our organization and activities reflect the input and needs of the community.



NEALS COMMITTEES

NEALS has established numerous committees focused on advancing ALS research and patient care.

These ad hoc committees are established by the Executive Committee, as needed, or by membership proposal. Subcommittees are chaired by NEALS members and meet throughout the calendar year.



NEALS Information & Community Education Leadership Working Group



Allison Bulat
NEALS/Mass General



Christina Fournier, MD, MSc
Emory University



Jinsy Andrews, MD, MSc
NEALS Co-Chair
Columbia University



James Berry, MD, MPH
NEALS Co-Chair
Mass General



Senda Ajroud-Driss, MD
Northwestern



Amy Chen, MD, PhD
University of South Florida



Lauren Elman, MD
Penn Medicine



Dominic Ferrey, MD
UC San Diego Health



Stephen Goutman, MD
University of Michigan



Kelly Gwathmey, MD
VCU Health



Stephen Johnson, MD
Mayo Clinic, Scottsdale



Justin Kwan, MD
NIH



John Novak, MD
Ohio Health



Bjorn Oskarsson, MD
Mayo Clinic, Jacksonville

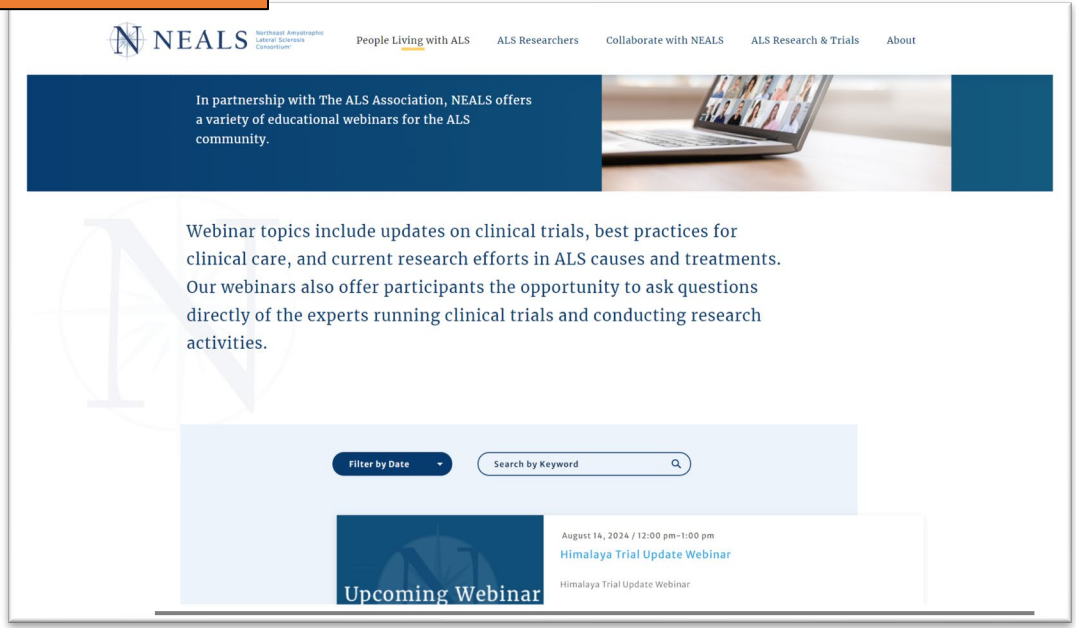


Xiaowei W. Su, MD, PhD
Penn State Health

Community Education

www.neals.org

For PLWALS



NEALS Northeast Amyotrophic Lateral Sclerosis Consortium

People Living with ALS ALS Researchers Collaborate with NEALS ALS Research & Trials About

In partnership with The ALS Association, NEALS offers a variety of educational webinars for the ALS community.

Webinar topics include updates on clinical trials, best practices for clinical care, and current research efforts in ALS causes and treatments. Our webinars also offer participants the opportunity to ask questions directly of the experts running clinical trials and conducting research activities.

Filter by Date Search by Keyword

Upcoming Webinar
August 14, 2024 / 12:00 pm-1:00 pm
Himalaya Trial Update Webinar
Himalaya Trial Update Webinar



NEALS Northeast Amyotrophic Lateral Sclerosis Consortium

People Living with ALS ALS Researchers Collaborate with NEALS ALS Research & Trials About

UPDATE
ALS CLINICAL RESEARCH LEARNING INSTITUTE® CALL FOR APPLICATIONS NOW OPEN!

We are pleased to announce that applications are now being accepted for the upcoming in-person ALS Clinical Research Learning Institute® scheduled for October 20th & 21st, 2024, in Clearwater, Florida. Call for applications closes July 15th, 2024.

Apply Here

Expanded Access



NEALS Northeast Amyotrophic Lateral Sclerosis Consortium

People Living with ALS ALS Researchers Collaborate with NEALS ALS Research & Trials About

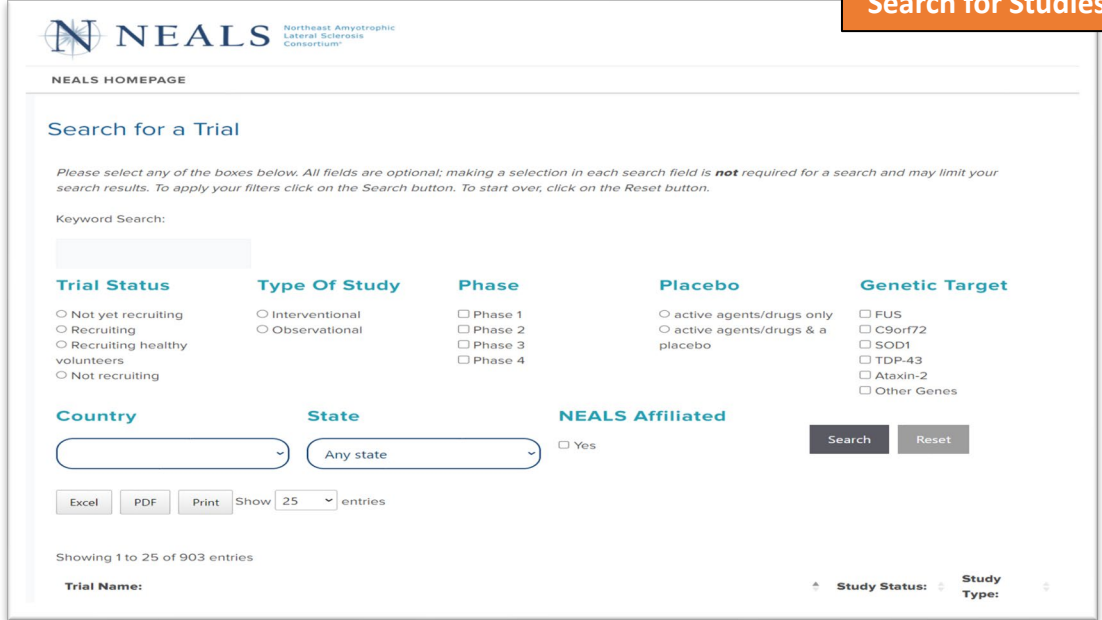
Expanded Access

Our mission is to translate scientific advances into new treatments for people with Amyotrophic Lateral Sclerosis (ALS) and Motor Neuron Disease (MND) as rapidly as possible. In addition to conducting clinical trials, NEALS is committed to sharing information about Expanded Access to investigational therapies.

WHAT IS EXPANDED ACCESS
Providing Access to Individuals Who Do Not Qualify for Trials

Expanded Access (EA) is a pathway for people living with a

Search for Studies



NEALS Northeast Amyotrophic Lateral Sclerosis Consortium

NEALS HOMEPAGE

Search for a Trial

Please select any of the boxes below. All fields are optional; making a selection in each search field is **not** required for a search and may limit your search results. To apply your filters click on the Search button. To start over, click on the Reset button.

Keyword Search:

Trial Status Type Of Study Phase Placebo Genetic Target

Country State NEALS Affiliated

Search Reset

Excel PDF Print Show 25 entries

Showing 1 to 25 of 903 entries

Trial Name: Study Status: Study Type:

THE INTERNATIONAL ALLIANCE OF ALS / MND ASSOCIATIONS



PALS and CALS Advisory Council

The PALS & CALS Advisory Council (PCAC) is charged with helping the Board of Directors of the International Alliance of ALS/MND Associations consider and include in its work the wide-ranging opinions and ideas of people with ALS/MND and caregivers to people with ALS/MND from across the globe.

Patient Fellows Program

Empowering people living with ALS/MND and their caregivers to actively participate in the scientific community's dialogue. By bridging the gap between patients and researchers, we aim to accelerate progress towards effective treatments and ultimately a cure for ALS/MND.

What is ALS/MND	Find a Member Association	Support for PALS & CALS	Support for Health Professionals	Events/Programs	About	Members
FUNDAMENTAL RIGHTS OF PEOPLE WITH ALS/MND AND CAREGIVERS	RESEARCH	ADVOCACY	CLINICAL CARE	DRUGS IN DEVELOPMENT	APPROVED DRUGS	
	Voice Preservation	Advocacy Toolkit	Genetic Counselling & Testing	AB Science – Masitinib	Radicava/Edaravone	
	Open Science	Emergency Preparedness	Mental Health Support	Amylyx – AMX0035	Neudexta	
	Expanded Access	Toolkit	Nursing and Symptom Management	Biogen – Tofersen	Riluzole/Tiglutik	
	Understanding ALS/MND Research	Equitable Access to Therapies	Nutrition and Swallowing	BrainStorm Cell Therapeutics – NurOwn		
	Improving Regulatory Pathways		Occupational Therapy and Activities of Daily Living	Clene Nanomedicine – CNM-Au8		
	Right to Try		Physiotherapy and Mobility	Collaborative Medicinal Development – CuATSM		
	US FDA Orphan Drug Designation		Respiratory Care	Cytokinetics – Reldesemtiv		
	Unproven (Off-Label) Treatments		Speech Therapy and Communication	ILB – Tikomed		
	Open Label Extension		Support for Family & Caregivers	Kadimastem – AstroRx		
			Technology	Mitsubishi Tanabe Pharma America – Oral Edaravone		
			Global Clinic Locator	NeuroSense PrimceC		
				T Regulatory Cell Therapies		

Alliance Meeting



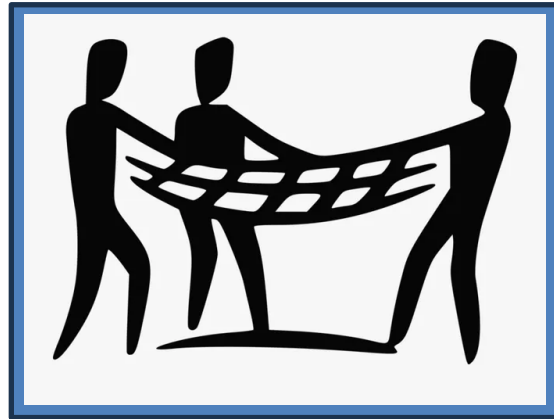
Allied Professionals Forum



MONTREAL, CANADA

Friday 6 December – Sunday 8 December 2024

ALS OFFICE HOURS



A Community-Led Program

ALS Office Hours

[HOME](#) [ABOUT](#) [REQUEST A SESSION](#) [CONTACT US](#) [CALENDAR](#) [RESOURCES](#)



WELCOME

We are here to help.

Get answers to your questions about care, research and community resources for ALS

Join ALS Office Hours on Zoom

Please join us each Friday at 10:00 am PT / 1:00 pm ET
Zoom Meeting

<https://zoom.us/j/95529244242?pwd=ODJzV0hvZG1QYm5ndEMvazFFd2hDQT09>

Resources

Please reach us at Contact@officehoursals.com if you cannot find the answer you need.

- ALS Organizations
- Adaptive Clothing
- Adaptive Feeding and Hygiene
- Adaptive Voice / Hands-Free Devices / Eye Gaze / Webinars
- Advocacy / Community Involvement
- Breathing Devices
- Exercises / Physical Activity / Outings
- Feeding Tubes
- Financial Resources
- Genetic Counseling, Testing and Resources

www.officehoursals.com

Who We Are

We are a group of caring people with various lived experiences with ALS, and professional expertise in the field of ALS. We understand the challenges associated with ALS, and simply want to be available to share resources and learnings that may help others. We do this as individuals, not representatives of any organization we may be associated with.

What We Do

- We make ourselves available via email, phone or Zoom.
- We are open and honest, and willing to discuss anything requested.
- We answer general questions about ALS pre-clinical and clinical research.
- We share resources from around the world that empower ALS families.
- We answer scientific questions as needed (scientists only)

What We Do Not Do

- Provide personal opinions about any options available.
- Represent any one organization or group - we are a group of volunteers from various backgrounds and affiliations, with information and compassion.
- Give any medical advice.
- Give any financial advice.
- Discuss any information that we can not confirm via legitimate scientific sources or personal experience.

AFTER ALS



A Community-Led Program

Vision Statement:

We are dedicated to offering a safe space for sharing experiences, expressing emotions, and lending support; we understand the unique challenges of ALS/MND/FTD-related grief. We want people to know they are not alone. Through empathy, education, and mutual support, we foster healing, resilience, and hope.

Facebook:

<https://www.facebook.com/profile.php?id=61562407402718>

Website:

Under construction

Mission Statement:

We provide a compassionate and supportive community for all who have lost someone to Amyotrophic Lateral Sclerosis (ALS), Motor Neuron Disease (MND), and/or Frontotemporal Degeneration (FTD). Our community is facilitated by individuals who have lost someone to ALS, MND, and/or FTD.

Meetings:

Weekly 3:00-4:00 EST every Wednesday for people wanting to volunteer as part of the group.

Monthly the second Wednesday of each month from 7:00-8:00 EST for support.

VETERANS ACTION GROUP (ALS Hope Foundation)



For more information, contact:
Mandibailey1@icloud.com

Objective: To bridge the gap between Veterans living with ALS and the ALS scientific/research community, by raising awareness about current ALS research , providing the Veteran community with accurate and reliable information about clinical trials and research programs, and creating a space for the Veteran stakeholder voice in the ALS drug development process.

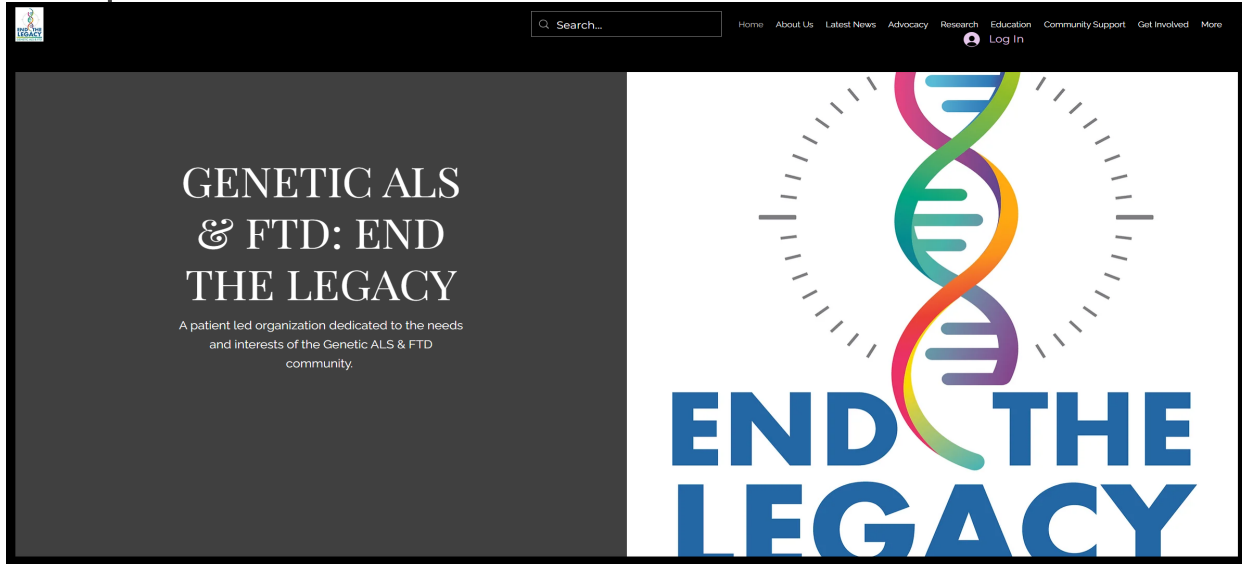


END THE LEGACY (ALS Hope Foundation)



www.endthelegacy.org

<https://www.endthelegacy.org/recruiting-studies>



Recruiting Studies with a Genetic ALS or FTD Focus

We strive to keep these up to date but know there will be a lag when changes are made to recruiting status. Please double check before assuming a study is still open or not open. If you have feedback contact us at info@endthelegacy.org

Recruiting Observational Studies in the United States

Genetic ALS & FTD: End the Legacy was born through the efforts and vision of dozens of people impacted by genetic ALS and FTD who collectively saw the need for our community's voice to be heard .

Since our forming as a group in 2020 and especially since our debut as a non-profit in 2023, we have been proud to witness how our activities have changed the conversation around inherited ALS and FTD. We are possible due to the commitment and passion of many volunteers, and we are always recruiting!

ALS CLINICAL TRIALS FACEBOOK PAGE



United States, 2014. MMWR Morb Mortal Wkly Rep 2018;67:216–218.

Lifetime risk:

Probability of developing ALS over a person's lifetime

1:350 for men

1:450 for women

Armon, C. Sports and trauma in amyotrophic lateral sclerosis revisited.
Journal of Neurological Sciences. 2007;269:188-190

ALS CTR

ALS Clinical Trials and Research

Public group · 6.7K members



+ Invite

Share

Joined ▾

Discussion

Featured

Events

Media

Files

People



<https://www.facebook.com/groups/2688029951255361>

ALS UNTANGLED



<https://www.alsuntangled.com/>



[HOW TO USE](#)

[MISSION & METHODS](#)

[COMPLETED REVIEWS](#)

[FUTURE REVIEWS](#)

[SEARCH](#)

[ENGLISH](#)

ALSUntangled® reviews alternative and off label treatments, with the goal of helping people with ALS make more informed decisions about them.



**About Our
Method**



**Completed
Reviews**



**Future
Reviews**

Mission & Methods

ALSUntangled® systematically reviews alternative and off label treatments (AOTs) to try and help patients with ALS make more informed decisions about them. The basic structure of all our reviews revolves around a “Table Of Evidence”. In this, each AOT is graded across 5 different categories: mechanistic plausibility, preclinical models, cases, trials and risks. Grades in each category range from A (best) to F (worst), with U meaning we could not find any useful disclosable evidence for the AOT in that category. Final grades are crowd-sourced across an international team of more than 100 clinicians and scientists from across 11 different countries.

ALSUntangled: Introducing The Table of Evidence

The ALSUntangled Group

<https://doi.org/10.3109/21678421.2014.987476>

PUBLISHED ONLINE:
27 December 2014

Table 1 of 2

Table I. ALSUntangled TOE.

Evidence category	Grade					
	U	F	D	C	B	A
Mechanistic plausibility	Unknown	Implausible; would violate known principles or laws of biology	Acts on a biological mechanism but it is not clear than this mechanism is relevant in ALS	Theoretically and plausibly acts on an ALS-relevant mechanism in humans	Shown in a peer-reviewed publication to act on a relevant mechanism in pre-clinical model(s)	Shown in a peer-reviewed publication to act on a relevant mechanism in humans
Pre-clinical models (animal or cell models recognized by ALSUntangled reviewers to be relevant to ALS)	None	The only studies available show no benefit	One or more non-peer reviewed studies reporting benefits (published on a website or in an abstract)	One or more peer-reviewed publication(s) reporting benefits in flawed studies (*)	One peer-reviewed publication reporting benefits in a well-designed study (*)	Two or more peer-reviewed publications reporting benefits in well-designed studies (*)
Patient case reports	None	The only reports available show no benefit	Subjective report(s) of benefit without validated diagnoses and/or benefits	One unpublished report of benefit with validated diagnosis and benefits	More than one unpublished report of benefit with validated diagnosis and benefits	One or more peer-reviewed publications reporting benefits with validated diagnosis and benefits
Patient trials	None	The only trials available show no benefit	One or more peer-reviewed publications reporting benefits in a flawed trial (**)	One or more peer-reviewed publications reporting benefits in a well-designed randomized, blinded, placebo-controlled phase I or II trial	One peer-reviewed publication reporting benefits in a well-designed randomized, blinded, placebo-controlled phase III trial	Two or more peer-reviewed publications describing benefits in well-designed randomized, blinded placebo-controlled phase III trials
Risks (harms that occurred on this treatment)	Unknown	At least 5% of exposed patients experienced death or hospitalization	More than 0% but less than 5% of exposed patients experienced death or hospitalizations	At least 10% of exposed patients experienced harms (no hospitalizations or deaths)	More than 0% but less than 10% of exposed patients experienced harms (no hospitalizations or deaths)	No exposed patients appear to have experienced harms



*Animal studies are assumed to be 'well designed' when they follow published guidelines (8). When they deviate from these they are considered 'flawed'.

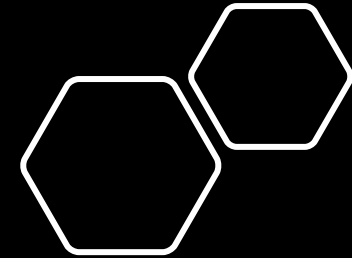
**Flawed trials means those in which there are identifiable problems with patient selection, randomization, blinding, controls or follow-up. These have 'high or unclear risk of bias' according to published criteria (9). Well-designed trials are those that have 'low risk of bias'.

Completed Reviews

Here you will find our published reports on AOTs, along with the grades we gave each of them in different categories, short summaries and even podcasts. Click on the name of the review to open the published report, or click on the podcast link to listen to a short interview about it. When new information about an AOT comes out after our published review, we update the summary and the assigned grades accordingly. Summaries and grades that have been updated since a published review are annotated with an asterisk.

Click on any completed review title or letter grade below for detailed information. Click on any column header ("Treatment", "Mechanism", etc.) to sort the entire table by that column. In addition, you can [click here to view the complete table of evidence](#).

Treatment ▾	Podcast	Mechanism ▾	Pre-Clinical ▾	Cases ▾	Trials ▾	Risks ▾
PoNS Device (2024)		U	U	U	U	C
Ashwagandha (2024)		B	A	C	U	B
Lions Mane (2024)		B	U	F	U	B
Insulin (2023)		C	B	U	U	F
Nuedexta (2023)		B	U	A	C	C
Caffeine (2023)		A	C	F	U	B
Astaxanthin (2023)		A	U	C	U	B
Ozone (2022)		A	D	C	U	D



Future Reviews

Here you will find AOTs that patients and families around the world have asked us to review in the future. We are working hard to get these done as quickly as we can but it does take a lot of time to complete a thorough review. We invite you to cast up to one (1) vote for each item below. More information coming soon.

Vote	Treatment ▾	Multiplier ▾	Votes ▾	Final Priority ▾
Vote	Nadir's ALS Remission Protocol	1	1747	1747
Vote	Dr. David Steenblock	1	1343	1343
Vote	Stem Cells at "Hanyang University in South Korea"	1	710	710
Vote	Placebo Therapy	2	292	584
Vote	Ibudlast and nootropics	2	225	450
Vote	Charlotte's Web Hemp Oil	1	376	376
Vote	N-acetylcysteine	2	187	374

Takeaways....



Everyone CAN be involved in research, or support research in some capacity if they choose



The Science and Patient Communities are working in partnership to create the most patient-centric approach to research possible



Research is where hope and the pathway forward live

THANK YOU!

Judi Carey: MGHALSResearch@mgh.harvard.edu

Allison Bulat: Abulat@neals.org

Clinical Research is a journey of curiosity, where every question leads to an answer, and every answer uncovers new possibilities. Keep exploring!

~ Author Unknown

Coming in August

Thursday August 29, at 12 PM CST

**Empower Your Voice: Navigating Conversations with your ALS
Care Team**



Ambereen Mehta, MD, MPH, FAAHPM
Johns Hopkins Center for Specialty ALS Care



Suzana K. E. Makowski, MD
Compassionate Care ALS