

## ANNUAL *Funding Activities*

For a few years now, Ataxia Canada - Claude St-Jean Foundation has organized an annual fundraiser (ATAXIA CHALLENGE: biking and walking) in addition to direct mail and activities organized by pairs (members or sympathizers to the cause) or by members of the Board of Directors.

This challenge allows us to raise funds for the research of Dr. Jacques P. Tremblay via gene therapy. The results are promising, as he has managed, with all his team, to cure mice.

The future seems promising, but we have an urgent need for money! Manufacturing and/or purchasing viral vectors under good manufacturing practices, toxicological testing, new mouse trials, etc. are necessary steps before clinical trials using humans.



## DONATE *Generously!*

1. MAIL CAMPAIGN
2. CHECK, VISA, MASTERCARD
3. I MAKE AN ONLINE DONATION:  
[www.imakeanonedonation.org/ataxiecanadaataxia/](http://www.imakeanonedonation.org/ataxiecanadaataxia/)
4. FUNDING ACTIVITIES:  
Ataxia Challenge  
(biking and walking)



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## MOVING TOGETHER *to Advance Research!*



ATAXIE  
ATAXIA  
CANADA



### WHY THE CHAMELEON?

Without having high physical capacity, the chameleon is very sensitive to its surroundings; it can adapt to any environment.

## WHO *Are We?*

Family ataxias are among the 7,000 rare and orphan diseases recorded worldwide (sources: RQMO). Hardly diagnosed with precision, these ataxias can't be treated, only medications can alleviate certain symptoms or reduce physical effects via occupational therapy, physiotherapy or speech therapy.

Founded in 1972, Ataxia Canada - Claude St-Jean Foundation fights this fatality by supporting medical research and the people living with the disease on a national scale. The foundation offers several services, such as financial support for research through the organization of fundraising, information dissemination and support to ataxic individuals and their relatives.

### **Mission**

To bring people living with an ataxia together, as well as the community of interest, in order to promote and protect the ataxic persons, improve their well-being and ultimately eradicate their disease.

### **Values**

- Respect and an open ear
- Dedication and determination
- Responsibility and integrity

## WHAT IS *Ataxia?*

Ataxia is a symptom and not a specific disease. The term «ataxia» refers to disorders of coordination resulting from central nervous system (CNS) impairment. Different types of ataxia are distinguished according to the affected region of the CNS, which is classified according to the mode of transmission, the symptoms and the age of onset.

Ataxia may be caused by lesions in some areas of the CNS, such as traumatic brain injury, or may be inherited by a gene from a single parent (dominant) or both parents (recessive). These hereditary ataxias are able to skip generations and are neurological, genetic and degenerative diseases of the CNS characterized by degeneration of the cerebellum or anatomical zones, including the various nerves attached to it.

The symptoms of family ataxia are mainly incoordination of the lower and upper limbs, the trunk, the neck, and it affects walking, standing, sitting, crawling, kneeling, crouching, etc. It also affects the fingers, hands, speech, larynx, eye movements and even the heart (Friedreich's Ataxia).

## MEDICAL *Research*

The team of Dr. Jacques P. Tremblay (Research Center of the CHU de Québec - Université Laval) undertook research to develop a treatment for Friedreich's Ataxia by targeting the cause of the disease, namely the decrease of frataxin. Friedreich's ataxia is due to a mutation in the frataxin gene, which results in a decrease in the production of this protein in the cells leading to the death of brain and heart cells.

This team works on different therapeutic approaches that all have one thing in common: the delivery of genes into cells of the brain and the heart. The best way to deliver these genes is to use an adeno-associated virus vectors (an AAV). Professor Tremblay and his entire team have successfully cured a Friedreich's Ataxia mouse by using an AAV coding for frataxin. It is therefore the humans' turn now, but several steps remain to be done.

Following a preliminary meeting with Health Canada to establish the objectives to reach for a clinical trial, Dr. Tremblay and his collaborator prepared an action plan to develop an adequate laboratory to produce AAV usable for a clinical trial. This laboratory would be located at the Research Center of Hôpital Maisonneuve-Rosemont (HMR) in Montreal if the project is funded by the Government of Quebec. This would allow clinical trials in Quebec not only for Friedreich's Ataxia but also for many other hereditary diseases. Such a laboratory will also allow the development of several biotechnology companies in Quebec.