NOVARTIS

# Understanding clinical trials

Information for potential participants and their loved ones

# Thank you for your interest

Before any medicine becomes available for use, it must first be studied in a clinical trial with the help of volunteers. Whether you're curious about how medical research works or thinking about taking part yourself, this booklet covers everything you need to know about clinical trials.

# What is a clinical trial?

A clinical trial (also called a clinical study) is a type of medical research. Some clinical trials are run to find out how changes in a person's behavior or lifestyle affect their health and wellbeing. Other trials help answer questions about an investigational medicine, such as:

- Is it safe?
- Is it effective?
- Does it work better than existing options?

**Thousands of people around the world take part in clinical trials every year. Some of these trials involve healthy volunteers, and others focus on people with a specific medical condition.**

Clinical trials are the foundation of modern medicine. All medicines today must undergo rigorous clinical testing, with regulatory authorities ensuring each one meets strict safety and effectiveness standards before it can be prescribed for use.

# Why are clinical trials important?

Clinical trials help advance medical knowledge so that we can learn more about medical conditions and how to diagnose, prevent, and treat them. Researchers are constantly working to evolve treatments, making them more effective, reducing side effects, and giving people alternatives that may suit them better than existing options.

# Why do people take part in clinical trials?

There are many reasons why people choose to take part in clinical trials. A few common reasons are:

- To contribute to research that may benefit others facing similar health conditions in the future
- The chance to access an investigational medicine that is not yet widely available
- For regular healthcare and close monitoring
- Hope for better treatment options
- To learn more about their own health condition

## What are the types of clinical trials?

There are two main types of clinical trials: interventional and observational trials. Interventional trials actively assess a treatment approach, such as a medicine, procedure, activity, or medical device, to see how it affects participants. Observational trials monitor a group of people over time, without introducing any new treatment, to collect data about a disease. This data can provide insights that may support the development of new treatments.

## What is a placebo and why is it used?

Many interventional trials are placebo-controlled. This means that some participants are given a placebo which looks like the investigational medicine but contains no active medicine. This comparison helps researchers to measure the effect, if any, that an investigational medicine has on symptoms.

Placebos are given in certain circumstances, such as when there are no approved treatments available for a medical condition, or if there is no agreement in the medical community about what the best treatment is. People may also be given the current standard of care in addition to the investigational medicine.

## What is meant by randomization and how does it work?

Randomization is a term used to describe the process of assigning participants to different treatment groups.

A computer randomly assigns participants, ensuring every participant has an equal chance of being in any group. Randomization also makes sure that each group has a similar mix of people of different ages, genders, and health statuses, which helps determine if the investigational medicine works effectively for everyone.

## What does double-blind mean?

In some clinical trials, neither the participant nor the trial team know who is receiving the investigational medicine and who is getting the placebo. This is called a "**double-blind**" trial. The approach eliminates bias and ensures that any differences observed are due to the investigational medicine and not some other factor, like receiving more health monitoring than usual. If there is an emergency, the trial doctor will be able to find out which trial medicine someone has been given.

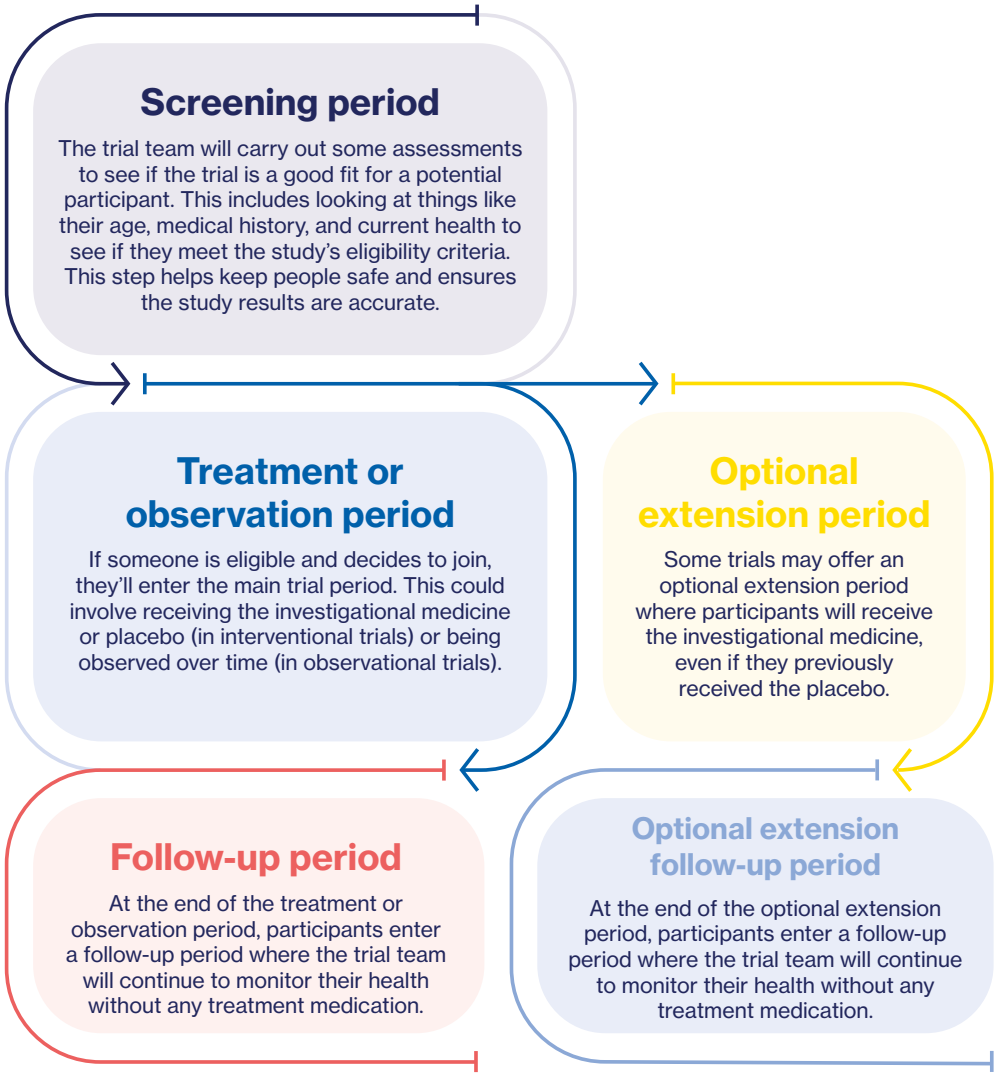
## What is an extension period?

At the end of some trials, participants may be invited to join an extension period. This is an optional continuation of a clinical trial where everyone is given the investigational medicine, even if they previously received a placebo. This means that both the participant and the trial team will know the participant is being given the investigational medicine. Extension periods are conducted so that researchers can continue to monitor the long-term effects of an investigational medicine.

# What does taking part in a clinical trial involve?

Every clinical trial is different, but most involve regular scheduled visits to the trial site along with assessments such as physical examinations, blood samples, and questionnaires.

Most clinical trials follow a similar structure. A typical trial consists of three main phases, as shown below:




# How are participants protected in clinical trials?

Participant safety is the top priority in every clinical trial. There are many safeguards in place to protect participants and ensure that trials are conducted ethically and responsibly. These include:

**Trial approval:**
A dedicated committee of people (called an ethics committee) review and approve a trial before it can begin. They check that the rights, safety, and dignity of participants will be respected, and decide if a trial is safe and ethical. Ethics committees are usually made up of members of the public, healthcare professionals, and researchers. In addition, regulatory authorities check that the trial complies with relevant laws and regulations, protects participant safety, and is planned and designed to the highest standards.

**Informed consent:**
Before a person can join a clinical trial, either they or their caregiver must be given all relevant information so they can decide whether to take part. A member of the study team will review an important document called an informed consent form with the person and/or their caregiver and answer any questions they have. This form includes information about the trial, such as its purpose, its length, and the risks and benefits of the trial to ensure that people have all the necessary information to make an informed decision.

**Data protection:**
Participant's personal data and medical samples will be labelled with a participant identification (ID) number. This number is unique to them and not related to or derived from information that identifies them (such as their name or date of birth).

**Deciding to leave:**
Participation in clinical trials is always voluntary. Participants can leave the trial at any time without giving a reason. This will not affect their current or future healthcare in any way. The trial team may ask participants to visit the clinic before they leave for some final health checks.

**A note on transparency and inclusivity**

Transparency and inclusivity are essential in clinical trials. All clinical trials must meet strict international and national ethical and scientific guidelines designed to protect participants. Researchers are committed to building trust through complete openness about trial procedures and ensuring everyone has an equal opportunity to participate and benefit from medical advances.

# What happens after a trial ends?

At the end of a clinical trial, researchers analyze the collected data. When an investigational medicine is proven to be safe and effective, health authorities may approve it for widespread use. Even if a medicine isn't approved, the research contributes valuable knowledge that advances medical knowledge.

# Thinking about joining a clinical trial?

We understand that taking part in a clinical trial is a big decision. If you're thinking about joining a trial, take the time to consider whether it's right for you. We also recommend discussing the trial with your doctor, family, and friends. If you decide to participate, you'll be joining a global community of volunteers who are helping advance medical knowledge and improving future care for people worldwide.

You can learn more about the clinical trials we're running all over the world by visiting us at novartis.com/clinicaltrials.