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CLINICAL TRIALS
INFORMATION KIT

www.cysticfibrosis.ca

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WHAT IS A CLINICAL TRIAL?

Clinical trials test potential new drugs, treatments and devices to ensure they are safe, to assess how well they work, and to determine whether they offer benefits over currently used drugs, treatments or therapies. Drugs and devices are first tested in research laboratories where experiments are done in animals and human cells.

Research is conducted to:

- ✓ Improve drug-like properties
- ✓ Test benefits, safety and best route of drug or therapy
- ✓ Test interactions with other drugs
- ✓ Determine dosage and potential adverse side effects
- ✓ Determine effectiveness compared to similar drugs or therapies

If the research is successful and the drug or therapy is deemed safe, it will go into the first phase of a clinical trial.

PHASE I

Phase I (also known as first in human trials) assesses the drug or therapy for human safety and it is tested on a small sample of healthy people first to determine the body's reaction and safe dosage ranges. It may then also be tested in the target patient population (Phase Ib). This phase lasts days to weeks and has a success rate of approximately 76% for rare diseases.

PHASE II

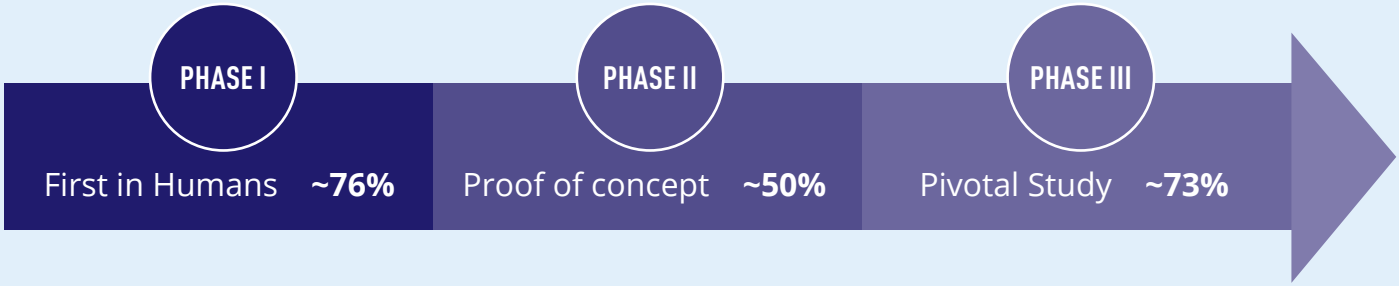
Phase II continues to evaluate safety and determines the efficacy of the drug or therapy. In this phase, the drug or therapy is tested on a larger group of patients with the disease that the drug or therapy has the potential to help. It lasts weeks to months. For rare diseases, the success rate of this phase is approximately 50%.

PHASE III

Phase III validates the effectiveness, the benefits and the possible side effects of the drug or therapy on an even larger group of patients with the disease. This phase can last several years and has a success rate of approximately 73% for rare diseases[i]. Once a trial is complete the participant may or may not continue on the medication, depending on the trial. It is important to speak with the research team prior to starting a trial to ensure you know what will happen before, during and after a trial. Information regarding accessing the treatment post-trial, as well as coverage information, can also be made available by the trial research team.

Once a clinical trial is complete, researchers will determine if the results have medical importance and if the benefits outweigh the possible risks. For the drug to be available to the public, an application must be submitted for market approval to Health Canada. The application presents the drug's safety, quality and effectiveness which are reviewed by Health Canada's experts. If it is deemed that the benefits of the drug outweigh the risks, with the lowest possibility of adverse effects, the drug will be approved for sale in Canada. Once approved, the drug will get a Notice of Compliance (NOC) and a Drug Identification Number (DIN) and may become available through private insurance plans shortly thereafter. Provincial and federal insurance plans take much longer - sometimes years - to cover new drugs.

SUCCESS RATES IN RARE / ORPHAN DISEASES



76% of trials are successful in Phase I trials. Of these 76%, only 50% are successful in Phase II, thus only 38% of trials reach a Phase III trial stage. Of this 38% that reach Phase III, 73% of these are successful. This means that for each new potential treatment initiated into a clinical trial, only 25% are successful from phase I to completing phase III[ii].

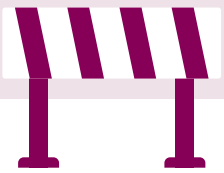
BENEFITS, BARRIERS AND RISKS

BENEFITS

- Taking an active role in your healthcare
- Understanding your diagnosis better
- Helping others living with the same condition
- Gaining access to new treatments not yet available to the public
- Receiving a treatment that may work better for you than your current treatments
- Receiving close and extra follow up care
- Many studies have shown that participants involved in clinical trials have better overall health outcomes, compared to those who are not involved in trials[iii]

BARRIERS

- Being required to travel to the study site
- Time involved in attending study visits (especially Phase I and II) and length of trials
- Potential monetary value of missing work
- Potential need for childcare while participating in a trial
- Potential of missing school to participate in a trial



RISKS

- Experiencing side effects or adverse reactions to medications or treatments
- Receiving a treatment that does not work for you
- Not being part of the treatment group (taking placebo)

CONSENT

After the lead of the trial research team has discussed a potential clinical trial and you are interested in learning more, the team will give you an informed consent form which discusses the trial in finer detail. The informed consent will outline the trial background information, who the sponsor is, how many participants the study will have, the length of the study, what will happen during the study, potential benefits and risks, participant responsibilities, the process of withdrawing from a trial, confidentiality, costs and compensations (if any) and any other details regarding the specific trial.

If any changes are made to the clinical trial, it is the participant's right to be notified and a new consent form should be given to the participant to sign. After all new information and changes are discussed a participant will have the choice to continue in the trial; should they want to continue the new consent form must be signed.

CHILDREN, CLINICAL TRIALS AND CONSENT

Before clinical trials are offered to children, safety data must be collected on adults and adolescents. If data shows safety and efficacy of the drug or therapy, it can be tested on a younger population. Children are very closely monitored during clinical trials and all safety precautions are followed, just as in adult clinical trials.

Children participating in clinical trials will need to have a consent form signed by their parent or legal guardian if they are under the province's or territories' age of majority. Children under the age of majority may still provide assent to participate or not participate in a trial if they have the capacity to understand the trial and its details. A healthcare professional must assess a child's capacity and determine if the child understands the purpose of the trial, the potential risks and benefits and participant responsibilities.[iv] It is important to involve children in the decision-making process to make them feel comfortable and to engaged them in their own healthcare.

Not every drug that is suitable for adults will be suitable for children; dosages, efficacy and safety may vary between adults and children. Drugs may need to be created into age appropriate formats, such as making the drug available in syrup form opposed to a pill which could be difficult for a child to ingest.

FREQUENTLY ASKED QUESTIONS

1. Who can participate?

Before joining a clinical trial, a participant must qualify for the study. Inclusion and exclusion criteria are used by clinicians to determine who is eligible to join a specific trial. These criteria ensure the safety of participants and ensure the results from the study will be accurate and useful.

At the start of a trial, an informed consent form must be signed so that the research team may collect your personal health information to assess inclusion and exclusion criteria. Once consent is given the researchers will provide the details of the study and answer any questions you may have. Researcher will look over your personal health information to ensure you fit the inclusion criteria of the specific trial. The age of participation will vary depending on the trial.

2. How safe are clinical trials?

The ethical and legal codes that govern medical practice in Canada also apply to clinical trials. Ongoing evaluation by a research ethics board and safety monitoring committee help to protect people who choose to participate in trials.

3. What infection prevention and control measures are taken when conducting clinical trials?

Infection prevention measures are taken seriously in clinical trials, and just as at your cystic fibrosis clinic, your research team, who are healthcare professionals, will have the same infection control practices. Your research coordinator and physician will gown and glove when they meet you. For any lab work, the same infection control practices are followed as when you attend your cystic fibrosis clinic. For further information, please refer to Cystic Fibrosis Canada's website for guidelines, tips and videos on infection prevention and control: www.cysticfibrosis.ca/about-cf/living-with-cystic-fibrosis/infection-prevention-and-control.

4. Can I leave a clinical trial once treatment has started?

Yes. You can withdraw from a clinical trial at any time, for any reason, even if you have signed a consent form. Leaving a clinical trial will not affect your current or future healthcare. If you decide to withdraw from a trial you should inform the research team; they may ask you to come in for a final visit. You should also inform your doctor and care team.

5. Will leaving a trial affect my opportunity of participating in future clinical trials?

No. Leaving a trial once treatment has started will not affect your opportunity to participate in future trials. However, the research team will ask you to read the consent form thoroughly and consider what is involved in participating in a trial before consenting to it to minimize participant drop out.

FREQUENTLY ASKED QUESTIONS CONT...

6. Who sponsors research for clinical trials?

Clinical trials are sponsored by industries, such as pharmaceutical companies or researchers within a cystic fibrosis clinic (also known as Investigator Sponsored).

7. How can I learn more about cystic fibrosis clinical trials in my area?

Visit the clinical trial finder section on our website, www.cysticfibrosis.ca/clinicaltrials, to view clinical trials that are currently recruiting in Canada. If you are interested in learning more about participating in a clinical trial, please discuss your eligibility with your cystic fibrosis healthcare team.

8. How does Cystic Fibrosis Canada support clinical trials?

In addition to its regular research programs and its financial assistance for clinical and transplant care, Cystic Fibrosis Canada also maintains the Canadian Cystic Fibrosis Registry (CCFR) and the Cystic Fibrosis Canada Accelerating Clinical Trials Network (CF CanACT). The establishment of CF CanACT will facilitate the development of new treatments for cystic fibrosis and increase capacity and enhance participation of people with cystic fibrosis in clinical trials.

9. What are the biggest challenges to finding a cure for cystic fibrosis?

In addition to raising funds for research, recruiting people to participate in clinical trials is one of the biggest challenges. Many potential treatments are having laboratory success, but must be thoroughly tested in clinical trials to prove they are effective and safe. Your participation can help bring effective treatments to the cystic fibrosis community sooner.

QUESTIONS TO ASK YOURSELF

Consider asking the following questions when considering participating in a clinical trial with your cystic fibrosis care team:

- What is the purpose of the study?
- How will the study affect my daily life?
- Who will be in charge of my care if I participate in the trial?
- Why do researchers think the experimental treatment(s) might work?
- How do the possible risks, side effects and benefits compare with my current treatment?
- Will hospitalization or additional clinic visits be required?
- Will I be reimbursed for certain expenses associated with the trial?
- Will the results of the trial be provided to me?
- Once the trial is complete will I continue on the same treatment if it worked for me?
- Who is sponsoring the trial?
- What will be my role in the study?
- How will the study benefit me, and/or others?
- What kinds of tests and experimental treatments are involved?
- How long will the trial last?
- Who will pay for my participation in the study?
- How will I know the experimental treatment is working?
- What types of long-term follow-up care will be required?
- How will my privacy and confidentially be protected?

CYSTIC FIBROSIS CANADA ACCELERATING CLINICAL TRIALS NETWORK

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In keeping with its vision of a world without cystic fibrosis, Cystic Fibrosis Canada has established a Canadian Clinical Trials Network called Cystic Fibrosis Canada Accelerating Clinical Trials (CF CanACT). World-class clinical trials are an integral part of the process that brings new therapeutics and better care to Canadians who are living with cystic fibrosis. The establishment of CF CanACT will facilitate the development of these new treatments for cystic fibrosis and increase capacity and enhance participation of people with cystic fibrosis in clinical trials.

As of 2018 the CF CanACT network consists of six sites across Canada (one in Montréal, two in Toronto, one in Calgary and two in Vancouver). Over time, the network will expand to include more sites. In the meantime, anyone with cystic fibrosis living in Canada is eligible to be referred to one of these sites to participate in a clinical trial, and can participate if they meet the inclusion criteria of the specific trial.

Each of the investigators and research coordinators from the six sites, along with an adult patient representative and a CF parent, collaborate together to review research protocols, standardize outcome measures between sites, and ensure that the clinical trials are feasible to perform and relevant to their patients.

HAVE QUESTIONS RELATED TO CLINICAL TRIALS OR CF CanACT?

Email us for more information at clinicaltrials@cysticfibrosis.ca.

GLOSSARY

Assent:	When a child agrees to take part in a clinical trial.
Controlled studies:	Trials where one group of participants is given the experimental treatment, while another is given a standard treatment or a placebo. Some controlled trials will test all three – experimental treatment, standard treatment, and placebo.
Dissent:	When a child does not agree to take part in a clinical trial.
Double Blind:	When neither the research team nor the patient knows which participants are taking the drug, and which participants are taking the placebo. This helps to eliminate potential bias in the results.
Efficacy:	Capacity for producing a desired result or effect.
Exclusion criteria:	Characteristics that disqualify a person from participating in a clinical trial.
Inclusion criteria:	Characteristics that a person must have to be able to participate in a clinical trial.
Informed Consent:	When a participant or parent (substitute decision maker) is given detailed information regarding a clinical trial and makes a decision whether to participate or not. A participant or parent will sign a consent form if they agree and understand the purpose, benefits and risks of the particular trial.
Orphan (rare) diseases:	An illness that affects a small percentage of the population.
Phase:	The different stages involved in developing new drugs or therapies.
Placebo:	A pill, an inhaled solution or a treatment that is designed to have no effect on the person who ingests it. In clinical trials, individuals taking experimental drugs are often compared with participants taking placebo drugs, to learn how well a drug really works. Often a placebo in pill form is a sugar tablet.
Protocol:	A carefully structured plan developed before the trial begins. It outlines the goals of the research, who can participate, a schedule of tests/procedures, and the length of the trial.
Randomized Trial:	Participants of a trial are assigned by chance to one of the treatment groups. Each participant is equally likely to receive the study drug or the placebo. After randomization the two (or more) study groups are followed in exactly the same way. This design helps ensure results are not biased.
Sample Size:	The number of participants in a clinical trial.[v]

FOOTNOTES

[i] ClinicalTrials.gov, www.clinicaltrials.gov

[ii] Thomas, D. W., Burns, J., Audette, J., Carrol, A., Dow-Hygelund, C., & Hay, M. (2016). *Clinical development success rates 2006-2015. San Diego: Biomedtracker/Washington, DC: BIO/Bend: Amplion. San Diego: Biomedtracker/*

[iii] M. K. Krzyzanowska, R. Kaplan, R. Sullivan; How may clinical research improve healthcare outcomes?, *Annals of Oncology*, Volume 22, Issue Suppl_7, 1 November 2011, Pages vii10–vii15, https://doi.org/10.1093/annonc/mdr420

[iv] The Canadian Medical Protective Association, https://www.cmpa-acpm.ca/en/advice-publications/browse-articles/2014/can-a-child-provide-consent

[v] U.S. National Library of Medicine, https://www.nlm.nih.gov/