MDA Muscular Dystrophy Association

Clinical Trials 101: A Guide for Individuals and Families

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.

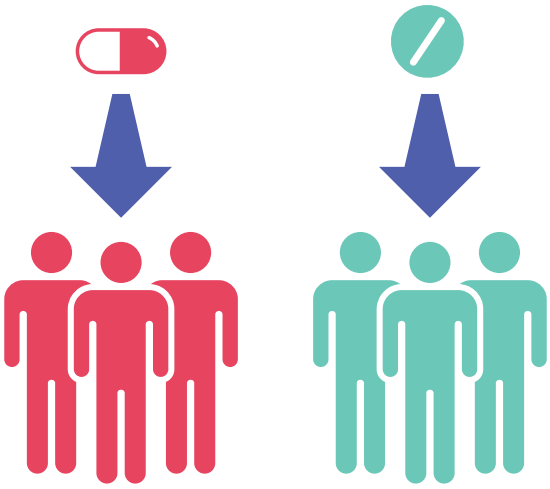
Participating in a clinical trial

Clinical trials provide the U.S. 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 patients that need them.

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

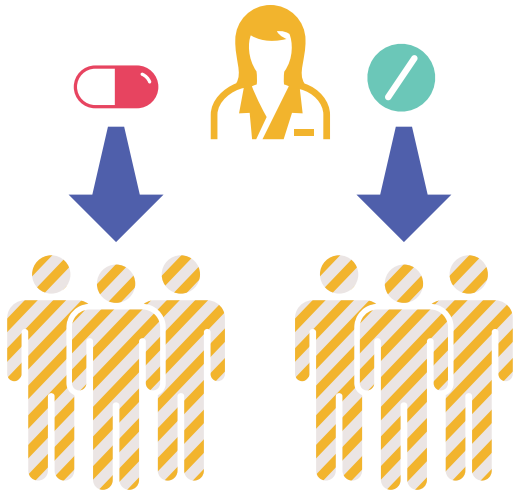
Finally, participating in a clinical trial gives you access to experimental, cutting-edge treatment options and a medical team that will carefully monitor your disease and overall health.

Common features in interventional trials



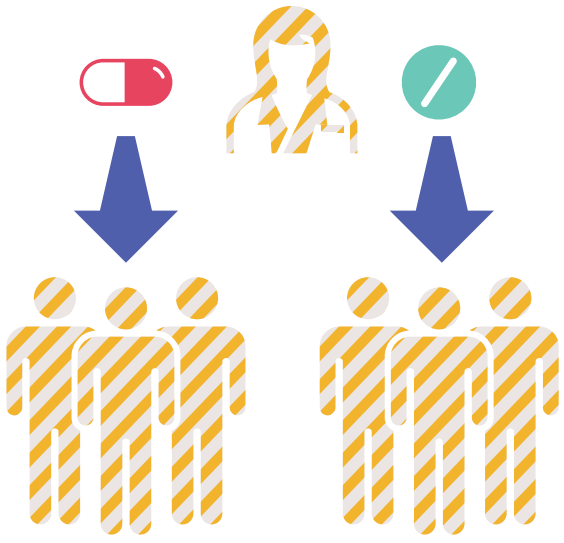
Placebo-Controlled: A placebo is an inactive substance, like a sugar pill, that is given to patients in place of medication.

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.

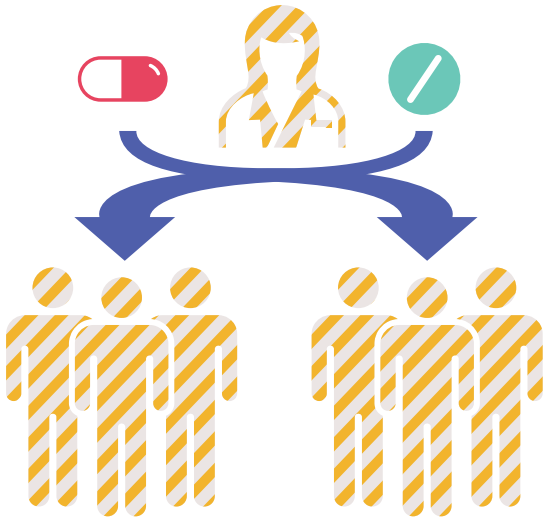


Single-Blind: A single-blind clinical trial is one in which only the investigators conducting the study know which treatment the participants are receiving.

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.



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



Randomized: A clinical trial that is randomized 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 patients in the target population for the drug under investigation. Sometimes the criteria are very narrow, resulting in recruitment of patients 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 of 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 on **mda.org** 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

- https://www.nih.gov/health-information/nih-clinical-research-trials-you
- https://www.fda.gov/patients/clinical-trials-what-patients-need-know
- https://www.fda.gov/patients/clinical-trials-what-patients-need-know/what-are-different-types-clinical-research
- https://www.fda.gov/patients/drug-development-process/step-3-clinical-research