

# What is a Clinical Research Study?

Choosing to participate in a clinical research study is an important decision. This brochure is designed to provide answers to frequently asked questions about clinical research.

Read it as many times as you need to. It is important that you read the consent form, too.



Scleroderma: Cyclophosphamide Or Transplantation

Patient Brochure

SCOT is sponsored by the National Institutes of Health (NIH) through its Division of Allergy, Immunology and Transplantation (DAIT) in the National Institute of Allergy and Infectious Diseases (NIAID).



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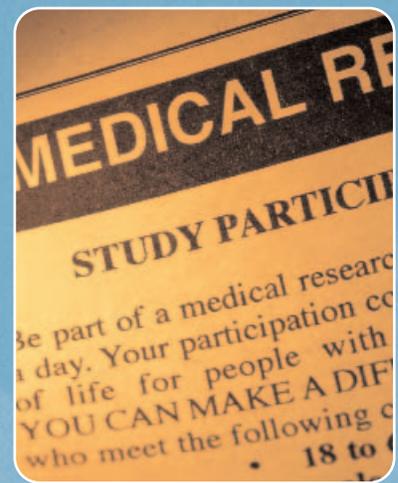
A clinical research study is a scientific investigation in which people can help doctors find ways to treat disease and improve healthcare. These studies are a vital part of the process by which new treatments are approved for use. Before drugs and devices can be used by the general public, they have to be tested in smaller groups of people to make sure they are both safe and effective.

## Why would you agree to take part in a clinical research study?

Participating in a clinical research study gives you a chance to play an active part in your own healthcare and help others, too. It might help you directly by giving you access to an investigational drug or procedure you would not otherwise be able to receive at that time. Or, you may agree to participate just so you can contribute to medical research, even though you may not receive any direct benefit from the research.

## Who can participate?

Before you can take part in a clinical research study, you must first be “screened” to be sure you are right for the study and that the study is right for you. All studies will follow established guidelines describing who should be included in or excluded from the study. Sometimes a study will be looking for people with a particular medical condition, and sometimes a study will want healthy volunteers.



### What is a protocol?

A protocol is the “blueprint” for the study. It explains in great detail exactly what is being studied and all of the procedures to be followed.

The consent form is a document that describes your rights as a study participant and includes details about the study, such as its purpose, time involved, required testing, and important contact information. Risks and potential benefits are also explained.

Signing the form means you understand what is involved and that you are voluntarily taking part in the study. Even after signing the consent form, you may withdraw from the study at any point. You will receive a copy of the consent form. Keep this copy to refer to it throughout the study.

## What happens during a clinical research study?

First, the study will be thoroughly explained to you and you will have an opportunity to ask all of your questions. When you have a good understanding of the study and are interested in taking part, you will be asked to sign a consent form.

The consent process involves more than signing a document; it is an ongoing, interactive process that will continue throughout the study. The research staff will review and discuss all information presented in the consent form to make sure you fully understand and can make an informed decision about participating in the study. You will have time to consider your decision, ask questions, and discuss the study with family and friends.

By signing the consent form, you are not releasing any of your rights as a research participant.

The rest of the study process varies, depending on the purpose of the study. Some studies are observational—no study drug or procedure is being tested, but participants' health habits are observed and/or participants complete questionnaires about an important health issue. Other studies are investigational—study drugs or procedures are tested by participants before they are approved for use in the general population.

## What is randomization?

In investigational studies, after you sign the consent form, you will be randomized to a study group. Randomization means that your study assignment will be done by chance, as in a flip of a coin. Neither you nor your doctor will be able to decide what group you will be assigned to. For example, when investigating a new drug, a study may have two similar groups of participants: one group may get the study drug, while the other group may get a similar looking substance that does not contain medication.

Randomization is done to produce study groups that are as similar as possible and to remove bias. Eliminating bias in clinical research is done so that one outcome is not favored over another. In randomized clinical studies, doctors can be confident that the research results reflect the effect of the study drug or procedure, not the differences in the makeup of the study groups.

## What if you change your mind and no longer want to participate?

You may withdraw your consent at any time, for any reason, and drop out of the study. If you decide to withdraw, inform the study staff of your decision. You may need to undergo some final tests and procedures.

## What are the risks?

The risks involved in taking part are that the study procedure or study drug might not work or may cause side effects. Side effects are problems that may be associated with the study drug or study procedure. These effects can range from unpleasant symptoms to life-threatening reactions. You will be told if there are any known side effects before you agree to take part.

## How will your safety be protected?

The same ethical and legal requirements that govern the practice of medicine apply to clinical studies. Clinical research studies are very closely controlled and have built-in safeguards. Investigators are required by law to follow very specific procedures. You will be monitored frequently to check your health and progress.

All clinical studies have to be preapproved by an Institutional Review Board (IRB) before participants can be enrolled. The IRB is an independent group made up of both medical experts and someone from the community. It is the IRB's job to make sure the study is as safe as possible and worth the risks involved. In addition to reviewing and approving the study, the IRB will also periodically review its progress.

The SCOT study has had a detailed review by the Food and Drug Administration (FDA), National Institutes of Health (NIH), and a Data Safety and Monitoring Board (DSMB) to ensure patient safety.



### What is an IRB?

An IRB is a committee of doctors, statisticians, and community advocates responsible for reviewing human clinical studies. They help ensure that studies are ethical and adequately inform and protect participants. Approval by an IRB is required before a study can begin to enroll participants.





## What about contact with your regular doctor?

Your relationship with your regular doctor will not be affected by the clinical research study. Be sure to inform your doctor that you are taking part in a research study. This is important so your doctor can check that any treatments or medications he or she may prescribe will not have a bad reaction with the study-related drugs or procedures.

## Does it cost anything to participate in a clinical research study?

In most clinical studies, the cost of services and laboratory tests that are considered part of the study will be covered by the study. This does not include routine medical care. Costs associated with routine medical care will be billed to you and your insurance company.

# What should you consider before you participate in a clinical research study?

- What is the study's purpose?
- Who will be in it?
- What tests and procedures are involved?
- Why do the investigators think the drug or procedure being tested may be effective? Has it been tested before?
- Am I willing to be assigned (randomized) to either study group? Will I be satisfied with this decision while I am participating in this study?
- Do I have a strong preference to be assigned (randomized) to one study group over another? If you do, a randomized study may not be right for you.
- How do the possible risks and benefits compare with the treatment I am getting now?
- How might this study affect my daily life?
- How long will the study last?
- Will hospitalization be required?
- Who will pay for the study drug and procedures?
- Will I or my insurance carrier be billed for medical expenses relating to, or arising from, this study?
- Will I be reimbursed for any expenses?
- What type of long-term care will I receive?
- How will I know if the study drug or procedure is working? Will the results of the study be provided to me?
- Who will be in charge of my care during the study?
- Who should I contact if I have any questions?

Be sure to ask all of the questions you have before you agree to participate. You might also want to talk with your doctor, family, and friends. Deciding to take part in a clinical research study is a decision that might have great impact not only on you, but on many people in the future.



