Understanding Clinical Trials: A Patient Guide

For patients and carers | RDW-01-06

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This guide explains how clinical trials work and what to ask before deciding whether to take part. A trial is research, so the benefits and risks are not fully known. Joining may help researchers learn, but it does not guarantee that you will benefit. Your decision should reflect your health, circumstances and priorities.

What a clinical trial is

A clinical trial tests an intervention in people, such as a medicine, device, procedure or approach to care. Researchers may study prevention, diagnosis, treatment or quality of life. An observational study is different: researchers collect information without assigning the intervention being studied. [1]

Every trial follows a protocol, a plan describing its question, who can join, procedures, monitoring and how results will be assessed. The principal investigator leads the research at a study site. The sponsor is the organisation or person responsible for initiating and managing the trial. Ask who is funding it and who to contact about concerns.

What the phases mean

Medicine trials often use phases. They describe the main research purpose, not a guarantee of safety or success. Some trials combine phases, and other types of research may not use these labels. [2]

- Phase 1 explores safety, dose and how the treatment behaves in the body. It may involve healthy volunteers or people with the condition.
- Phase 2 looks for evidence of benefit in people with the condition and gathers further safety and dose information.
- Phase 3 assesses benefit and safety, often by comparison with another treatment or a control group.
- Phase 4 studies a medicine after approval, for example to learn more about longer-term effects or its use in wider practice.

A treatment may still cause unexpected harm in any phase. Ask what is already known about the particular intervention and what the trial is trying to find out.

How a trial is designed

Groups and comparisons

Some trials have one treatment group. Others compare two or more groups, for example a new treatment against usual care. Ask what each group will receive and whether your existing treatment would continue. [3]

- Randomisation means assignment by chance, usually by computer. You and your clinician generally cannot choose the group. Ask about your chance of receiving each option.
- A control group provides the comparison. It may receive usual care, another treatment, a placebo or no study intervention, depending on the design.
- A placebo resembles the study treatment but has no active study medicine. In some trials, both groups also receive usual treatment. Ask exactly what would be withheld or continued.
- Blinding or masking means that participants, some researchers, or both do not know the treatment assignment. Ask who is blinded and how the assignment can be revealed if medically necessary.

What is being measured

An outcome is something measured to assess an effect, such as symptoms, daily functioning, hospital admissions or side effects. An endpoint is a specified outcome assessed at a defined time or in a defined way. Ask which outcomes matter most in this trial, and whether they reflect changes that matter to you.

Why rare disease trials may differ

Who can take part

Eligibility criteria are the rules for joining. They may concern diagnosis, genetic findings, age, previous treatment or organ function. Screening checks whether you meet them; it does not guarantee a place. Not being eligible does not mean your illness or need for care is less important. [1,3]

Making an informed choice

Consent is a continuing conversation

Informed consent means understanding the purpose, procedures, foreseeable risks, possible benefits and alternatives, then deciding freely. Ask for the participant information in a language and format you can use, enough time to consider it and a copy of anything you sign. You may involve someone you trust. Signing a form does not remove your right to ask questions or change your mind. [5]

The team should explain relevant new findings during the trial. For children or adults who cannot consent for themselves, legal permission and safeguards vary by country. Ask how the person will be involved, how their wishes will be respected and who can legally give permission.

Weigh the possible benefits and burdens

You may receive an intervention that helps, but it could be ineffective, less helpful than usual care or cause harm. Extra monitoring does not guarantee better health. Participation may mean additional tests, travel, time away from work, fatigue or pressure on carers. Ask what care is available if problems develop and what happens if the trial stops early. [6]

Before agreeing, confirm costs, travel and accommodation support, payments, insurance and arrangements for research-related injury. These differ between studies and countries. Ask what routine care remains available if you decline, and whether any effective study treatment could continue after the trial. Continued access is not automatic.

Leaving a trial

Participation is voluntary. You can choose to leave without giving a reason. Tell the team so they can arrange safe treatment changes and discuss follow-up; do not abruptly stop a study medicine on your own. Ask how your usual care will continue and which parts of participation you wish to stop. [5,6]

Your information and samples

Ask who can access your records, how data and samples will be stored, whether they will leave your country and whether future research or commercial use is planned. Leaving a trial may not mean that data already collected can be deleted or that analyses already completed can be undone. Ask what choices you have about further contact, samples and future use; the study arrangements and local law matter. [7]

Confirm consent, privacy, insurance and compensation details with the study team and, where needed, an appropriate local adviser. Keep the written answers with your study information.

Finding a trial and asking questions

Where to look

Ask your specialist team about relevant research. Patient organisations may also help you identify studies, but suitability must be checked by the trial team. WHO's International Clinical Trials Registry Platform search portal brings together records from multiple registries. [8] ClinicalTrials.gov also lists studies in many countries. [9]

Check the trial identifier, sponsor, study location, date updated and recruitment status. Contact the listed research team to confirm that a site is open and whether screening is possible. A registry listing is not a recommendation or proof that the intervention works; the US government does not approve the safety and science of every study on ClinicalTrials.gov. [9]

Questions to take to the research team

- What is the main question, and what is already known about benefits and harms?
- What would I receive in each group, and what are my alternatives?
- Which visits, tests and treatment changes would be required, and for how long?
- What symptoms should I report, and who can I contact outside office hours?
- What support is available for travel, disability, language needs and carers?
- Who pays for routine care, study procedures and treatment for research-related injury?
- What happens to my care, data and samples if I leave or the trial ends?
- How will I receive a plain-English results summary, and will individual findings be returned?

Key points

A trial aims to answer a research question; personal benefit is uncertain. Understand the comparison, the practical demands and your alternatives. Participation should be voluntary, with questions welcomed throughout. Ask about arrangements in your country and take time to decide whether the trial fits your life.

Keep the participant information sheet and contact details somewhere accessible. Do not send medical records to Rare Disease Watch to use this guide. Share records for screening only through a verified study team's agreed secure route.

If you become unwell during a trial, follow the study's safety instructions and seek urgent local medical help when needed. Tell the treating team that you are taking part in research.

References and further reading

Sources are numbered in order of first mention. Select a title to open its source URL. Sources checked on 3 November 2026. National sources describe their own systems; local rules and individual study arrangements may differ.

1. US National Institutes of Health. The Basics

2. US Food and Drug Administration. Step 3 Clinical Research

3. US National Institutes of Health. Glossary of Common Terms

4. European Medicines Agency. Clinical trials in small populations

5. US National Institutes of Health. Guiding Principles for Ethical Research

6. NHS. Clinical trials

7. UK Health Research Authority. Data subject rights and research exemptions

8. World Health Organization. ICTRP search portal

9. US National Library of Medicine. ClinicalTrials.gov

About this guide

This leaflet provides general information for patients and carers. It cannot determine whether an individual should join a particular trial. Study-specific risks, eligibility and legal arrangements need to be confirmed with the responsible research team.

https://rarediseasewatch.com/

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