Understanding

Clinical Trials from the

Patient Perspective

with Dr. Aneal Khan





This education session was made possible

through the provision of an

unrestricted education grant

from



and





Understanding Clinical Trials -What we'll cover today

- Why clinical trials are important
- Understanding what a clinical trial is
- Exploring the benefits and risks of participating in a clinical trial
- Questions to ask in order to make an informed decision
- Myths vs reality





Why clinical trials are important

Safety and efficacy

of any new treatment must be tested before it can be reviewed, approved for use, prescribed and taken by patients. Data about whether a treatment works and whether it is safe to use comes from

clinical trials.





What is a clinical trial?

- a controlled study that collects data on the safety and efficacy of a new drug or device
- conducted before the product is authorized for sale in Canada
- Pre-ceded by extensive laboratory research in non-living and living systems
- once approved for humans, testing is generally conducted in four phases with each phase being considered a separate trial
- data must be submitted for review and be approved by regulatory bodies before continuing to the next phase of testing for approval (e.g. Health Canada)



Consider the following:

Data Set 1

- Sally has a mitochondrial disease
- she started using CoEnzyme Q10 and was feeling better
- the dose is not known
- side effects were not recorded
- blood tests and response were not recorded

Is Sally's response representative of what most people would experience?

Data Set 2

- 300 people were randomized to receive 300 mg CoEnzyme Q10 vs. placebo
- a 6 minute walk test was used to measure their response
- a fatigue scale was recorded on every person
- all complications were recorded
- both the patient and doctor were blinded



Levels of Evidence

Level	Type of Trial
1	Randomized Controlled Trial (RCT)
2	Cohort Study2 groups exposed to different things
3	 Case Control Study cases get exposed but controls do not
4	Case Seriesa group of patients with some similarities
5	Expert Opinion

In rare diseases...

the number of patients that can participate is small.

In some cases,

natural history studies or disease registries

are used to compare the response of a new drug.



How patient involvement benefits drug development

- patients data is critical to understanding whether a drug works
- patients help others, such as researchers, clinicians, regulators, payers (insurance), understand their real-life experiences
- patient input helps create better studies
 - purpose
 - protocols
 - patient report outcomes (PROs) and real world evidence (RWE)
 - quality of life (QOL) measures



Why people want to participate in clinical trials

- help/offer hope to others by advancing research
- potential to access to new treatment that may improve current health status and quality of life
- potential access to medicine at no cost
- potential for better care



Why people may not want to participate in clinical trials

- fear about the safety of a new drug
- fear of receiving placebo (no active ingredients) or drug already on the market
- fear that drug may worsen current disease / symptoms
- fear that drug may cause unwanted short and/or long-term side-effects
- fear that new drugs may impact a child differently than an adult
- concerns/stress about potential travel, tests, hospital stays, expenses and time
- too sick



Worldwide Clinical Trials

Welcome to the world of clinical trials.

Find, learn and connect with a study relevant to you

51,803 Active trials

283,645 Study sites

967

Medical conditions

1,172 Treatments

Find a trial



https://www.centerwatch.com/clinical-trials/





Realities of Rare Diseases

- those with rare diseases are often misdiagnosed, undergo unnecessary surgeries, experience social isolation, financial hardship, lack of treatment options, and early death
- 300 million people live with rare diseases
- 1 in 12 Canadians (2/3 of them children), are affected by rare diseases
- only 5% of diseases have therapies
- in Canada, only 60% of treatments for rare disorders make it to our borders and some get approved up to 6 years after approval by US and Europe
- the overwhelming majority of new treatments that are approved all come from data in clinical trials



Recruiting for clinical trials for rare diseases is particularly challenging

- few people experiencing the illness so few people to recruit to join trial
- people are often spread out across large geographic areas
- finding enough people who fit the inclusion/exclusion criteria
- few participants may not truly represent larger patient population
- health teams have few opportunities to see/interact with patients so they have a poor understanding of disease and its progression
- how do patients know there is a trial going on?
- difficult to generate convincing data on safety and efficacy



Patients may not benefit from potential new therapies because clinical studies cannot be completed



Who's Who

Participant | the individual who has consented to participate

could be you or someone you care for (child, parent, spouse, sibling, other)

Investigators | the people who carry out clinical trials

often physicians but could be researchers, nurses, other health professionals

Study Coordinator | works with investigators, research team and participants

Sponsors | organizations, companies or people that test drugs including

drug companies or researchers from a hospital, university or research organization



Clinical Trials in Canada | Protecting Participants

Before recruiting patients, clinical trials in Canada must:

- have stringent protocols in place to protect participants, abide by the requirements of the Declaration of Helsinki, the team members have GCP training, maintain up-to-date records, and have regular inspections
- be approved by a Research Ethics Board
- protect the health of participants
- be well-designed and conducted by trained professionals
- be monitored and side effects reported
- data generated is scientifically useful and data from the trial site is trusted



Clinical Trials in Canada | Protecting Participants

Sponsors must submit application to Health Canada to review to make sure:

- best interests of the people taking part in the trial have been considered
- any risks in using the drug are lowered as much as possible
- drugs are used properly for the patients being studied
- goals of the trial can be met
- it is not necessary that the drug or device have a known benefit but there must be a potential of benefit compared to the usual
- sometimes the Investigator can be a Sponsor these are called Investigator Initiated Trials



Stages of Clinical Trials in Canada

Pre-clinical studies

- first tests of a new drug
- tests are done using cells, tissue samples or animals

Clinical trials

- to determine if a drug is safe and effective for people
- take place in hospitals, medical clinics, doctors' offices and universities
- study participants are volunteers who may be:
 - patients with a disease
 - healthy people who want to help advance medical knowledge



Clinical Trials in Canada | Phases of Clinical Trials

Phase 1

Tests an experimental drug on a small group of people for the first time to:

- ensure drug safety
- determine safe dosage range
- discover if there are any side effects

Phase 2 – RARE exceptions

Drug is given to a larger group of people (usually 100 or more) to:

- gather data on how well the drug works to treat a disease or condition
- check the drug's safety on a wider range of people
- determine the best dose

Phase 3

Drug to larger groups of people (usually 1,000+) to:

- make sure it is still effective
- monitor side effects
- compare it to commonly used treatments
- collect information about the drug that will allow it to be used safely on the market

Phase 4

Takes place after the drug is approved and on the market to gather further information on things like:

- best way to use a drug
- long-term benefits and risks





How are clinical trials for rare diseases different?

- U.S. Orphan Drug Act allows exclusive market access to the first product in a disease category ~ 20 years
- patent rules in Canada allow for data protection for the first product ~ 7 years
- some consideration is made, understanding that the sample size may be small
- results may not reach statistical significance because sample size is small or patients are having different severities and stages of the disease
- the local centre is responsible for clinical care and may not necessarily inform patients about clinical trials
- results may take years as it can take a long time to recruit enough patients
- clinical trial may outlast the life expectancy of participants



Only you can determine if the decision to participate in a clinical trial is the right decision for you.

Participation in clinical trials is voluntary.

Participating in a clinical trial is a personal decision. You must make the decision freely, without influence or coercion.

It is your responsibility to ask questions and seek answers

so that you are properly informed and fully understand what it means to enroll in a clinical trial.





Be an informed clinical trial participant. You should understand...

- purpose of the clinical trial
- treatment options available to you now and how they are better/worse than being in a clinical trial
- risks, side effects, or discomforts that might occur
- what will happen during the study (procedures, drugs, devices)
- potential benefits can be reasonably expected
- medical treatments available if complications occur during trial
- opportunity to access to/funding for experimental medicine after the trial



Ask your questions prior to consenting and as new questions arise throughout the trial.



Be informed. Ask Questions.

- Why is this study relevant to me?
- What is the main purpose of the study?
- What is the likelihood that there may be potential benefit?
- What risks are involved?
- Does the study involve a placebo or a treatment already on the market?
- Can I leave the trial at any time?



Be informed. Ask Questions.

- What kinds of tests will be done? Will they hurt? If so, for how long?
- How will the tests in the study compare to tests I would have outside the study?
- Will I be able to continue to see my own doctor during the study?
- Will I be able to continue to take my regular medications during the study?
- If I have side effects, can they be treated during the study?



Be informed. Ask Questions.

Questions about how study participation will affect you personally:

- How much of my time will participating take?
- Do I have to travel?
- Who will review information collected about me during the trial?
- What happens if I decide to guit the study?
- Can the investigator take me out of the study, even if I want to continue?

Questions about compensation and costs:

- Will I be compensated to participate in the study?
- Do I have to pay to be in the study? If so, will insurance cover the cost?
- Will my expenses be covered? (travel, parking, hotel stays, food, missed work)



Common Misconceptions

- clinical trials are dangerous because they use untested drugs
- drugs that approved are considered "safe" and drugs that are not approved yet are not "safe"
- I must give up my usual treatments to join a clinical trial
- I can only participate in clinical trials at the medical center where I am currently being treated
- Clinical studies only use placebos
- Study site/teams know who is actually getting the drug
- I won't be able to see my current doctor if I am in a clinical trial



Common Misconceptions

- I will have to pay all of my expenses if I am in a clinical trial
- once I join a clinical trial, I can't change my mind and leave it
- clinical trials are not confidential
- kids cannot participate in clinical trials
- I will have to invest huge amounts of time and energy to participate in a clinical trial
- I won't have access to the drug once the trial ends, even if it's working



Finding Clinical Trials

MitoCanada.org

ClinicalTrials.gov



mitocanada

is dedicated to creating a world where all lives are powered by healthy mitochondria.









MitoCanada.org

