CLINICAL TRIALS

What Are Clinical Trials and Studies?

On this page:

Clinical research is medical research involving people. There are two types, observational studies and clinical trials.

Observational studies observe people in normal settings. Researchers gather information, group volunteers according to broad characteristics, and compare changes over time. For example, researchers may collect data through medical exams, tests, or questionnaires about a group of older adults over time to learn more about the effects of different lifestyles on cognitive health. These studies may help identify new possibilities for clinical trials.

Clinical trials are research studies performed in people that are aimed at evaluating a medical, surgical, or behavioral intervention. They are the primary way that researchers find out if a new treatment, like a new drug or diet or medical device (for example, a pacemaker) is safe and effective in people. Often a clinical trial is used to learn if a new treatment is more effective and/or has less harmful side effects than the standard treatment.

Other clinical trials test ways to find a disease early, sometimes before there are symptoms. Still others test ways to prevent a health problem. A clinical trial may also look at how to make life better for people living with a life-threatening disease or a chronic health problem. Clinical trials sometimes study the role of caregivers or support groups.

Before the U.S. Food and Drug Administration (FDA) approves a clinical trial to begin, scientists perform laboratory tests and studies in animals to test a potential

therapy's safety and efficacy. If these studies show favorable results, the FDA gives approval for the intervention to be tested in humans.

Read and share this infographic (PDF, 317K) to learn why researchers do different kinds of clinical studies.

What are the four phases of clinical trials?

Clinical trials advance through four phases to test a treatment, find the appropriate dosage, and look for side effects. If, after the first three phases, researchers find a drug or other intervention to be safe and effective, the FDA approves it for clinical use and continues to monitor its effects.

Clinical trials of drugs are usually described based on their phase. The FDA typically requires Phase I, II, and III trials to be conducted to determine if the drug can be approved for use.

- A **Phase I trial** tests an experimental treatment on a small group of often healthy people (20 to 80) to judge its safety and side effects and to find the correct drug dosage.
- A **Phase II trial** uses more people (100 to 300). While the emphasis in Phase I is on safety, the emphasis in Phase II is on effectiveness. This phase aims to obtain preliminary data on whether the drug works in people who have a certain disease or condition. These trials also continue to study safety, including short-term side effects. This phase can last several years.
- A **Phase III trial** gathers more information about safety and effectiveness, studying different populations and different dosages, using the drug in combination with other drugs. The number of subjects usually ranges from

several hundred to about 3,000 people. If the FDA agrees that the trial results are positive, it will approve the experimental drug or device.

- A **Phase IV trial** for drugs or devices takes place after the FDA approves their use. A device or drug's effectiveness and safety are monitored in large, diverse populations. Sometimes, the side effects of a drug may not become clear until more people have taken it over a longer period of time.

Mr. Jackson's story

Mr. Jackson is 73 years old and just found out that he has **Alzheimer's disease**. He is worried about how it will affect his daily life. Will he forget to take his medicine? Will he forget his favorite memories, like the births of his children or taking part in the March on Washington with Martin Luther King, Jr.? When Mr. Jackson **talked to his doctor** about his concerns, the doctor told him about a clinical trial that is testing a possible new Alzheimer's treatment. But Mr. Jackson is not sure about this clinical trial business. He does not want to feel like a lab rat or take the chance of getting a treatment that may not work or could make him feel worse. Dr. Moore explained that there are both risks and benefits to being part of clinical trials, and she talked with Mr. Jackson about these studies—what they are, how they work, and why they need volunteers. This information helped Mr. Jackson feel better about clinical trials. He plans to learn more about how to join a study.

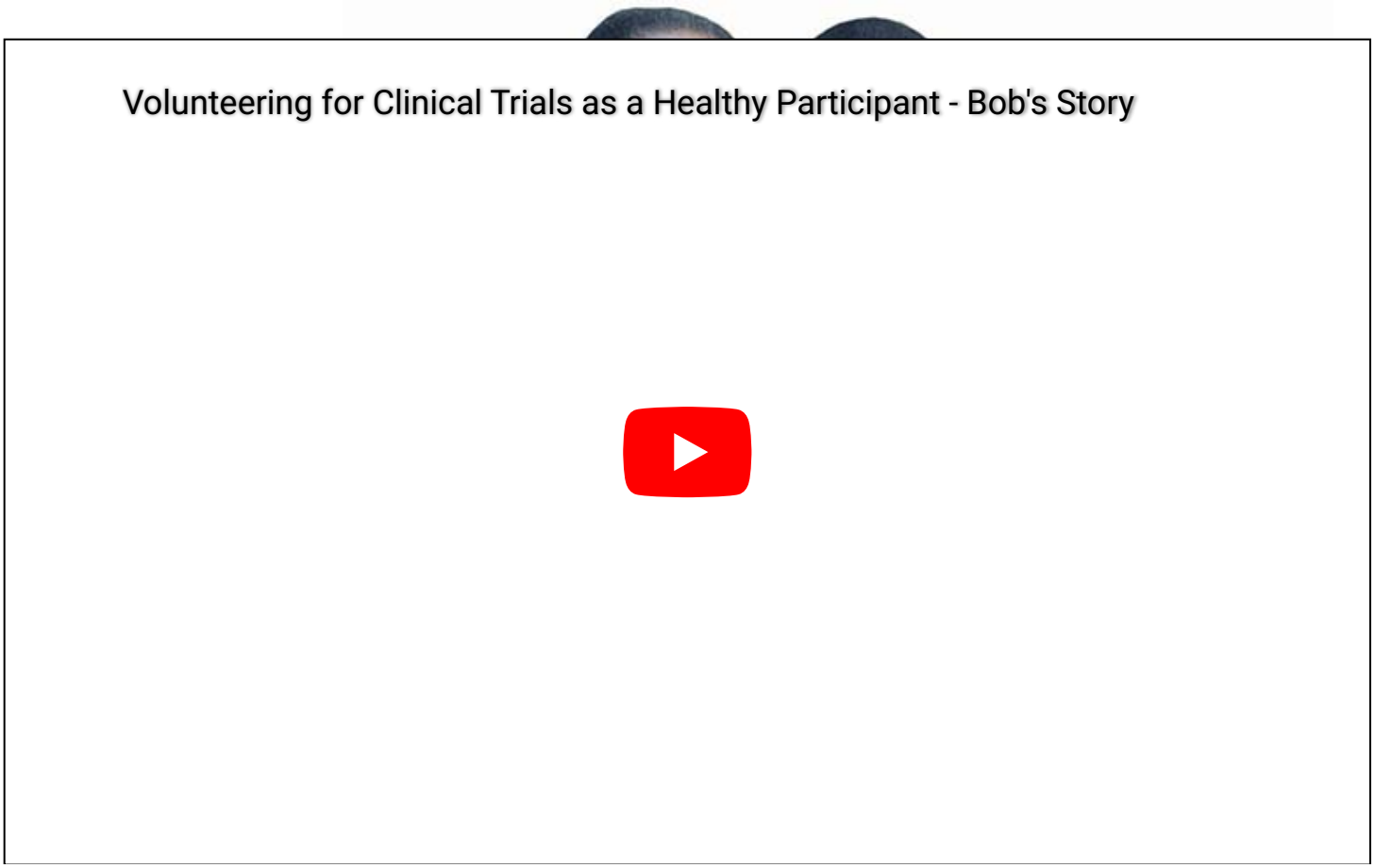
Like Mr. Jackson, you might have heard of clinical trials but may not be sure what they are or if you want to join one. Here is some information that can help you decide if participating in a clinical trial is right for you.

Why participate in a clinical trial?

There are many reasons why people choose to join a clinical trial. Some join a trial because the treatments they have tried for their health problem did not work. Others participate because there is no treatment for their health problem. By being part of a clinical trial, participants may find out about new treatments before they are widely available. Some studies are designed for, or include, people who are healthy but want to help find ways to prevent a disease, such as one that may be common in their family.

Many people say participating in a clinical trial is a way to play a more active role in their own health care. Other people say they want to help researchers learn more about certain health problems. Whatever the motivation, when you choose to participate in a clinical trial, you become a partner in scientific discovery. And, your contribution can help future generations lead healthier lives. Major medical breakthroughs could not happen without the generosity of clinical trial participants—young and old.

Watch a video of a participant explaining why he decided to join a study:



Here's what happens in a trial:

1. Study staff explain the trial in detail and gather more information about you.
2. Once you have had all your questions answered and agree to participate, you sign an informed consent form.
3. You are screened to make sure you qualify for the trial.
4. If accepted into the trial, you schedule a first visit (called the "baseline" visit). The researchers conduct cognitive and/or physical tests during this visit.
5. You are randomly assigned to a treatment or control group.
6. You and your family members follow the trial procedures and report any issues or concerns to researchers.
7. You may visit the research site at regularly scheduled times for new cognitive, physical, or other evaluations and discussions with staff. At these visits, the research team collects information about effects of the intervention and your safety and well-being.
8. You continue to see your regular physician for usual health care throughout the study.

Where can I find a clinical trial?

There are many ways you can get help to find a clinical trial. You can talk to your doctor or other health care provider. Or, you can search ClinicalTrials.gov. You can sign up for a registry or matching service to connect you with trials in your area. Support groups and websites that focus on a particular condition sometimes have lists of clinical studies. Also, you may see ads for trials in your area in the newspaper or on TV.

Learn more about participating in Alzheimer's disease and related dementias clinical trials and research.

What is the next step after I find a clinical trial?

Once you find a study that you might want to join, contact the clinical trial or study coordinator. You can usually find this contact information in the description of the study. The first step is a screening appointment to see if you qualify to participate. This appointment also gives you a chance to ask your questions about the study.

Let your doctor know that you are thinking about joining a clinical trial. He or she may want to talk to the research team about your health to make sure the study is safe for you and to coordinate your care while you are in the study.

How do researchers decide who will participate?

After you consent, you will be screened by clinical staff to see if you meet the criteria to participate in the trial or if anything would exclude you. The screening may involve cognitive and physical tests.

Inclusion criteria for a trial might include age, stage of disease, sex, genetic profile, family history, and whether or not you have a study partner who can accompany you to future visits. Exclusion criteria might include factors such as specific health conditions or medications that could interfere with the treatment being tested.

Many volunteers must be screened to find enough people for a study. Generally, you can participate in only one trial or study at a time. Different trials have different criteria, so being excluded from one trial does not necessarily mean exclusion from another.

Why are older and diverse participants important in clinical trials?

It is important for clinical trials to have participants of different ages, sexes, races, and ethnicities. When research involves a group of people who are similar, the findings may not apply to or benefit everyone. When clinical trials include diverse participants, the study results may have a much wider applicability.

Researchers need the participation of older people in their clinical trials so that scientists can learn more about how the new drugs, therapies, medical devices, surgical procedures, or tests will work for older people. Many older people have special health needs that are different from those of younger people. For example, as people age, their bodies may react differently to drugs. Older adults may need different dosages (or amounts) of a drug to have the right result. Also, some drugs may have different side effects in older people than younger people. Having seniors enrolled in drug trials helps researchers get the information they need to develop the right treatment for older people.

Researchers know that it may be hard for some older people to join a clinical trial. For example, if you have many health

problems, can you participate in a trial that is looking at only one condition? If you are frail or have a disability, will you be strong enough to participate? If you no longer drive, how can you get to the study site? Talk to the clinical trial coordinator about your concerns. The research team may have already thought about some of the obstacles for older people and have a plan to make it easier for you to take part in the trial.

Read more about diversity in clinical trials.

Download and share this tip sheet in Chinese (PDF, 455K).

Share this infographic and help spread the word about the benefits of participating in clinical trials and studies.

What happens when a clinical trial or study ends?

Once a clinical trial or study has ended, the researchers will collect and analyze the data to see what next steps are needed as a result of the findings. As a participant, you should be provided information before the study starts about how long it will last, whether you will continue receiving the study treatment after the trial ends, if applicable, and how you will be kept informed about the results of the study. Be sure to ask if you have specific questions. Learn more about **what happens when a clinical trial or study ends**.

Questions to ask before participating in a clinical trial

The following are some questions to ask the research team when thinking about a clinical trial. Write down any questions you might have and bring your list with you when you first meet with the research team.

About the trial

- What is this study trying to find out?
- What treatment or tests will I have? Will they hurt? Will you give me the test or lab results?
- What are the chances I will get the experimental treatment or the placebo?

- What are the possible risks, side effects, and benefits of the study treatment compared with my current treatment?
- How will I know if the treatment is working?
- How long will the clinical trial last?
- Where will the study take place? Will I have to stay in the hospital?
- Will you provide a way for me to get to the study site if I need it, such as a rideshare service?
- Can I do any part of the trial with my regular doctor? Is there a closer clinical trial to me?
- How will the study affect my everyday life?
- What steps ensure my privacy?

Medical care

- How will you protect my health while I am in the study?
- What happens if my health problem gets worse during the study?
- Can I take my regular medicines while in the trial?
- Who will be in charge of my care while I am in the study? Will I be able to see my own doctor?
- How will you keep my doctor informed about my participation in the trial?
- If I withdraw, will this affect my normal care?

Costs and reimbursement

- Will being in the study cost me anything? If so, will I be reimbursed for expenses such as travel, parking, or lodging?
- Will my insurance pay for costs not covered by the research trial, or will I need to pay out of pocket? If I don't have insurance, am I still eligible to participate?
- Will I need a study partner? If so, how long will he or she need to participate? Will my study partner be compensated for his or her time?

After the trial ends

- Will you follow up on my health after the end of the study?
- Will you tell me the results of the study?
- Whom do I call if I have more questions?

For more information about clinical trials

ClinicalTrials.gov
www.clinicaltrials.gov

U.S. Food and Drug Administration
888-463-6332
druginfo@fda.hhs.gov
www.fda.gov

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