ERDERA

European Rare Diseases
Research Alliance

What is a Clinical Trial

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Co-funded by
the European Union

ERDERA has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement N°101156595.
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Introduction

Every medicine, vaccine, or treatment we use today had to be tested before it became available.

Scientists cannot just give a new drug to everyone, they must first check that it is **safe and works properly**.

A **clinical trial** is a research study that involves people and is designed to answer specific health questions.

These studies help doctors understand whether a new treatment is better, safer, or more effective than what already exists.

Clinical trials are an important part of improving healthcare for everyone, including young people.

Why Are Clinical Trials Important?

Clinical trials are the reason we have treatments for many serious diseases today.

They help researchers develop new medicines for conditions like asthma, cancer, or rare diseases.

They also help improve existing treatments, making them safer or easier to use.

Without clinical trials, doctors would not know which treatments actually work.

They also help ensure that medicines are safe for different groups of people, including children and teenagers.

How Do Clinical Trials Work?

Clinical trials follow a step-by-step process to make sure everything is done safely. Before testing in humans, treatments are studied in labs and sometimes on animals.

Then, the treatment is tested in humans in different **phases**:

Phase 1:

- Involves a small number of participants
- Focuses on safety and finding the right dose

Phase 2:

- Includes more participants
- Looks at how well the treatment works and continues safety checks

Phase 3:

- Involves large groups of people
- Compares the new treatment to existing ones

Phase 4:

- Happens after the treatment is approved / Monitors long-term safety and effectiveness

Who Can Take Part?

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Clinical trials include volunteers who choose to participate.

Some trials involve **healthy volunteers**, while others involve people with a specific condition.

Each trial has specific rules about who can join, called **eligibility criteria**.

For young people, parents or guardians are usually involved in the decision.

Participation is always **voluntary**, meaning no one is forced to take part.

Safety and Ethics

Clinical trials are carefully controlled to protect participants.

They are reviewed by independent groups called **ethics committees**.

These committees make sure the study is fair and that risks are minimized.

Doctors and researchers closely monitor participants throughout the trial.
If any serious problems occur, the trial can be stopped immediately.

Risks and Benefits

Benefits:

Participants may get access to new treatments before they are widely available.

They contribute to scientific research that can help others in the future.

They often receive close medical attention during the study.

Risks:

The new treatment may not work as expected.

There may be side effects that are not yet known.

Participation may require extra hospital visits or tests.

The role of YPAG

Young people can play an important role in shaping clinical trials.

Groups like YPAG help researchers design studies that are more suitable for young participants.

They can give feedback on things like:

Study materials (are they easy to understand?)

Study design (is it practical and fair?)

This helps make clinical trials more inclusive and effective.



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Thank you!



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