



AMYOTROPHIC LATERAL SCLEROSIS SOCIETY OF MANITOBA

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RESEARCH FAQs

Fact Sheet

STEM CELLS

What are stem cells?

Stem cells are cells not yet specialized to perform a particular function. They have the potential to differentiate into blood cells, muscle cells, neurons, and many other types of cells that perform a specific function. Stem cells have two possible fates: they can divide repeatedly or they can differentiate to produce the cells needed for growing tissues through self-renewal.

What are pluripotent stem cells?

In 2006, a new source of stem cells was identified. These cells are called induced pluripotent stem (iPS) cells. iPS cells are reprogrammed adult cells most commonly taken from a patient's own skin, but muscle, liver and other tissue types can also be sources. Scientists found a way to "turn back the clock" on skin cells, such that they will have a pluripotent potential believed to be equivalent to embryonic stem cells.

Jean-Pierre Julien, professor of anatomy and physiology at the Centre hospitalier de l'Université de Laval (CHUL) Resource Centre, Quebec, explains, "Studies on iPS cell-derived motor neurons might lead to the discovery of biological defects associated with ALS."

How stem cells treatments might work for ALS

To benefit people living with ALS, stem cells would ideally support neuroregeneration, replacing damaged neurons and spurring functional recovery, but also could serve a secondary role by protecting neurons from injury.

Does the ALS Society of Canada fund stem cell research?

ALS Canada is encouraging researchers to apply revolutionary methods that create stem cells from a patient's own body to find treatments and cures for ALS.

For more information about stem cells please visit www.als.ca to download our stem cell fact sheet.

CLINICAL TRIALS

What is a Clinical trial?

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.

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.

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Treatments under study 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, before 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.

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.

Source: <http://clinicaltrials.gov/info/resources>

Are there clinical trials taking place in Canada?

Currently two Phase III clinical trials are taking place in Canada: Dexamipexole (closed) and Ceftriaxone (closed). For more information about these clinical trials, please visit our web site at www.als.ca.

CANADIAN ALS RESEARCH NETWORK (CALS)

Created and operated by its physician members, CALS is a national alliance of leading ALS clinicians and researchers committed to the expansion of ALS clinical research studies in Canada and to the pursuit of effective therapies for patients with ALS.

This includes both investigator and industry initiated multicentre national and international studies testing promising therapeutics for patients with ALS. CALS is a non-profit, non-share corporation which is supported by the ALS Society of Canada and is a recognized affiliate society of the Canadian Neurological Sciences Federation (CNSF). CALS has a co-operative relationship with the Northeast ALS Consortium (NEALS) in the United States and the two networks have collaborated in a number of NIH, ALS Society of Canada and ALS Association funded clinical trials.

IS THERE A CANADIAN ALS REGISTRY?

The Canadian Neuromuscular Disease Registry (CNDR), established in 2011, is a Canada-wide database of patients who have been diagnosed with a neuromuscular disease. CNDR includes 17 clinics across Canada. All patients both adults and children across Canada who have been diagnosed with a neuromuscular disease are able to join the registry.

This national registry will help patients connect with researchers to participate in clinical research that will benefit them by offering possible new therapies, treatments and understanding of their disease.

The CNDR is supported by the ALS Society of Canada, Jesse's Journey and the Marigold Foundation. For more information about the registry, please visit www.cndr.org.