

## Participating in clinical studies

Scientists have made enormous strides in understanding how Alzheimer's disease affects the brain. Many of these insights point toward new therapies and improved ways to diagnose the disease and monitor its progression.

At any given time, dozens of studies are recruiting participants to help explore these exciting new approaches. Every clinical study contributes valuable knowledge, regardless if favorable results are achieved.

Without study participants, however, progress is stalled, and scientists report growing difficulty finding enough volunteers to complete these studies.

### What is a clinical study?

A clinical study is any type of clinical research involving people, regardless of whether it is testing a specific intervention. Clinical studies can also look at other aspects of care, such as improving quality of life. Every clinical trial and study contributes valuable knowledge, regardless if favorable results are achieved.

### What is a clinical trial?

Clinical trials test new interventions or drugs to prevent, detect or treat disease.

#### Phases of clinical trials

Preclinical studies in laboratories establish a scientific basis for believing a drug is reasonably safe and may be effective.

- **Phase I** trials, the first stage of human testing, typically involve fewer than 100 volunteers. These studies look at the risks and side effects of a drug. Participants at this phase are often healthy volunteers.
- **Phase II** trials enroll up to a few hundred volunteers who have the condition the drug is designed to treat in order to gain further information about safety and to determine the best dosage. These trials are generally too small to provide clear evidence about a treatment's benefit.
- **Phase III** trials enroll several hundred to thousands of volunteers, often at multiple study sites worldwide. They provide the chief evidence for safety and effectiveness that the FDA will consider in deciding whether to approve a drug.
- **Phase IV** trials, also called post-marketing studies, are often required by the FDA after a drug is approved. During this phase, researchers continue to monitor the health of people taking the medication to gain further insight into its long-term safety and effectiveness.

## Types of Alzheimer's-related clinical research

- **Treatment trials** test new treatments or combinations of treatments.
- **Diagnostic studies** find new tests or procedures for diagnosing a disease or condition.
- **Prevention trials** investigate ways to prevent the onset of diseases.
- **Quality of life studies** explore ways to improve quality of life for individuals who have a chronic illness, their caregivers and family members.
- **Online studies** are web-based and conducted entirely online.

## Who can participate?

Everyone interested in advancing Alzheimer's research can participate, including:

- Individuals living with dementia.
- Caregivers.
- Healthy volunteers without dementia.

Individuals may qualify for a study if they meet specific criteria based on factors such as age, gender, disease stage, previous treatment history and other medical conditions.

## Benefits of participation

- Provides hope for people living with Alzheimer's, their families and future generations.
- Enables people living with the disease to play a more active role in their own health care.
- Gives access to potential treatments before they are widely available.
- Offers expert medical care at leading health care facilities — often free of cost — while participating in important medical research.

## Risks of participation

Patient safety is the most important aspect of every Alzheimer's clinical trial. The procedures for each study are reviewed by an Institutional Review Board not directly involved in the trial. However, there are risks to trials, including:

- Unpleasant or even serious side effects related to the potential treatments being studied.
- Ineffective experimental treatments.

## Placebos

Scientists have learned that people sometimes feel better, and even have improved results on medical tests, when they believe a treatment is helping them. Doctors may also convince themselves a treatment is working because they care about their patients.

There are two main strategies used to reduce the likelihood that hopes and beliefs will affect trial outcomes:

- **Trials are placebo-controlled.** Study participants are randomly chosen to receive the experimental treatment and some receive a placebo, an inactive pill, liquid or powder that has no treatment value. Experimental treatments are often compared to placebos to assess effectiveness.
- **Trials are “double-blinded.”** Participants and study staff are unaware of who receives the drug and who gets the placebo.

When a standard of care — a typical treatment plan for a condition — is available, it is often used instead of a placebo. In such cases, the experimental treatment and the standard treatment are compared.

### **Monitoring safety behind the scenes**

Although participants and study staff don't know who's getting the treatment and who's getting the placebo, most trials have a separate, independent Data Safety and Monitoring Committee that has access to this information. Committee members regularly analyze data and step in if they notice any worrisome patterns of serious side effects.

### **Informed consent: knowing what to expect**

Informed consent is the process of learning key facts about a study before deciding whether to volunteer. The Food and Drug Administration (FDA) requires potential participants to have complete information about the study in writing. Study staff members are required to meet with each prospective participant to explain risks, possible benefits and answer any questions. People who decide to join the study must sign an informed consent form. Individuals who are invited to participate in a study are not required to join. Participants are also free to leave a study at any time.

### **Matching participants to studies**

Enrolling the right participants helps researchers maximize the likelihood of accurately measuring the effect of an experimental treatment. Some drugs, such as antibiotics for infections, have an obvious effect that is fairly easy to detect. It is often more challenging to assess the impact of drugs for chronic, serious diseases, including Alzheimer's. To eliminate certain factors that make it harder to evaluate a treatment, researchers define “inclusion and exclusion criteria” for each clinical study. Examples of these criteria include:

- Limiting participants to a certain age range.
- Requiring participants to be in a certain stage of the disease being studied.
- Not allowing health conditions other than the one being studied.
- Not permitting use of certain medications other than the study drug.
- Requiring participation of a caregiver or “study partner.”

### Questions to ask the trial research team

Once you qualify for a trial, you will work closely with the research team to understand the benefits and risks of participation. You may find it helpful to go over the trial information with your doctor before making a final decision and signing an informed consent form with the research team.

The National Institute on Aging suggests asking the trial research team the following questions:

- What is the purpose of the study?
- What tests and treatments will be given?
- What are the risks?
- What side effects might occur?
- What may happen with/without this research?
- Can I continue with treatments for Alzheimer's and other conditions as prescribed by my regular doctor?
- How will you keep my doctor informed about my participation in the trial?
- Does the study compare standard and experimental treatments?
- How long will the study run?
- How much of my time is required?
- Where and when will the testing occur?
- How much flexibility will I have?
- How will it affect my activities?
- If I withdraw, will this affect my normal care?
- Will I learn the results?
- Could I receive a placebo?
- What steps ensure my confidentiality?
- Are expenses reimbursed?
- Will I be paid?

Be sure to bring a list of any additional questions you have for the research team.

### How to find a study near you

**Alzheimer's Association TrialMatch®** is a service that provides customized lists of clinical studies based on user-provided information. The free, easy-to-use platform allows you to see which studies are a good fit for you or a family member. Search for studies, sign up for study updates, or connect with researcher teams with the click of a button.

To learn more, visit [alz.org/TrialMatch](https://alz.org/TrialMatch). You can also call 800.272.3900 or email [TrialMatch@alz.org](mailto:TrialMatch@alz.org) to get started.

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