Fact Sheet

National Scleroderma Foundation

Clinical Trials 101

WHAT IS A CLINICAL TRIAL?

A clinical trial is a research study involving the active participation of volunteers to test the safety and effectiveness of new medical treatments or new ways of using an existing treatment. The term 'study' is used very loosely to refer to an analysis. The medication in a trial begins in a research laboratory (called pre-clinical trials) and may take up to ten years of study in test tubes and lab mice before it reaches the point of testing its safety and effectiveness in humans. Only the treatments with the most promising lab results are moved into clinical trials. For many patients, a clinical trial is an opportunity to gain access to a medication as much as five or six years before it becomes commercially available. The clinical trials are designed to answer five basic questions about what they're investigating:

1. Is the medication safe?

2. Is it effective?

3. What side effects does it produce?

4. What dosage is most effective?

5. Is it more effective than or equally as effective as other treatments on the market?

Clinical trials are conducted according to a plan called a protocol. The protocol describes what type of patients may enter the study, schedules of tests and procedures, drugs, dosages, the length of the trial as well as the outcomes that will be measured. Each volunteer in the study must agree to the rules set out by the protocol.

THE CLINICAL TRIAL PHASES

BClinical trials are commonly classified into four phases. Each phase of the drug approval process is treated as a separate clinical trial. The drug development process will normally proceed through all four phases over many years. If the drug successfully passes through Phase I, II, and III, it will usually be approved for use in the general population. Phase IV is referred to as the post marketing phase.

There are four phases to a clinical trial, each with a different set of objectives and requirements.

PHASE I

The drug is tested for the first time in a small group (20-100) of healthy volunteers. It typically lasts up to one year and its primary purpose is to evaluate the drug's safety, determine a safe dosage range, and to identify side effects. About 70% of investigational drugs pass this phase.

PHASE II

The treatment is now given to a larger group of people (100-300) who actually have the disease or condition the medication is being tested for. It usually lasts from one to three years and is used to further evaluate its safety as well as its effectiveness. Only about one-third of the drugs that begin testing successfully complete this phase.

PHASE III

This stage provides hard, statistical facts about a drug. It is given to a much larger group of people (1000-3000) and lasts anywhere from two to four years. It is used to confirm the medication's effectiveness, monitor side effects, and compare it to other commonly used treatments. At this stage, researchers also compare the drug's safety and effectiveness in different subsets of patients – men vs. women, elderly vs. young etc. They also test different dosage levels to determine how much of the drug is needed to achieve the

best possible effects with the least amount of side effects. About 70-90% of drugs successfully pass this phase. Once this phase is complete, a pharmaceutical company can request FDA approval to begin marketing the drug.

Phase IV

This is referred to as the post-marketing phase. It involves several thousand patients and often lasts two to ten years. After a company has received FDA approval to market a drug, they will conduct this phase to uncover additional information about a new treatment such as:

- What is the long-term safety and effectiveness of a drug?
- What impact does it have on improving a patient's day-to-day lives?
- When do physicians prescribe this treatment versus other similar treatments?
- How does this treatment compare to other similar treatment?

The results are used to determine the drug's risks, benefits and optimal use.

The gold standard for clinical trials is called a randomized, double-blind, placebo-controlled trial. Here's what that means:

- Randomized means that the researchers assign the people involved in the trial, at random, to either the experimental group or the placebo group. This is done to make sure the study outcome isn't biased or influenced by pre-existing differences among patients assigned to study groups.
- Double-blind means that neither the patients or the researchers know who is getting the actual medication or who is getting the placebo.
- A placebo refers to an inactive medication that has no treatment value.
- A controlled trial simply compares the effects of a treatment on two or more groups of people. The experimental group gets the active treatment being studied while the control group may get a different treatment or a placebo.

FACTORS TO CONSIDER BEFORE PARTICIPATING

Choosing to participate in a clinical trial is a very important personal decision. In fact, only about 2% of the American population get involved in clinical research each year. For some, getting involved in a clinical trial offers them hope and is a way to access investigational drugs that may be their only chance of survival. For others, their reason for participation may be as simple as not having medical insurance and needing help covering medical costs.

All clinical trials will carry some degree of risk. Let's take a look at some of the basic risks you need to be aware of:

Risks

- The treatment may not be effective for you (ie: you may receive the placebo). You may be trading a known treatment for an unknown treatment which may not have any benefits to your health.
- There may be unpleasant or very serious side effects to the treatment.
- The clinical trial protocol may require a big time commitment on your part. This may include multiple trips to the study site, hospitals stays, medical procedures and complex dosage requirements. The trials can last anywhere from one month up to several years.

However, there are many benefits too:

Benefits

- Participating in a clinical trial allows you to play an active role in your medical care.
- You can gain access to new treatments long before they are commercially available.
- A clinical trial may give you access to expert medical care at leading institutions you wouldn't normally have access to.
- Your medical care is free during the trial.
- From an altruistic viewpoint, you'll be contributing to medical research and helping to advance science.

QUESTIONS TO BE ANSWERED BEFORE PARTICIPATING

1. What is the main purpose of the study?
2. Who is sponsoring the study?
3. Who is participating in the study?
4. What are the eligibility requirements?
5. How long will the trial last?
6. What do researchers already know about the drug and what other studies have been done?
7. Where is the study being conducted? Multiple sites?
8. Does the study involve a placebo and if so, what are my chances of getting a placebo?
9. What are the side effects of the drug?
10. How do the long-term risks and side effects compare to my current treatment?
11. What kinds of tests and procedures are involved?
12. Will I be reimbursed for any out-of-pocket expenses?
13. Will hospitalization be required?
14. What type of long-term follow-up is involved in the study?
15. How will I know if the treatment is working?
16. What happens if I quit the study?
17. What if I get the placebo but need the actual drug?
18. Will the results of the trial be provided to me?
19. Who is in charge of my safety?
20. How much of my time will this take?
21. How will this affect my daily life?
22. What are the percentages that the drug will be effective?

FINDING CLINICAL TRIALS THAT MAY BE RIGHT FOR YOU

If you're interested in joining a clinical trial or want to find out more information, try the following:

- Talk with your doctor first about what the best trial would be for your particular issues.
- The National Scleroderma Foundation has partnered with Carebox to provide patients, caregivers, and healthcare professionals with an easy way to search and match to scleroderma clinical trials. The free, confidential, and easy-to-use service allows patients and family members to search for studies. Learn more at - https://scleroderma.org/find-a-clinical-trial/
- Go to Centerwatch.com. This is an information source for the clinical trials industry. You can search for clinical trials by medical condition or therapeutic area.
- Look up Clinicaltrials.gov. This website is sponsored by the federal government and provides a listing of clinical trials nationwide.

###

Please note that this brochure is provided for educational purposes only. It is not intended to substitute for informed medical advice.

The National Scleroderma Foundation wishes to thank **Karen Gottesman,** for her input on this brochure.