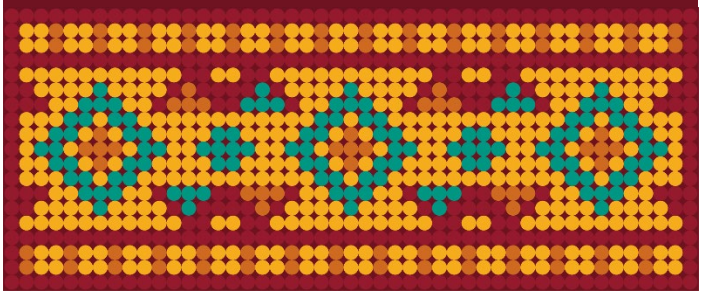
Understanding Clinical Trials



Learn about clinical trials, how they work, things to consider before participating in a trial, and more.

What is a clinical trial?

Clinical trials are studies where scientists (often called researchers) recruit research participants (also known as volunteers) to test new treatments, vaccines, or other interventions for preventing disease to make sure they are safe and effective.[1] These treatments, vaccines, or interventions may include new drugs or new drug combinations, new surgical procedures, new medical devices, or new ways to use existing treatments, vaccines, or other prevention strategies.

What is a protocol?

A protocol is a plan that researchers follow when conducting clinical trials. This plan helps answer questions such as:[1]

- Who is eligible to participate in the trial?
- How many people are needed to determine whether a treatment, vaccine, or other intervention is effective and safe?
- What samples are collected (ex., urine, blood, saliva), and how often will samples be collected?
- Will medical records or other information be collected?

Why are clinical trials conducted?

Researchers conduct clinical trials for a variety of reasons, including but not limited to:[1]

- Determining if a new drug or treatment, vaccine, or other intervention is safe for people to use.
- Testing whether a new drug or treatment, vaccine, or other intervention is effective (i.e., if it works better than what is standardly being used).
- Finding new ways to treat symptoms or prevent the development or recurrence of a disease or medical condition.
- Exploring ways to improve the quality of life for people with chronic illness.

What are the phases of clinical trials?

Clinical trials occur in four phases, and each phase has a different purpose that helps researchers answer different questions.[2] For prevention trials, all four phases of clinical trials will enroll healthy volunteers. In treatment trials, phase 1 trials are conducted with healthy volunteers, whereas people who have the condition or disease are usually brought in during phase 2. Any experimental drug being studied is usually tested in pre-clinical settings (ex., in animal studies) to ensure safety before testing on people begins.

Phase 1: Researchers give the experimental drug or treatment to approximately 20 – 100 healthy people for the first time. They evaluate if the treatment is safe, identify side effects, and determine appropriate dosage amounts.

Phase 2: The experimental drug or treatment is given to approximately 100 – 300 people to determine if it is effective (this is determined with questions like: does the drug or treatment work to reduce symptoms or treat the disease?) and to further evaluate its safety.

Phase 3: Researchers give the experimental drug or treatment to approximately 300 – 3,000 people. In this phase, researchers confirm its effectiveness, continue monitoring side effects, compare it with other treatments available, and collect information that will allow the experimental drug or treatment to be used safely.

Phase 4: This phase occurs after the experimental drug or treatment has been approved by the U.S. Food and Drugs Administration (FDA) or granted FDA Emergency Use Authorization (EUA) and marketed for use. Researchers continue monitoring the drug and treatment after approval and gather additional information about the treatment or drug's benefits, risks, and best uses.

For more information about FDA EUAs visit:
https://www.fda.gov/emergency-preparedness-and-response/mcm-legal-regulatory-and-policy-framework/emergency-use-authorization

For more information about the FDA approval process visit:
https://www.fda.gov/drugs/development-approval-process-drugs

Prepared by Urban Indian Health Institute, a division of Seattle Indian Health Board

Published February 24, 2021

What are research participants?

Research participants, also known as volunteers, are people who voluntarily choose to participate in a research study. For example, say the Urban Indian Clinic (UIC) wants to see if people who eat only salads for dinner lose weight versus those who eat only pizza for dinner. They might put up a flyer about the study. You see the flyer and call UIC for more information. After receiving all the information about the study, including the possible risks and benefits, you decide you want to join. You let UIC know that you would like to voluntarily participate and, after you give your informed consent, you are now a research participant. As a research participant, you will now eat only salad or pizza for dinner until the study ends. **Participation in a study is completely voluntary. You may withdraw and leave a study at any time, even if the study is not over.**[1]

Who can participate in a clinical trial?

All clinical trials have guidelines about who can and cannot participate. This is called "eligibility criteria." Some trials have volunteers who are healthy, others have volunteers who may have illnesses. The factors that allow you to participate are known as "inclusion criteria." The factors that disqualify you from participating are called "exclusion criteria." These criteria are based on things like age, gender, the type and stage of a disease, whether you have other medical conditions, and your history of treatment. For example, UIC's study on salad or pizza might only allow people who are 25 years and older to participate. If you are younger than 25, you would not be eligible to participate.

I identify as American Indian/Alaska Native; why should I participate?

When research studies include diverse populations, medical products are safer and effective for everyone.[3] When American Indians and Alaska Natives (AI/ANs) don't participate, research does not reflect how or if the experimental drug or treatment will work in AI/AN populations. This can lead to AI/ANs being misdiagnosed or given higher dosages of medication than needed.[4] Participation in clinical trials contributes to medical knowledge and helps the development of new medications and treatments that are safe and effective for our people.

How will I be protected?

Informed consent is the process in which potential and enrolled volunteers are provided with information about a clinical study. Informed consent helps to ensure you understand the benefits, risks, and alternatives to the study. **Signing an informed consent form or providing consent is not a contract. You may withdraw and leave a study at any time, even if the study is not over.**[1] For more information about informed consent, please visit: https://www.fda.gov/regulatory-information/search-fda-guidance-documents/informed-consent.

Every federally supported clinical trial that happens in the United States must be approved by an institutional review board (IRB). IRBs are dedicated to making sure you are not exposed to unnecessary risks or potential harm.[1]

In addition to the IRB, some clinical studies are monitored by a data and safety monitoring board (DSMB). The DSMB periodically reviews a study's progress and will stop a trial right away if the experimental treatment is not working or is harming volunteers.[1] DSMBs will also stop the study early if the treatment or intervention is found to be working in order to make it available.

What is a tribal institutional review board?

American Indians and Alaska Natives are well aware of the historical abuse, exploitations, and failed accountability that our people have experienced in the name of research. To ensure the protection, rights, and welfare of tribal members, tribal IRBs may review research for a clinical trial, and work to protect the sovereign status of tribal nations. Other bodies may perform research reviews for AI/ANs that reside outside of federally recognized tribal lands. For example, the salad and pizza study would require approval by the Urban Indian Clinic Research Review Committee before you or anyone else can volunteer to participate.

Tribal IRBs and research review committees review research proposals and assess potential benefits and risks to AI/AN populations. Furthermore, tribal IRBs and established tribal resolutions ensure that data collected by researchers from AI/AN populations will not be misused.[4]

What should I think about before participating in a clinical trial?

Before joining a clinical trial, it is important to consider both the potential benefits and risks of participating.[1]

Example questions to discuss with researchers or your provider:

If I get sick from the trial, who will pay for my health expenses?
Will the trial interfere with the medicine I am taking?
Am I too old or too young to participate?
If samples are taken from my body (ex., blood sample, urine, or saliva), will I get them back, or will they be destroyed?
What do I have to do during the trial?
What are the potential benefits of being in a study?
What are the potential risks of being in a study?
What tests or procedures will be done?
Who will see my records?
What will happen if I decide to withdraw from the study?
What will happen when the study ends?

For more information about genetics research, you can read the "American Indian and Alaska Native Genetics Resource Guide for Tribal Leaders and Citizens" on the National Congress of American Indians' Genetics Resource Center site (https://www.ncai.org/policy-research-center/initiatives/projects/genetics-resource-center).

For more information about clinical trials, please visit www.clinicatrials.gov or www.fda.gov.

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