



CFA

CANADIAN  
FABRY  
ASSOCIATION

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# NEWSLETTER

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## Save the Date! June 3rd, 2023

our next Patient Empowerment Meeting for those living in Alberta, Saskatchewan, and Manitoba. Registration will be opening soon.

**CFA** | CANADIAN FABRY ASSOCIATION

### Patient Empowerment Workshop

**SAVE the DATE**  
June 3<sup>rd</sup>, 2023  
CALGARY, ALBERTA

“Patient empowerment is a process that helps people gain control over their own lives and increases their capacity to act on issues that they themselves define as important.”

**empowerment**

**CALGARY, ALBERTA**  
Calgary Marriott Downtown Hotel  
110 9th Avenue SE, Calgary

*Be rare be you!*

*WE CAN DO hard things*

*WE CAN DO hard things*

## Courageous Parents Network

(CPN) is hosting a webinar on Understanding & Addressing Medical Trauma: April 12, 2023.



### UNDERSTANDING AND ADDRESSING MEDICAL TRAUMA

12 April 2023  
8:00 PM ET  
ZOOM

**PANELIST**

Meghan Marsac  
Pediatric Psychologist and Author


**PANELIST**

Melissa Hogan  
Parent & Author

**IN THE ROOM**


[www.courageousparentsnetwork.org/events](http://www.courageousparentsnetwork.org/events)

PATIENT EMPOWERMENT QUESTION: Q. How many rare diseases have been identified in the world?



## Frequently Asked Questions about the PERIDOT Study

A clinical research study for patients with Fabry disease who have nerve pain and abdominal pain.



Patient Experience in Fabry Disease On Venglustat

MAT-CA-2300157 **a sanofi study**

# Venglustat Clinical Trials

Link: <https://www.fabrycanada.com/venglustat-trials-approved-sanofi/>

Venglustat Trials Approved! (Sanofi) We have exciting news, Sanofi has two new trials that were approved!

## CARAT:

Excerpt from the document:

This clinical research study is seeking adults with Fabry disease to investigate the effect of a new investigational oral drug on the heart.

Clinical Trials (.gov) link. This is the official information posted on the government site.

<http://clinicaltrials.gov/ct2/show/study/NCT05280548>

## PERIDOT:

Excerpt from the document:

This clinical research study is seeking adults with Fabry disease to investigate the effect of a new investigational oral drug on nerve pain and abdominal pain.

Clinical Trials (.gov) link. This is the official information posted on the government site.

<http://clinicaltrials.gov/ct2/show/study/NCT05206773>

## Stay tuned for the WORLD Symposium highlights with Dawn Laney - Date TBA



## Rare Disease Day

Thank you to the Fabry community for participating in Rare Disease Day. A day that aims to bring awareness to the 7000+ rare diseases. We stand together as we bring awareness, understanding, and recognize everyone who makes up the rare disease community and their determination to better the lives of all those affected.

## Chiesi Mental Health + Fabry Disease Survey

To help improve the understanding of how Fabry Disease can affect the mental health of individuals and their families, we launched a special anonymous survey with Chiesi intended for members of the Fabry community. We invite you to share your perspectives.

Link: <https://lnkd.in/gQ6hTet4>



**Donations to the CFA are greatly appreciated**

# Sangamo Gene Therapy Trial



Clinical studies Fabry disease **Gene therapy** Study overview FAQ [See if you qualify](#) CANADA

Gene therapy is a rapidly growing field of scientific research in which healthy genes are introduced into patients' cells to treat or prevent their genetic disease.

### What is gene therapy?

Genes are inherited from your parents, they act as a set of instructions for many functions in the cells of the body. However, some people are born with a gene that does not work properly (called a mutation) which can lead to conditions such as Fabry disease.

Gene therapy is an experimental technique that uses genes to treat or prevent disease. There are already a few gene therapies that have been approved to treat diseases. In the future, this technique may allow doctors to treat more disorders by inserting a gene into patients' cells instead of using drugs or surgery.

**Researchers are testing several approaches to gene therapy to fight disease:**

- Replacing a gene that is not normal (mutated) with a healthy copy
- Making a mutated gene inactive
- Introducing a new gene into the body to help fight a disease



### What is ST-920 and how does it work?

In Fabry disease, the gene called galactosidase alpha (GLA), that provides instructions for making an enzyme called alpha-galactosidase A (α-Gal A), does not function correctly.

When α-Gal A enzyme is produced in low quantities, a fatty substance called Gb3 builds up in the tissues, causing disease in vital organs.



ST-920 is designed to deliver a healthy copy of the GLA gene to the liver. The liver should then be able to produce the α-Gal A enzyme and secrete it via the blood stream to the rest of the body. It is hoped that a potential increase in the α-Gal A enzyme in the blood could reduce the need for use of current treatments for Fabry disease.

[More information on Fabry disease](#)

**Infographic download**

To learn more about gene therapy approaches in development for Fabry disease, please click and download the infographic below.

[Download PDF](#)

References available on request

[Contact us](#) [Privacy policy](#) [Glossary](#)

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## OUR INTERNET PRESENCE

Our Website: [www.fabrycanada.com](http://www.fabrycanada.com)  
Facebook: Canadian Fabry Association  
Instagram: Canadian Fabry Association  
Twitter: [@CdnFabry](https://twitter.com/CdnFabry)

## THANKS TO OUR SUPPORTERS

We would like to thank all of our supporters that helped make this newsletter possible.

We receive financial support from these Pharmaceutical companies who are currently providing hope for Fabry patients through their research and the products they provide.



We would also like to thank all of the physicians, specialists and medical professionals that have helped in so many ways. From providing guidance on medical terms and details to caring for members of our community every day.

And of course we would like to thank all of the patients and family members that have volunteered their time and energy to assist in all the many ways that are necessary in the creation of such a large effort. It is through their efforts that we hope to inform and build a community of Fabry patients for the benefit of patients, their families and caregivers.

## MAKE A DONATION

Would you or a family member like to make a donation so that we can continue to educate and advocate for the best treatment as well as communicating with and for Fabry patients in Canada?

The Canadian Fabry Association (CFA) is a registered not-for-profit organization. If you are interested in making a charitable donation and would like a tax receipt, please make your cheque payable to The Fabry's Charity Association.

100% of donations to the CFA are used to promote education, patient support and access to treatment for Canadian Fabry patients. You can make donation cheques payable to The Fabry's Charity Association and mail the cheque to us.

Send the cheque to:  
**The Fabry's Charity Association**  
1964 Hawkrigde Dr.  
Thunder Bay, ON  
P7J 1H2

or register online by visiting our website:  
[www.fabrycanada.com](http://www.fabrycanada.com)

Thanks for your donation to the CFA! It goes to help Canada Fabry patients, their families and caregivers.

## PATIENT EMPOWERMENT ANSWER:

A. 7000+ rare diseases have been identified around the globe.