MDA Muscular Dystrophy Association

Clinical Trials 101

A **clinical trial** is a type of study that evaluates whether a drug works or tests the effectiveness of a clinical care strategy in human volunteers (called participants).

There are two main types of clinical trials. In an **observational trial**, clinical data is gathered from patient medical visits. Observational studies help researchers learn more about a disease and the effects of clinical care.

An **interventional trial** is used to assess whether a type of intervention is effective. It can test a procedure, a drug, or a disease management protocol.

Regulatory agencies, doctors, and pharmaceutical companies rely on clinical trial data to make evidence-based decisions on the safety and effectiveness of treatments.

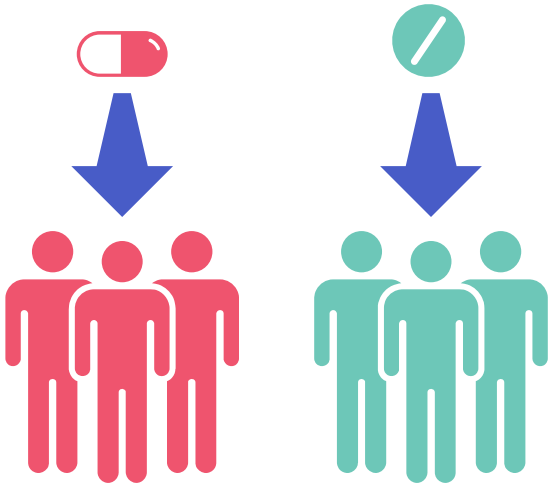
Participating in a clinical trial

Clinical trials provide the US Food and Drug Administration (FDA) with information to determine if new tests and treatments are safe and effective enough to be made available to the public. When you participate in a clinical trial, you are contributing to research that advances these new treatments, bringing them one step closer to people who need them.

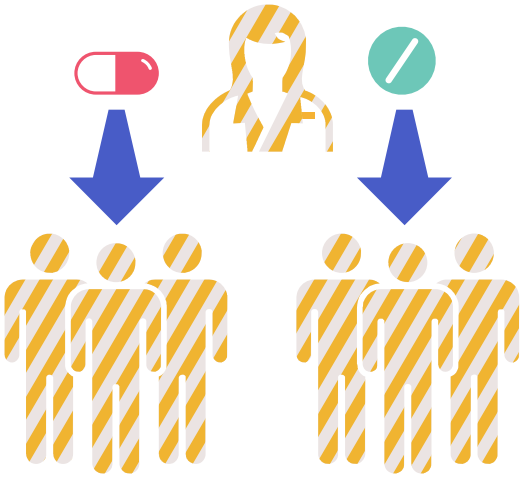
Clinical trials also produce new knowledge that improves our understanding of disease.

Finally, participating in a clinical trial gives you access to experimental, cutting-edge treatment options and a medical team that will carefully monitor your disease and overall health.

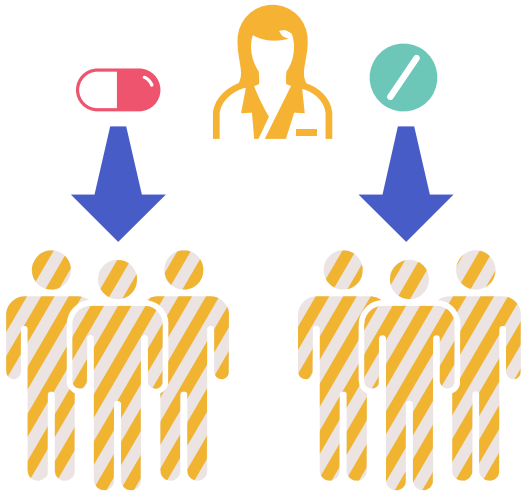
Common features in interventional trials



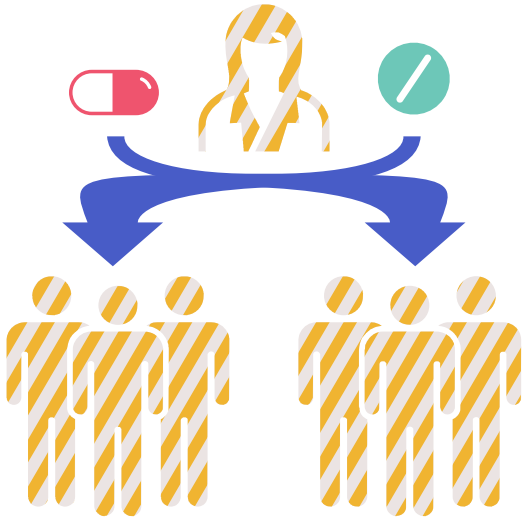
Placebo-controlled: A placebo is an inactive substance, like a sugar pill, that is given to patients in place of medication. During a placebo-controlled clinical trial, one group of participants (known as the control group) is given a placebo, while another group is given the drug being studied. Using this study design, researchers can compare the therapeutic effects of the drug against the effects of a substance with no medicinal activity.



Double-blind: A double-blind clinical trial is one in which neither the investigators conducting the study nor the participants know which participants are receiving the treatment or placebo until after the trial is over.



Single-blind: A single-blind clinical trial is one in which only the investigators conducting the study know which participants are receiving the treatment or placebo. The participants will not be given this information until after the trial is over in order to minimize the possibility of unrelated factors or biases affecting the study outcomes.



Randomized: A randomized clinical trial is one in which participants are randomly assigned to placebo or drug treatment groups.

The highest-quality studies are double-blind, randomized, placebo-controlled trials. The acronym DBRCT is commonly used for these types of studies.

Qualifying for a clinical trial

Your eligibility to participate in a clinical trial is based on inclusion and exclusion criteria. These criteria are determined as part of the clinical trial plan, with the goal of recruiting people who are in the target population for the drug under investigation. Sometimes the criteria are very narrow, resulting in the recruitment of people with few differences. This allows researchers to pick up on subtle signals that let them know if the drug is working.

- **Inclusion criteria** are characteristics that you must have to be included in the study. Some examples are having a particular diagnosis or having a relative with a particular diagnosis.
- **Exclusion criteria** are characteristics that disqualify you from inclusion in the study. Examples include not having a particular diagnosis, age, length of time since diagnosis, and current schedule of drugs and treatments.

Identifying trial options

The best place to start is by having a conversation with your doctor to learn more about clinical trial options. You can also use MDA's Clinical Trials Finder tool at **mda.org/clinical-trials** to find clinical trials that are currently recruiting in your area and for your diagnosis.

Responsibilities of clinical trial participants

If you enroll in a clinical trial, you will be expected to:

- Adhere to taking the trial medication according to the prescribed dosage and schedule
- Report any side effects or unexpected events during the trial
- Maintain your own health and avoid unnecessary risks while on the trial
- Discuss with the trial team any significant changes in behavior patterns that may impact or bias trial results

Providing informed consent

The main purpose of a clinical trial is to study new medical treatments in people. A clinical trial is first and foremost an experiment — meaning investigators don't have all the answers yet. To make an informed decision about whether to participate in a clinical trial, you need to understand the purpose of the research, including what your role will be and how the trial will work. This is known as informed consent.

If you are considering participation in a clinical trial, the researchers will provide you with an informed consent document. You should read it carefully and ask questions about any information you do not understand.

What are the phases of a clinical trial?

Clinical trials are typically conducted in four phases that provide answers to different questions about the safety and effectiveness of a drug or treatment under development.

Phase 1

- Tests the drug in a small group of people
- Assesses safety, drug dosage, and side effects

Phase 2

- Tests the drug in a larger group of people
- Evaluates effectiveness as well as safety

Phase 3

- Tests the drug in a large group of people
- Confirms effectiveness, monitors side effects, compares drug to available treatments, and collects information that will allow safe usage

Phase 4

- Conducted after a treatment is approved for use by the FDA
- Provides additional information, including risks, benefits, and best-use practices

Additional resources about clinical trials

nih.gov/health-information/nih-clinical-research-trials-you

fda.gov/consumers/health-education-resources/clinical-trial-participation

fda.gov/patients/drug-development-process/step-3-clinical-research

hhs.gov/ohrp/education-and-outreach/about-research-participation/index.html

Clinical Trials FAQ

Is there a fee to join a clinical trial?

There is typically no fee to join a clinical trial. All trial-associated clinic visits and drug or intervention costs are covered as part of the trial. Health insurance is not required to participate in a clinical trial; however, some routine lab exams may need to be paid for by patients and/or their health insurance.

Do I get paid for joining a clinical trial?

Some, but not all, clinical trials compensate participants. Financial support for travel is sometimes available.

Is it safe to join a clinical trial?

All clinical trials are approved and closely monitored for safety concerns by an Institutional Review Board (IRB) at the research institution conducting the trial. However, there is an inherent risk to any investigational therapy, and each person, with their physician, must weigh the potential benefits and risks before deciding to participate in a clinical trial.

What does the IRB do?

The IRB's goal is to protect the rights and welfare of participants enrolled in clinical trials at their institution.

How often will I need to visit a doctor or hospital for a clinical trial?

The frequency of visits will depend on the trial plan. In general, a study will consist of a screening visit, where your eligibility is confirmed, and the baseline is established. Then there will be a visit to receive the intervention and several follow-up visits to monitor disease progression and make sure the drug is safe.

Will I still see my primary healthcare provider while I'm in a clinical trial?

Yes. Most clinical trials provide trial-associated care during the term of the study, but they do not provide overarching healthcare. It is helpful for your primary healthcare provider to work with the research team to ensure that other medications or treatments will not conflict with the trial protocol.

What is a placebo, and why is it used in interventional clinical trials?

A placebo is an inactive substance, like a sugar pill, that is given in place of medication. During a placebo-controlled clinical trial, one group of participants (known as the control group) is given a placebo, while another group is given the drug being studied. Using this study design, researchers can compare the therapeutic effects of the drug against the effects of a substance without medicine in it.

What is the difference between a disease registry and a clinical trial?

Disease registries collect basic health information over time from volunteers with specific diseases or conditions. This information can then be used for many purposes, including historical studies that "look backward" and examine the outcomes resulting from prior patient care. Clinical trials are investigational; they "look forward" at the outcomes of participants who undergo a specific treatment or drug protocol to study that therapy.

Why is genetic testing important with regard to clinical trials?

Genetic testing can confirm your clinical diagnosis and potentially provide information about your prognosis and/or likelihood of responding to certain treatments. Therefore, when applicable, a confirmatory genetic test is often required to participate in a clinical trial.

What happens if I decide to leave a clinical trial while it's in process?

You can leave a clinical trial at any time. Depending on the study, leaving can be simple, with just a few requirements to transfer back to regular care. In other cases, you may need to be closely monitored until you are safely off the study drug. The researchers will generally ask to follow up with you from time to time. These follow-ups can provide them with additional information that may be important for the study, as well as to ensure your safety.

Can I participate in multiple clinical trials?

It is recommended that patients enroll in only one clinical trial at a time. Each trial is designed to focus on a specific therapy, and participation in multiple trials makes it difficult to identify the risks and benefits of each therapy on its own. However, some people participate in several clinical trials in succession.

How will participating in one clinical trial affect my ability to participate in another clinical trial?

Each clinical trial has a set of inclusion and exclusion criteria. These are the rules about who can participate in the study, and they may include characteristics such as genetic diagnosis, age, or medical history. Participation in one clinical trial may either make you eligible or ineligible for future studies, depending on the inclusion/exclusion criteria of the later study.

To learn more about clinical trials, contact the MDA Resource Center at **833-ASK-MDA1 (833-275-6321)** or **ResourceCenter@mdausa.org**.