



## WHAT IS A CLINICAL TRIAL?

Clinical trials can be an important option for some individuals living with neurofibromatosis (NF). Clinical trials are research studies designed to evaluate new treatments, new uses for existing treatments, or new approaches to care. Participation is always voluntary. Deciding whether to join a clinical trial is a personal decision that should be made in consultation with your healthcare provider. You do not have to make this decision alone.

## WHAT'S INVOLVED?

A clinical trial is a carefully designed research study in which volunteers receive investigational treatments under the supervision of physicians and research professionals. These treatments may include medications, medical devices, surgical procedures, behavioural therapies, or supportive interventions. Clinical trials help researchers determine whether a treatment is safe, effective, and appropriate for wider use. Almost every approved medical treatment has gone through clinical trials.

## THE PHASES OF CLINICAL TRIALS



The first time a new treatment is tested in people. Researchers evaluate safety, dosage, and side effects. Usually involves a small number of participants.



Continues safety evaluation and examines how well the treatment works.



Compares the new treatment to current standard therapy. Often involves many participants across multiple centres and may last several years.



Occurs after a treatment is approved and on the market. Researchers gather long-term safety and effectiveness data.



## WHO IS ELIGIBLE?

Having NF does not automatically make someone eligible for a clinical trial. Each study has specific inclusion and exclusion criteria designed to protect participant safety and ensure accurate study results. If you are interested in participating, speak with your physician first.

## INFORMED DECISION-MAKING

Before enrolling, understand the purpose of the study, potential risks and benefits, time commitments, sponsorship, and what happens if you withdraw. You should never feel pressured to participate. Take your time and ask questions.

## QUESTIONS TO ASK BEFORE PARTICIPATING

- What is the purpose of the study?
- Why might this treatment work for NF?
- What are the risks and potential benefits?
- How does it compare to my current treatment?
- Who is sponsoring the trial?
- Will travel or other expenses be reimbursed?
- What tests and follow-up visits are required?
- What happens after the trial ends?
- How will my information be stored and protected?

## TRAVEL, TIME AND FINANCIAL CONSIDERATIONS

Participation may require travel within or outside British Columbia, time away from work or school, and multiple appointments over months or years. Ask whether travel, accommodation, or meal expenses are reimbursed before enrolling.

## YOUR RIGHTS AS A PARTICIPANT

Participation is voluntary. You may withdraw at any time. You must receive clear information about risks and benefits, and your personal health information must be protected.

## CLINICAL TRIALS INVOLVING CHILDREN

When children participate, parents or guardians provide consent. Whenever possible, children should be part of the conversation and supported to express whether they feel comfortable participating. It may be helpful to consult a physician unaffiliated with the trial before making a decision.

## CLINICAL TRIALS AND DRUG APPROVAL IN CANADA

After clinical trials are completed, Health Canada reviews the evidence to determine safety and effectiveness. The Canada Drug Agency evaluates cost-effectiveness, and provincial governments decide on public funding. This process can take time.

## WHERE TO FIND ACTIVE CLINICAL TRIALS

Search for recruiting clinical trials in Canada at:

*Search using “neurofibromatosis” and filter by location.*

## FINAL THOUGHTS

Clinical trials can bring hope and uncertainty. There is no single correct decision. If you are considering participation, speak with your healthcare provider and take the time you need to make an informed choice.