

Participating in Clinical Trials and Research

Over the last 15 years, scientists have made enormous strides in understanding how Alzheimer's disease affects the brain. Many of these recent insights point toward new breakthroughs for treatment or prevention, as well as improved ways to diagnose the disease and monitor its progression.

Scientists continue to make advances, and progress is advancing very quickly on many fronts. In fact, at any given time, several hundred studies are recruiting participants to help explore these exciting new approaches. Every clinical study contributes valuable knowledge, whether or not the experimental strategy works as hoped.

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

If you or a loved one have Alzheimer's, a related disorder or memory loss — or even if you don't — you could help advance our knowledge about this illness. By participating in a clinical study, you may help some of these new treatments, preventive strategies and diagnostic tools become a reality.

What is a clinical study?

A clinical study is any medical research project involving human volunteers. Research into improved approaches usually begins with laboratory work or animal studies. Following early success with these methods, new strategies must demonstrate their effectiveness in the final proving ground of human testing.

What is a clinical trial?

A clinical trial is a specific type of study in which one group of volunteers gets an experimental treatment, while a similar group gets a placebo (a look-alike “dummy treatment”). Scientists evaluate the effect of the new treatment by comparing outcomes in the two groups.

Phases of clinical trials

The U.S. Food and Drug Administration (FDA), which regulates medical products and drugs, oversees a rigorous process, based on sequential phases, for testing experimental treatments. The treatments must perform well enough in each phase to be allowed to progress to the next one. If a treatment performs adequately in all stages through Phase III, the FDA reviews all the data and determines whether to approve the drug for use in general medical practice.

These are the phases of clinical trials:

- **Preclinical studies** in the laboratory establish a scientific basis for believing a treatment is reasonably safe and may be effective.
- **Phase I trials**, the first stage of human testing, typically enroll fewer than 100 volunteers. These studies are primarily concerned with assessing risks and side effects associated with a drug.

- **Phase II trials** enroll up to a few hundred volunteers with the condition the drug is designed to treat. These studies provide further information about safety and focus on determining the best dose of a drug. Scientists also watch for signs of effectiveness, but Phase II trials are generally too small to provide clear evidence about benefit.
- **Phase III trials** enroll several hundred to thousands of volunteers, often at multiple study sites nationwide. They provide the chief evidence for safety and effectiveness that the FDA will consider in deciding whether to approve a new drug.
- **Phase IV trials**, also called post-marketing studies, are often required by the FDA after a drug is approved. The trial sponsor must monitor the health of individuals taking the drug to gain further insight into its long-term safety and effectiveness and the best way to use it.

Ensuring accuracy of study results

Scientists have learned that people can sometimes feel better, and even have improved results on medical tests, just because they believe a treatment is helping them. Doctors can also convince themselves a treatment is working because they care about their patients and want to help them get better. There are two main strategies to reduce the likelihood that hopes and beliefs will affect the outcome of clinical trials:

1. **Trials are “placebo-controlled.”** This means that some study participants are randomly chosen by a computer to receive the experimental treatment and some receive a “placebo” (an inactive, look-alike treatment).
2. **Trials are “double-blinded.”** This means that neither participants nor study staff know who is getting the drug and who is getting the placebo.

Some studies are designed so the group of participants getting the treatment is larger than the group receiving the placebo. And some studies can be designed so all participants get the treatment for part of the study.

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 FDA requires potential participants to be given complete information about the study in writing. Study staff are required to meet with each prospective participant to explain risks and 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 and related disorders. 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"

How to find a study near you

If you would like to consider participating in research, call our 24/7 Helpline at 1.800.272.3900 to learn about studies in your area. Or, visit our online [Clinical Trials Index](#) at www.alz.org. Your local Alzheimer's Association may be aware of additional opportunities in your area.

The Alzheimer's Association is the leading voluntary health organization in Alzheimer care, support and research.

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