NORD®
National Organization for Rare Disorders

Clinical Trials

Frequently Asked Questions

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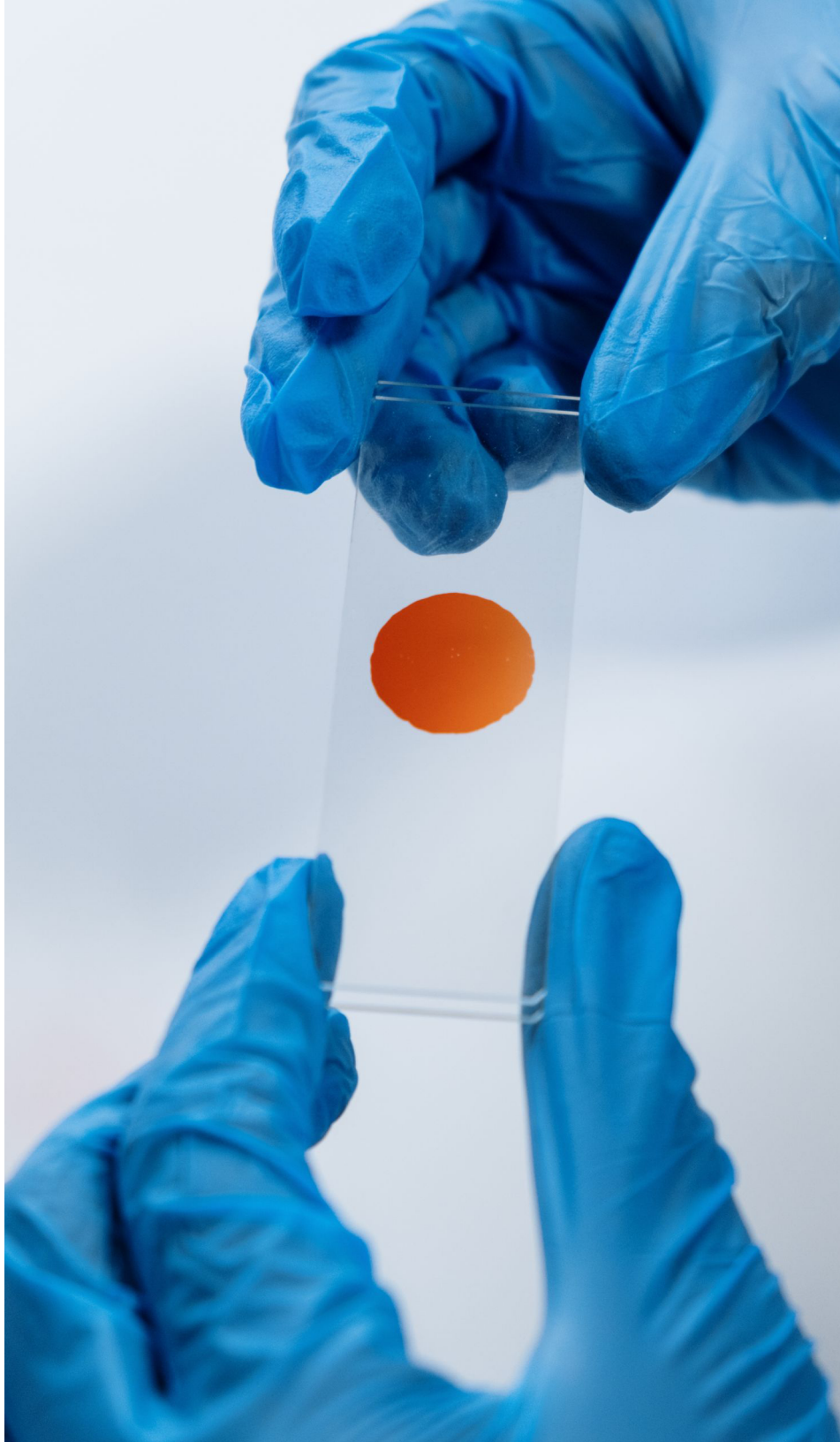

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Why are clinical trials important?

Clinical trials help researchers learn whether new treatments are safe and effective. They are required before most new medicines can be approved by the U.S. Food and Drug Administration (FDA) for widespread use. They also help researchers identify the best dose, understand possible side effects, and learn which patients are most likely to benefit. Every approved treatment available today was studied in clinical trials, and participation in research helps advance medical knowledge that may benefit future patients.

Where can I find a clinical trial for my condition?

Your healthcare provider or specialist may know a trial that could be a good fit for you. Rare disease centers of excellence and academic medical centers can also be helpful.

Disease Specific Patient Advocacy Organizations often share information about available clinical trials and can help families find resources and support.

ClinicalTrials.gov is a searchable database of clinical trials in the United States and around the world. You can search by medical condition, location and age.

The National Organization for Rare Disorders (NORD) Find Clinical Trials and Research Studies webpage allows you to search for studies by keyword. NORD's Information and Resource Services provide disease education, specialist referrals, and help finding financial or clinical trial assistance. Call the NORD Communication Center at 800-999-6673. The NORD Organizational Database enables you to find disease specific organizations for support.

Research Match is a free volunteer registry that connects people interested in research with researchers who are looking for participants.

What is informed consent?

Informed consent is the process of learning about a clinical trial before deciding whether to join. It explains the study's purpose, possible benefits and risks, what participation involves, and your rights. All of this is outlined in the informed consent document. Joining a trial is voluntary. You can take your time and ask questions before making the decision to join. Signing an informed consent form does not mean you must stay in the study.

Who can help me understand more about a particular trial that I am considering?

You can talk with your healthcare team to learn more and ask questions. Clinical research coordinators or study doctors can explain details about the site, frequency of visits, tests, travel and who can join. Patient advocacy organizations may also help explain general information about clinical research, although they cannot provide medical advice.

Clinical research is reviewed by an Institutional Review Board (IRB). If you have questions or concerns about the study, you can contact the IRB that reviewed the study. This information will be found on a study's informed consent form.

The informed consent document also provides a detailed explanation of the study.

What are some questions I should ask when deciding whether to participate in a trial?

- What is the purpose of the study?
- What is already known about the drug being tested?
- Who will take care of me during the trial?
- What will I need to do? (visits, travel time, tests, etc.)
- Who pays for the study, and will I have any costs?
- What are the possible side effects?
- What are the potential benefits of participating in the study?
- Can I continue taking my current medications?
- Who will have access to the information collected about me as a part of this study?
- If I no longer want to participate in the study, what should I do?

Will participating in a clinical trial cost me anything?

The costs of participating in a clinical trial vary depending on the study. The investigational treatment and research-related tests are often provided at no cost to participants. However, you or your insurance may still be responsible for routine medical care that you would receive even if you were not participating in the study. Some studies also reimburse or help cover expenses such as travel, lodging, meals, or childcare. Before deciding whether to participate, ask the study team what costs will be covered and whether any financial assistance is available.

Will I receive a placebo?

Not all clinical trials use a placebo. In some studies, participants receive either the investigational treatment or a placebo. In others, the investigational treatment is compared with the current standard treatment instead of a placebo. Whether a placebo is used depends on the purpose and design of the study. Before deciding whether to participate, the research team will explain how treatments are assigned to participants in the study and whether a placebo may be used.

What are some potential benefits of participating in a clinical trial?

Potential benefits of participating in a clinical trial will depend on the particular clinical trial. Some clinical trials may not have a direct benefit to participants. Some potential benefits may include:

- Access to an investigational treatment that is not yet widely available.
- Care from experts and more time with the research team than in regular medical visits
- A chance to discuss your condition and treatment options with the trial team
- An opportunity to help advance research that may benefit future patients and the broader rare disease community.

What are some potential risks of participating in a clinical trial?

Potential risks of participating in a clinical trial will depend on the particular trial. The study team can help you understand more about these risks. Depending on the study, some potential risks may include:

- The treatment may cause side effects, including serious side effects.
- The study procedures may have their own risks; for example, there are risks involved in having blood drawn, which may be done for research purposes.
- The treatment may not work for you.
- The treatment may work better, the same as, or worse than your current treatment.
- Some trials use a placebo, meaning you may not receive the study treatment.
- Participation may require extra visits, tests, travel, time away for work or childcare.

It can be stressful or disappointing if the treatment does not help.

What protections are in place for people participating in clinical trials?

- Trained research professionals conduct and oversee trials
- Trials are designed to reduce risks and protect participants
- Participants are closely monitored by experts throughout the study
- Trial sites have qualified staff and systems to support safety, appropriate medical care and collection of accurate study data.
- An institutional Review Board (IRB), an independent committee, reviews the study to protect participants' rights, safety and well-being.
- For clinical trials testing new drugs that are not yet FDA-approved, the FDA and other regulatory authorities establish rules that clinical trials must follow to protect participants.

Can I leave a trial after joining?

Yes, you may withdraw from the clinical trial at any time, for any reason.

Talk to the trial team first so they can help you leave safely. They may ask why you are leaving, but you do not have to give a reason.

What is a trial protocol, and why is it important?

The trial protocol is the plan for a clinical trial. It explains the treatment, tests, visits and how side-effects will be monitored. It helps the study team conduct the trial safely and consistently. It also describes who can participate, how participants will be monitored, and how researchers will evaluate whether the treatment is safe and effective.