

WHAT IS A CLINICAL TRIAL?

This resource has been created for those in the NF community seeking information about clinical trials and the factors to be considered before making decisions about participating in a clinical trial.

CLINICAL TRIALS, WHY ME?

Participating in clinical trials is a decision only you can make with guidance from your trusted healthcare provider. A few reasons to consider participating:

- 1. Personal benefits.** Clinical trials are on the cutting edge of treatment and can, in some cases, result in improved personal health, function, and quality of life.
- 2. Community benefits.** Clinical trials can provide the NF community with more information about how NF affects people differently as well as why some treatments are effective and why other treatments do not work.
- 3. Better Research.** Clinical trials, even when they do not lead to a new treatment, guide the direction and focus of future research.

DEFINING THE BASICS

Purpose: The aim or goal of the research study; the reason the study is being conducted.

Clinical Trial/Interventional Study: A patient is given a potential treatment and the response is assessed. Example: patients with NF1 and a plexiform are given Cabozantinib and changes in tumor size are measured.

Observational Study: No treatments or interventions are involved; patients are only observed. Examples: the genetic evaluation of NF1 patients with scoliosis, participants give a sample of their DNA for evaluation by researchers; patients are surveyed about their experiences with NF and effects on their relationships.

DEFINING THE PLAYERS

Principal Investigator (PI): The person responsible for the scientific direction of the research study.

Sponsor: The organization or person responsible for overseeing the study and analyzing study data.

Study Coordinator: Primary point of contact for research study, works directly with study participants (also sometimes called "subjects") and helps with data collection.

Participant/Subject/Patient: Person consenting to participate in research study.

Collaborators: An organization other than the Sponsor that provides support which may include funding, design, implementation, data analysis, or reporting.

DEFINING THE PROCESS

Protocol: Written description of the study plan. A protocol describes the participants, the schedule of tests, procedures, medications and dosages, and length of the study. All protocols must be approved by an Institutional Review Board, which is designed to protect the safety of the participants.

Inclusion/Exclusion Criteria: Required factors which are necessary for someone to participate in a research study, or which prevent them from participating. These can include age, gender, medical condition, and other variables for which a study may want to control or assess. Not everyone who applies for a clinical trial will be accepted based on the eligibility criteria and/or the number of participants needed to collect the information.



There are five phases for clinical trials. Each phase aims to answer a different question.

DEFINING THE CONSENT

Consent/Assent: *Consent* is how you give your permission for researchers to include you in a research study. It is important that you understand the consent documents you are asked to sign. For minors a parent/guardian will sign a consent. *Assent* is used to express willingness to participate in research for individuals too young to give legal consent but old enough to understand the proposed research study. It may be offered in addition to parental consent.

Risk/Benefits: It is important to understand the risks and benefits a study poses for you as an individual as well as for the community. This is an important part of consent! Before signing a consent document, be sure to ask questions and make sure you understand the purpose and method of the clinical trial.

FINDING A CLINICAL TRIAL

- 1. Talk to your NF doctor!** Access to clinical trials is one reason to be seen in a CTF-affiliated NF clinic. Your NF provider will have the latest information on clinical trials and will be able to help determine if you are eligible.
- 2. Join the NF Registry!** If you give your permission, you will receive emails about research studies that may apply to you. Whether or not you join the registry, there is a current list of open clinical trials updated regularly on the registry website: nregistry.org.
- 3. Go to Clinical Trials website.** There is a Clinical Trials website that you can search for NF relevant clinical trials: clinicaltrials.gov. This website includes contact information for researchers conducting trials, but we always recommend reviewing the information with your NF doctor before considering any type of research study.

SOCIAL-EMOTIONAL CONSIDERATIONS

Currently, there is no cure for the three types of Neurofibromatosis. We have come a long way in our understanding of these three conditions, but there is still much to learn. This is what makes clinical trials so important. Knowledge is power and research fuels knowledge! Learning that you, your child, or your loved one has been accepted into a clinical trial can be exciting, but it is important to understand that a trial makes no promises. A drug, for example, being researched for its effectiveness in shrinking tumors is exciting. Researchers cannot, however, guarantee every clinical trial will result in an effective treatment for each patient.

FINANCIAL CONSIDERATIONS

It is important to ask who will pay for the treatment and if your insurance company will pay for any extra costs of participating in the trial. Health insurance typically covers costs related to tests and treatments that would be done for standard care of treatment. Experimental drugs are usually provided for free, but other costs such as travel and lodging also need to be considered.

FINAL REMINDERS

Participation in a clinical trial may be available for you, but there are many factors to consider. It is always a good idea to bring a trusted friend or family member with you to doctor visits to make sure that you have heard and understood everything that the team is explaining, especially when considering a clinical trial. Find out what the study involves and what other options are available. Make sure you ask questions, get answers and understand all risks and benefits. Use a professional medical interpreter if English is not your first language.