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Ataxie Canada - Ataxia Canada

CLINICAL TRIAL TOOLBOX

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Who are we?

Ataxia Canada - Claude St-Jean Foundation is an organization with a rich 50 years history. It begins with the involvement of Claude St-Jean who chose not to remain inactive in the face of his diagnosis. He convinced a medical community to begin the research which is described as the modern foundation of Friedreich's ataxia research.

The mission of Ataxia Canada is to improve the well-being of people affected by familial ataxia, contribute to research in promising treatments and bring together the community of interest in Canada.

CLINICAL TRIALS: INTRODUCTION

Whether you are considering joining a clinical trial , you would like to play a more active role in your own health care, to help researchers learn more about ataxia, or simply because there is no available treatment for Ataxia available in Canada yet, this guide will help you through this journey.

We crafted this guide to address and demystify the most common concerns surrounding clinical trials. We understand that the world of medical research can be intricate! We are here to empower you with knowledge and **provide insights to better inform your decision making**! As a patient considering participation in a clinical trial, it's essential to ask the right
The terms "clinical trials" and "studies" are often used interchangeably, causing confusion for many patients. We'll clarify the distinction between these two concepts, explaining how clinical trials differ from observational studies and other types of medical research.

What Are the Different Stages of a Drug Trial?
Understanding the various stages of a drug trial can be enlightening. We'll walk you through each phase, from preclinical research to post-market surveillance, illuminating the critical steps taken to ensure the safety and efficacy of new treatments.

What Does That Word Mean?
Medical jargon can be overwhelming, but we've got you covered. This guide includes a comprehensive glossary of commonly used terms in clinical research. Armed with this knowledge, you'll navigate the complexities of clinical trials with confidence and understanding.

How Is Safety Monitored?
Your safety is paramount, and we want you to be fully aware of the rigorous safety protocols in place during clinical trials. In this section, we'll detail the measures taken to monitor your well-being throughout the trial, ensuring you can trust in the process.

What Is Common Etiquette During Clinical Trials
Being a participant in a clinical trial comes with certain responsibilities and etiquettes. We'll provide you with valuable insights on what to expect and how to be a proactive and engaged participant, fostering a positive experience for yourself and others.

As a patient advocacy group, we are committed to supporting you on your clinical trial journey. With this guide in hand, you'll be equipped with the knowledge and confidence to navigate the world of clinical research. Remember, your involvement in clinical trials is pivotal in advancing medical science and improving the lives of people with Ataxia. It is nevertheless, an important and personal decision with important responsibilities and a risk/reward analysis that must be talked with your medical team.

Together, let's embrace this path of discovery and hope. Thank you for entrusting us with your journey.

François-Olivier Théberge
General manager – Ataxia Canada

CLINICAL RESEARCH: TRIAL VS STUDY

CLINICAL TRIALS

- Interventional
- Involves testing a drug / treatment / device
- Typically, cannot participate in more than one clinical trial at a time

WHY ARE CLINICAL TRIALS IMPORTANT

- Clinical trials in rare diseases are essential to gather data on potential treatments, as there is often limited information available due to the rarity of the condition.
- By participating in these trials, patients contribute to scientific knowledge and advancements, potentially benefiting future generations affected by the same rare conditions.

CLINICAL STUDY

- Observational
- Includes:
  - Biomarker Studies
  - Natural History Studies
- Describing / measuring /observing the disease
- No intervention is being tested: no drugs, no treatments, no devices
- Informed Consent still applies
- Can evaluate a single point in time or over the course of time (longitudinal)

WHY ARE CLINICAL STUDIES IMPORTANT

- Define best clinical practices for ataxia to provide the highest level of clinical care to patients;
- Gather data to better understand ataxia progression and quantify changes over time.
- Create and validate outcome measures (e.g., timed walk tests, vision tests) for future clinical trials.
- Expand the network of research centers focusing on ataxia, making clinical trial design and implementation more efficient.
- Collect biological samples (cheek swabs, blood) to identify biomarkers, studying their role in the disease process and tracking changes over time.
- The FA COMS Natural history data was invaluable at providing additional control arm data that lead to the approval of omavelexolone drug application after initial refusal following clinical trials

WHAT IS THE PROCESS FOR A DRUG TO BE APPROVED?

- TIMELINE: A drug or treatment only becomes available to the community after a long and complex process involving several steps. On average, this process takes 12-15 years.
- At any point, for any reason, the drug development program can be paused or cancelled.
- Canada's access: Each country has its own regulatory approval process. When a drug treatment is approved in one country, it does not mean that is will suddenly become available globally.

DISCOVERY

A drug or a treatment is identified that might help people

PRECLINICAL

The discovery is validated on animal models.

REGULATORY EVALUATION

Regulatory agency reviews the preclinical studies and the proposed plan for human studies

CLINICAL TRIALS

If the potential benefits appear to outweigh the risks, then clinical trials can be carried out on humans

REGULATORY APPROVAL

Ensuring that medications are safe and effective

DRUG ACCESS

If approved, the focus is on facilitating drug access through manufacturing, distribution, provider education, and collaboration with health payers

CLINICAL TRIALS: TERMS TO KNOW

THE JARGON!

COHORT — A group of people who participate in the study together. They may be in different study arms Cohort but the same cohort.

CROSSOVER — Participants who receive placebo initially but have access to treatment later in the trial.

DELAYED START — Individuals either get randomized to treatment at the outset of the study or delayed to a specified time.

DOUBLE-BLIND — All participants, investigators, health care providers, and sponsors are unaware of which study arm the participant is in.

OPEN LABEL — Participants, investigators, and health care providers are all aware of which treatment the participant is being given.

PLACEBO-CONTROLLED — One group gets the active treatment, the other gets the placebo. Everything else is the same between the

RANDOMIZED — An experimental study in which people are allocated to study arms randomly. Reduces bias.

STUDY ARM — Each agent of treatment (or placebo) is a Study Arm. Examples of Study Arms: Placebo, Low-Dose, High-Dose..

ADVERSE EVENTS — An unexpected medical problem that happens during a trial. May be mild, moderate, or severe. May be caused by something other than the drug or therapy

BIO-MARKERS — A measurable indicator used to assess disease progression or treatment response

EFFICACY — Effectiveness. The ability of a drug or treatment to produce an effect

ENDPOINT — Specific outcomes measured to evaluate the effectiveness and safety. They determine trial success: Primary and secondary endpoints are defined before the trial begins.

PLACEBO — Placebo is a harmless substance that has no therapeutic effect. It is used as a control in testing new drugs.

TERMINATION — Discontinuing a trial before completion. Can be at a site or the entire study. Can be the decision of the sponsor, site, IRB, or regulatory agency.

TOLERABILITY — The degree to which the adverse effects from a drug or treatment can be tolerated by participants.

WASHOUT — A period of time that participants need to stop an ongoing treatment before becoming eligible for the trial.

WITHDRAWAL — An individual discontinuing participation in a trial. The participant may choose to withdraw or the investigators may require the participant to stop.

OBSERVATIONAL — Collects information about current health status. No drugs, interventions, or treatments.

INTERVENTIONAL — An experiment that tests if a new drug, device, intervention, or treatment is safe and/or effective.

FIRST IN HUMAN — A Phase 1 trial when a new drug is tested in people for the first time. The treatment would have been tested in cells and animals...but not yet in humans. The aim is to find the safe dose range.

SAD — Single Ascending Dose.Participants in a cohort receive a dose, one time. If there are minimal side effects, a new cohort receives a single higher dose.

MAD — Multiple Ascending Dose. Participants in a cohort receive a dose multiple times. If there are minimal side effects, a new

EFFICACY STUDY — Phase 2 or Phase 3. Goal: Determine whether the drug works in treating a specific symptom.

INVESTIGATOR INITIATED STUDY — Physician or academic researcher initiates and conducts the study. No industry sponsor.

REGISTRATION STUDY — Clinical trial intended to provide sufficient data to support the filing of an Approval with a regulatory agency..

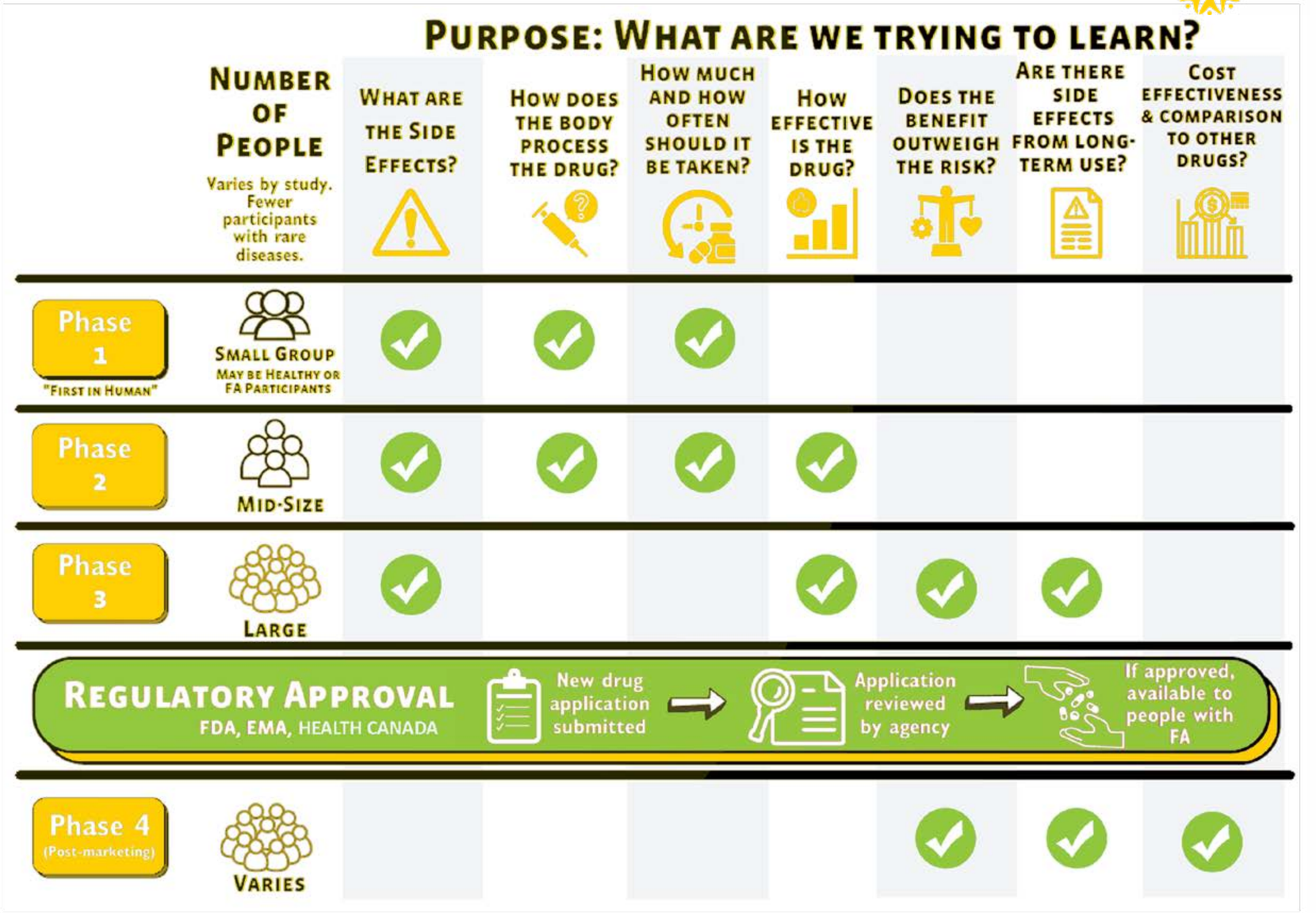
SAFETY STUDY — Establishes safety in either the short- or long-term. Or, if a drug is being repurposed Safety Study confirms safety in targeted disease.

Asking your physician or lead investigator about unfamiliar medical terms in a clinical trial is crucial to ensure clear understanding and informed decision-making about your health.

PHASES OF CLINICAL TRIALS

TIMELINE:

FROM THE FIRST IN HUMAN DOSE UNTIL A DRUG IS AVAILABLE IN PHARMACIES AROUND THE WORLD, THE PROCESS CAN TAKE MANY YEARS OR EVEN DECADES.

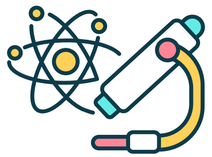


SAFETY MONITORING IN CLINICAL TRIALS: ROLES & RESPONSIBILITIES

BEFORE STUDY

Study Sponsor

- Takes responsibility for and initiates the trial
- Creates a protocol, including guidelines for monitoring safety
- Sponsors are pharmaceutical compa-nies, academic in-stitutions or non-profit



Regulatory Agencies (fda, ema,Health Canada)

- Provides guidelines and advice for trial design and supervision for how drugs or gene therapies are to be tested in humans
- Reviews pre-clinical data to assess safety

IRB: Institutional Review Board Ethics Committee Participant's Role

- Each Site Study Team conducts their own institutional or ethics review
- Assures informed consent
- document clearly explains the study protocol andpossible risks / benefits

Data Safety Monitoring Board (dsmb)

- Set up by Study Sponsor
- Includes clinical experts in both: Ataxia and Safety monitoring
- Can include members from the Ataxia community

Site Study Team

- Assesses health records of potential Participants
- Clinicians determine whether potential Participants can safely participate in the trial

Participant's Role

- Listens closely to the protocol and risks / benefits during Informed Consent
- Asks questions or clarifies any information you read or are told by the study team -this is critically important!

DURING STUDY

- Communicates regularly with the Site Study Team
- If an "adverse event" is reported Study Spon-sor's medical ex-perts: Review it in real time
- Report it to the DSMB and Regulatory Agencies

- Receives ongoing notifications from the Study Sponsor about safety concerns in the study ("adverse events")
- Provides regulatory oversight, including the ability to recommend pausing the study for serious safety concern

- Provides general oversight and monitoring
- Approves any changes to the study protocol that might occur throughout the study time period

- Monitors and reviews all study data throughout the trial
- Recom-mends ac-tion if a safety risk is found
- NOTE: Adverse events do not necessarily result in stoppage of the study because they can range in degree of severity (e.g., mild nausea or headache versus organ damage)

- Monitors Participants' health during the study
- Evaluates all reported concerns ("adverse events") and communi-cates with the IRB and Study Spon-sor
- Can with-draw Participants from the study if there are safety concerns

- Follows the study protocol, including: Instructions for taking drug
- Attending clinic visits
- Undergoing agreed upon procedures
- Alerts the Site Study Team if there are any health and safety concerns
- Participation is voluntary

DURING A CLINICAL TRIAL ETIQUETTE: DO'S & DON'TS

DO'S 

Talking about the Trial

- Share with family and friends that you've enrolled in a clinical trial.

Compliance

- Follow the protocol. If you have a question, call the site.

Follow-Through

- Understand the number of visits the tests that will be done, and any other requirements that you'll be asked to fulfill.

Good Will

- Share your concerns.
- If something is not as you expected, ask.
- Be resilient and patient with the process.

DON'TS 

Talking about the Trial

- If the trial is blinded, don't share how you think the drug / treatment is making you feel . This can jeopardize the integrity of the research.

Compliance

- Don't assume.
- Don't conduct your own study within the study.
- Don't change your medication, vitamins, etc. without speaking to the coordinator.

Follow-Through

- Don't lose interest in the study before you've completed it.
- Participation is voluntary. If you choose to enroll, it's important to fully participate through the end of the trial (assuming no adverse events, etc.).

Good Will

- Share your concerns.
- If something is not as you expected, ask.
- Be resilient and patient with the process.

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