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ALS & PARTICIPATION IN CLINICAL RESEARCH

A Les Turner ALS Foundation Guide for People Living with ALS

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Contents

Disclaimer Statement: The information in this guide is not medical advice. Talk to your ALS care team before making any decisions about your health or treatment. Together, you and your care team can find a treatment plan that works for you.

Last Reviewed: April 22, 2024

lesturnerals.org & neals.org

People living with ALS, along with family members and caregivers, are essential partners in ALS research. You can help scientists and doctors learn about ALS and develop new treatments by participating in a clinical research study.

What is clinical research?

If you're living with ALS, you can volunteer to participate in a type of scientific study called a clinical research study. Through clinical research, scientists and doctors can learn about ALS from people who've experienced it firsthand, gain a deeper understanding of genetic differences linked to ALS, and test potential treatment options.

There are many ways that people living with ALS can get involved in clinical research:

Clinical trials

A clinical trial, also known as an interventional trial, is a medical experiment that people living with ALS can volunteer to participate in. These experiments help researchers identify information about investigational products: study drugs, special diets, or medical devices. All investigational products being tested in clinical trials are considered experimental. That means it is not known if, or how, these products could potentially impact your disease or symptoms.

Observational studies

During an observational study researchers follow a group of people living with ALS to learn more about their condition. 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) is a pathway for people who do not qualify for a clinical trial to access an investigational product that is not yet approved by the Food and Drug Administration (FDA).

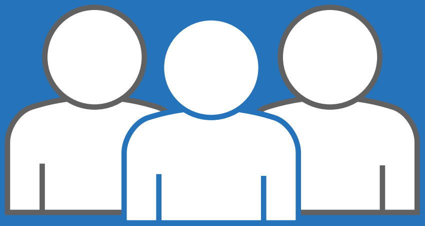
End-of-life tissue donations

Anyone living with ALS can choose to make an end-of-life tissue donation to ALS research. A donation of tissue (most commonly brain and spinal cord) is a significant and impactful gift to ALS research.

What are the benefits of participating in clinical research?

If you join a clinical trial, you may try a new treatment option that's not available to the public yet. During a clinical trial, you will be monitored frequently.

By participating in any type of clinical research, you can help researchers make scientific discoveries that will help people living with ALS in the future. For many people, participating in research can be an empowering experience.



ALS research needs diverse perspectives.

It's important for people with many different backgrounds and experiences to get involved in ALS research. When people of all ages, genders, races, and ethnicities participate in research, scientists can learn more about how ALS affects everyone — and develop treatments that work for as many people as possible.

What are the phases of a clinical trial?

Clinical trials are performed in four steps or phases. Here's what happens in each phase:

Phase 1: Researchers test the investigational product on a small group of people. The purpose of the first phase is to determine safety, tolerability, and dosing: if the investigational product is safe, if people can tolerate it, and how the drug affects people at different doses.

Phase 2: The purpose of the second phase is to continue to monitor the safety, tolerability, and dosing and start to learn about the potential effects of the investigational product.

Phase 3: The purpose of the third phase is to continue to monitor the safety, tolerability, and dosing and confirm the potential benefit of the investigational product. If researchers confirm the benefit of the investigational product, it may be approved as a treatment for ALS.

Phase 4: The fourth phase occurs after the approval of a new treatment for ALS. The purpose of this phase is to monitor safety, rare side effects, and effectiveness of the treatment over a longer period of time.

How do I decide if I want to participate in a clinical research study?

Participating in a clinical research study is an important and personal decision. You can follow these steps to learn about clinical research studies, explore your options, and decide if participating in a study is right for you.

Step 1

Learn about current ALS clinical research

The first step is to learn about ALS clinical research from reliable sources. Talking to your doctor or ALS care team is a great place to start. They can walk you through the basics of clinical research and give you input based on your personal situation. You can also explore these online resources:

ClinicalTrials.gov is a central database of clinical trials for all health conditions, including ALS. All studies that are looking for participants in the United States (and most studies worldwide) are posted on **ClinicalTrials.gov**.

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Clinical trials are experimental.

While the effects of the investigational product being tested in ALS is unknown, clinical trials are still the best way to develop new treatments for people living with ALS.

> “As a caregiver of a loved one, I agreed that if there was a trial available, it was best to participate. Either way it’s a win, the best outcome is it proves to help, the worst outcome is it doesn’t, but we can move forward with new knowledge.

> “With a disease where so much is out of your control, deciding to participate in a trial was empowering for me. We have the drugs we have now because others were involved in trials. Positive things can come from drug research.

NEALS (NEALS.org) has a database of clinical research studies related to ALS. If you have questions about the database or you'd like to learn more about a study, contact the Clinical Trial Liaison at **alstrials@neals.org** or (855) 437-4823.

The ALS Trial Navigator (ALS.net/ALS-Trial-Navigator) is a comprehensive resource that guides people living with ALS as they learn more about finding and participating in clinical trials for ALS. After answering a few questions, you can learn about research opportunities, set personal priorities, and find trials that meet your criteria. The Navigator also has an interactive map to help you find trials in your area.

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If you have questions about ALS clinical research, you can reach out to the following organizations:

- Your local study coordinator
- NEALS: **alstrials@neals.org**
- Massachusetts General Hospital (MGH) ALS Research: **mghalsresearch@mgh.harvard.edu**
- MGH Sean M. Healey and AMG Center for ALS: **healeyalsplatform@mgh.harvard.edu**

If you have questions about a specific study listed on **ClinicalTrials.gov**, you can reach out to the study team. Look for the Contacts and Locations table under each study.

In addition to exploring clinical research, some people living with ALS consider trying alternative or off-label treatments (AOTs). ALSUntangled provides scientific reviews of AOTs to help people living with ALS make more informed decisions.

Explore treatment reviews, questions and answers, podcast episodes, and more at ALSUntangled.com.

Step 2: Think about your needs and goals

Now that you've learned the basics about ALS clinical research, it's time to think about your needs and goals. Why do you want to participate in clinical research? What do you want to get out of participating in a research study?

Keep in mind that participating in a clinical research study is an investment of your time and energy.

Most clinical trials may require weekly to monthly appointments across several months. Observational studies often have fewer appointments. While some study appointments can be done virtually, in most cases you'll need to go to a hospital or medical center. At these appointments, you'll answer questions about your health and get medical tests done, like blood draws or lumbar punctures. If you have a busy schedule, other responsibilities, or difficulty with mobility, it could be more difficult to make multiple trips to the study location

Some clinical trials will reimburse you (pay you back) for travel and parking costs, while others may provide you a stipend or allowance for your participation. No study should ever ask you to cover the cost of a drug or treatment outright. Your study team can further explain any out-of-pocket expenses associated with your participation.

Finally, some people living with ALS ask a support person, like a partner, family member, or close friend, to come with them to study appointments. As you're learning about clinical research, it's a good idea to talk to your loved ones and find out who might be able to support you during a research study.

Step 3

Find a study

Next, look for clinical research studies that align with your needs. Ask your doctor or ALS care team if they know of any studies that might be a good fit for you based off the type of ALS you have. You can also use the websites listed under Step 1 to find studies.

As you review study descriptions, look for information about where the study will happen and how long it will last. Some clinical trials test the same study drug in multiple locations, making it possible for more people to participate.

Study descriptions may also list eligibility criteria, or rules about who can participate in the study. For example, researchers may be looking for people in a certain age group or people who have a specific type of ALS.

What are my rights as a clinical research study participant?

Before you can participate in a clinical research study, the research team will ask you to sign an informed consent form. Informed consent means you understand what will happen during the study — including possible risks — and you agree to participate. You have the right to take your time reading the consent form, ask as many questions as you like, and get a copy of the form after you've signed it.

Participating in research is voluntary and you have the right to be treated with respect.

Step 4:

Talk to the research team

If you find a study that you're interested in joining, contact the research team. If your doctor told you about the study, they can help you get in touch with the research team. If you found the study online, the study description will usually include contact information or an online form to fill out.

If the research team thinks you may be a good fit for the study, they may ask you questions and review information about your health, like your:

- **ALS symptoms**
- **Family medical history**
- **Breathing scores**
- **ALS Functional Rating Scale (ALFRS-R) scores**
- **Genetic testing results**
- **Medicines**

Based on all this information, the team will decide if you qualify to participate in the study. If you qualify, they'll explain how the study works and answer any questions you may have. Check out the right side of this page for a list of questions to ask the research team.

Step 5:
Talk to your doctor or ALS care team

Share what you've learned about the study with your doctor or ALS care team. They can help you decide if it's a good idea to participate in the study based on your personal situation and your health care needs.

Questions to ask before participating in a study

Participating in a research study is an important decision. It's important to ask questions and make sure the study is right for you. Here are some questions you may want to ask the research team.

What to expect during the study

- How long will the study last?
- Where will the study take place?
- What are the possible risks of participating in the study?
- Will I get any medicine or treatment as part of the study?
- Will I need to have any medical tests during the study? If so, will I find out my results?

Health care during the study

- Can I keep seeing my regular doctors during the study?
- Will you communicate with my doctors and let them know how I'm doing?
- Can I keep taking my current medicines while I'm participating in the study?

Health insurance and costs

- Do I need to have health insurance to participate in the study?
- Will I be reimbursed (paid back) for expenses related to the study, like travel or parking costs?

What happens after the study

- Can I keep receiving treatment after the study ends?
- Will you tell me the results of the study?
- Will you tell me if I was taking the actual study drug?
- Who can I contact if I have questions after the study ends?

Step 6:
Check in with yourself and make a decision

Finally, check in with yourself and decide if you want to participate in the study. Is this study the right fit for you? Here are a few questions to consider:

What do I expect to get out of this study?

What are the benefits and risks of participating in this study?

Will I need help with traveling to study appointments, taking medicine, or getting medical tests done? If so, who can help me during the study?

It may also be helpful to talk through these questions with someone you trust. **Remember, there's no right or wrong answer — it's all about what works best for you.**

> **“ After I got diagnosed, it was very important for me to get involved. I continue to look for any observational research studies I can participate in because I want to do everything in my power to help answer the 'why's' of ALS.**

> **“ I was surprised by a couple of things when participating in research. One was how it made me feel so hopeful and encouraged to be doing something proactive toward finding a cure. And the other surprise was how passionate and approachable the researchers themselves have been and to see this happening around the whole world is inspiring.**

Join the National ALS Registry

Signing up for the National ALS Registry is another way to get involved with ALS research. Today's research may lead to a better understanding and potential treatments tomorrow. By joining the National ALS Registry, being counted, and taking risk factor surveys, people living with ALS can help find answers. When you sign up for the registry at **CDC.gov/ALS**, you will receive notifications for clinical trials and other research studies.

Notes

LES TURNER ALS FOUNDATION®

Founded in 1977, the Les Turner ALS Foundation is the oldest independent ALS group in the country. For more than 45 years, it has been our mission to provide the most comprehensive care and support to people living with ALS and their families so they can confidently navigate the disease and have access to the most promising therapies. We treat each person like family, supporting them every step of the way, and provide their loved ones with answers and encouragement. Our Les Turner ALS Center at Northwestern Medicine is advancing vital care and research in pursuit of life-enhancing treatments and a cure.

lesturnerals.org

NEALS Northeast Amyotrophic Lateral Sclerosis Consortium®

The Northeast Amyotrophic Lateral Sclerosis Consortium® (NEALS) is the world's largest consortium of clinical research sites dedicated to rapidly translating scientific advances into clinical research and new treatments for people with Amyotrophic Lateral Sclerosis (ALS) and motor neuron disease (MND). Through the ALS Clinical Research Learning Institute® (CRLI) Research Ambassador Program for people impacted by ALS, NEALS actively supports the integration of lived experience into the global drug development process by creating collaboration opportunities within the patient community, academia and industry that ensure a patient-centric approach to treatment discovery.

neals.org