Clinical Trials - A Primer

Should you participate in clinical trials?

Knowing the facts can help you make an informed choice.

A clinical trial is a research study using human volunteers to study the safety and effectiveness of a drug, treatment or device in changing the course of health outcomes. Clinical trials follow basic scientific research that has been conducted to better understand disease pathways and pre-clinical studies that test treatment effects in the laboratory. Trials can be conducted to prevent (e.g., immunization trials) or treat disease (e.g., drug and therapeutic device trials). In ALS research, most trials have been drug trials. Drug trials study whether experimental (new) treatments or new ways of using known therapies are safe and effective under controlled conditions outlined in the study protocol.

What is a study protocol?

A protocol is an action plan based on strict scientific and ethical principles that describes how the study will be conducted and how the patients' safety will be protected. The protocol will outline the number of participants needed, eligibility criteria (characteristics required for inclusion such as age, gender, medical history, etc.), tests that will be performed, length of the trial, treatment details and side effects that may occur.

Locations and sponsors

Trials are usually sponsored by government agencies, pharmaceutical, biotechnology and medical device companies, private organizations, healthcare institutions or individual researchers who are searching for ways to improve the health of persons living with a chronic or life-threatening illness. Clinical studies often take place in hospitals, universities, doctors'. offices and community health clinics. Trials may be conducted in one site or at multiple sites at the same time.

Phase I

Researchers test a new drug or treatment in a small group of people to assess its safety, determine a safe dosage range and identify side effects.

Phase II

The drug or treatment is given to a larger group of people to see if it is effective to further evaluate its safety.

Phase III

The drug or treatment is given to large groups of people to confirm its effectiveness, monitor side effects, compare it to commonly used treatments and collect information that will allow the treatment to be used safely. Participants are randomized into either the treatment or standard care group for comparison.

Phase IV

After a drug or treatment has already been on the market, researchers gather information on the drug's effect in various populations and further study possible side-effects with long-term use.

BENEFITS	√ Access to superior healthcare by leading ALS physician researchers and care teams √ Access to new treatments before they are generally available √ Opportunity to make valuable contribution to clinical
RISKS	x Unknown side effects x May be ineffective or unvalued x Time-consuming with travel to and from study sites x Potential costs associated with travel

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Why are clinical trials important?

Until a cure for a disease is found, it is necessary to continue to search for better, safer and faster-acting treatments to halt or slow down the progression of the disease. Since riluzole (Rilutek®), the only currently approved drug for ALS, has a very modest effect on extending life, other treatments still need to be developed and tested to more effectively treat ALS. Clinical Trial Treatments must go through several phases of testing before conclusions about safety and effectiveness can be drawn. It can take up to five years or longer to develop drugs before human testing begins. In Canada, prior to initiating a clinical trial, the drug developer (usually a pharmaceutical company) must apply to the Therapeutic Products Directorate (TPD) of Health Canada for permission to test on humans.

How safe are clinical trials?

Clinical trials in Canada are regulated by the previously mentioned TPD, similar in function to the FDA in the United States. All federally regulated trials must be approved and monitored by an independent committee of doctors, scientists, advocates and others to ensure safety. These committees are called Institutional Review Boards (IRBs) or Ethics Review Boards (ERBs). The review boards study and approve all study-related documents such as protocols, informed consent forms, physician credentials and eligibility and patient recruitment methods. Data and Safety Monitoring Committees are also common to clinical trials. These independent groups of experts carefully monitor data to detect benefit or harm and validity of results.

What is informed consent?

Understanding all the facts about a clinical trial before you decide to take part is known as 'informed consent'. Before you join a trial, you are required to sign a consent form confirming that you have agreed to conditions. The form outlines key details of the study, including those related to tests, treatments, potential risks, benefits, side effects and participant rights and responsibilities. A member of the research team should discuss the study with you and answer any questions you may have before you sign. You have the right to continue to ask questions throughout the study period and withdraw your consent at any time.

Questions to ask:

- What is the purpose of the study?
- Will it be available after the trial is over?
- Will there be follow-up after the trial is over?
- Will the study require extra time, effort or expenses on my part?
- What does my family need to know?
- Where can I learn more about the study/ researchers?
- What are the risks and benefits?
- How does this treatment compare with other treatments available?
- What kinds of tests and procedures will be required?
- Where will they take place?
- How long will I recieve the treatment?
- How does it support progress toward a better treatment and cure for ALS?
- Who has reviewed and approved the study?
- What sponsors will be paying for the treatment and tests?
- How does the money get spent?

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