



CANADIAN  
FABRY  
ASSOCIATION

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# NEWSLETTER

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Therapy

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Jerry Walter

Women's Retreat  
Flyer

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Survey

## My Fabry App

### Our Mission

The myFabry app is designed to **help people** with Fabry disease to **better manage their disease** in their daily lives. This app can be used to learn more about Fabry disease, **easily track the evolution of symptoms** and facilitate **better patient-doctor communication**.



Julia, Canada  
Fabry Disease

SCAN &  
DOWNLOAD



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CANADIAN  
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MAT-CA-220030E (v10) / Janvier 2022



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### myFabry

myHealth



### Notre Mission

L'application myFabry est conçue **pour les patients atteints de la maladie de Fabry** afin de **les aider à mieux gérer leur maladie** dans leur vie quotidienne. Cette application peut être utilisée pour en apprendre davantage sur la maladie de Fabry, **suivre plus simplement l'évolution des symptômes** et faciliter une **meilleure communication** entre patient et médecin.



Julia, Canada  
Maladie de Fabry

SCANNEZ &  
TÉLÉCHARGEZ



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### myFabry

maSanté



### Why myFabry App?



**EXPAND YOUR MEDICAL KNOWLEDGE**

Visualize the human body through a wide range of **educational content** with **3D modelling**. Learn more about Fabry disease, and **discover the potential progression of the disease** within the organs. Using the **glossary**, familiarize yourself with medical terms.

**SHARE THE SYMPTOM TRACKER DATA WITH YOUR DOCTOR**

Choose the time frame and the categories of symptoms you would like to share with your doctor. **Visualize the changes in your symptoms with practical graphs. Export your data from My Tracker**, and have more specific conversations with your healthcare professionals.



**EASILY RECORD YOUR WELL-BEING AND SYMPTOMS**

**Easily track your daily mood and record the changes** in your symptoms with our **questionnaire specifically designed** for Fabry disease. **Monitor** the changes in your condition. **Get notified** of your upcoming medical appointments and tests.

### Pourquoi l'application myFabry?



**DÉVELOPPEZ VOS CONNAISSANCES MÉDICALES**

Visualisez le corps humain à travers un large éventail de **contenu éducatif** grâce à la **modélisation 3D**. Apprenez-en plus sur la maladie de Fabry et **découvrez la progression potentielle de la maladie** au sein des organes. À l'aide du **glossaire**, familiarisez-vous avec les termes médicaux.

**PARTAGEZ VOTRE HISTORIQUE DE SUIVI AVEC VOTRE MÉDECIN**

Choisissez la période et les types de symptômes que souhaitez partager avec votre médecin. **Visualisez les changements de vos symptômes avec des graphiques pratiques. Exportez vos informations** pour aiguiller vos conversations avec vos professionnels de santé.



**SUIVEZ FACILEMENT VOTRE BIEN-ÊTRE ET SYMPTÔMES**

**Suivez facilement votre humeur** au quotidien et **enregistrez les changements de vos symptômes** grâce à notre **questionnaire spécialement conçu** pour la maladie de Fabry. **Surveillez** les changements de votre état. **Restez Informé** de vos prochains rendez-vous médicaux et examens.

PATIENT EMPOWERMENT QUESTION:

Q. How many people in Canada have been diagnosed with Fabry?

# SANGAMO GENE THERAPY

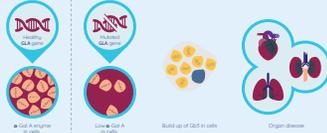
## Gene Therapy Approaches for Treatment of Fabry Disease

Fabry disease is a rare, genetic condition which is estimated to affect around 1 in 100,000 people.



### About Fabry disease

In Fabry, an enzyme called  $\alpha$ -galactosidase A ( $\alpha$ -Gal A) is missing or there is a reduced amount. This means that the body cannot break down a certain type of fat called **globotriaosylceramide (Gb3)**. Gb3 continues to build-up in body cells causing damage to tissues and organs. Gradually, this leads to a range of physical symptoms and complications, which vary from one person to another.<sup>1</sup>



**There is no cure for Fabry disease.** Current treatments can provide an enzyme to break down Gb3 on an ongoing basis. These include Enzyme Replacement Therapy (ERT) which requires frequent burdensome infusions and, for eligible patients, chaperone therapy, a daily oral pill. New treatments are being studied that may prevent organ damage or slow the progression of disease, resulting in improved quality of life for patients.

### What is Gene Therapy?

Gene therapy is a way of altering genetic instructions inside the body's cells to treat or stop disease.

To get the correct copy into the cells, a new healthy gene is created in a laboratory, and placed in a modified (harmless) version of a virus called a "vector", to carry the altered genes into targeted cells.

The new working gene instructs cells to start producing a missing protein or enzyme, and slow or stop the progression of disease.

*In vivo* and *ex vivo* approaches can be used to deliver the working gene into the cells with new instructions. *In vivo* means that the treatment is delivered directly into the body. *Ex vivo* means the person's own cells are modified outside the body, and then returned.<sup>2</sup>

Multiple gene therapy approaches are being studied as a one-time treatment that may provide stable, continuous production of  $\alpha$ -Gal A to slow or stop the progression of Fabry disease.

These include:

Liver-Targeted Adeno-Associated Virus (AAV) Gene Therapy

Cardiomyocyte-Targeted Adeno-Associated Virus (AAV) Gene Therapy

Hematopoietic Stem Cell Therapy



The ongoing STAAR phase I/II clinical study is investigating the safety and tolerability of ST-920 in men and women aged 18 and over with Fabry disease. ST-920 is a Liver-Targeted Adeno-Associated Virus (AAV) Gene Therapy.

The STAAR clinical study is sponsored by Sangamo Therapeutics.

Other gene therapy approaches for Fabry disease

Liver-Targeted Adeno-Associated Virus (AAV) Gene Therapy	Cardiomyocyte-Targeted Adeno-Associated Virus (AAV) Gene Therapy	Hematopoietic Stem Cell Therapy
<ul style="list-style-type: none"> <li><i>In vivo</i></li> <li>Using a vector called an AAV, a healthy copy of the gene responsible for the production of the <math>\alpha</math>-Gal A enzyme is delivered into the body</li> <li>The AAV is administered through an intravenous infusion and targets cells in the liver</li> <li>Once inside the liver, the new working gene is expected to instruct liver cells to make the <math>\alpha</math>-Gal A enzyme</li> <li>Liver cells are then expected to secrete the <math>\alpha</math>-Gal A enzyme via the bloodstream for delivery to other organs</li> <li>No pre-conditioning is administered</li> <li>The patient is monitored for a minimum of 5 years</li> </ul>	<ul style="list-style-type: none"> <li><i>In vivo</i></li> <li>Using a vector called an AAV, a healthy copy of the gene responsible for the production of the <math>\alpha</math>-Gal A enzyme is delivered into the body</li> <li>The AAV is administered through an intravenous infusion with a primary objective of targeting the heart</li> <li>Once inside the heart, the new working gene is expected to instruct heart cells to make the <math>\alpha</math>-Gal A enzyme</li> <li>Heart cells are then expected to secrete the <math>\alpha</math>-Gal A enzyme via the bloodstream for delivery to other organs</li> <li>No pre-conditioning is administered</li> <li>The patient is monitored for a minimum of 5 years</li> </ul>	<ul style="list-style-type: none"> <li><i>Ex vivo</i></li> <li>Hematopoietic stem cells are collected from the patient and then modified in a laboratory with a lentivirus, a vector carrying a healthy copy of the gene responsible for the production of the <math>\alpha</math>-Gal A enzyme</li> <li>Before the cells are injected, a pre-conditioning chemotherapy agent is required to avoid rejection</li> <li>Modified stem cells are administered back to the patient</li> <li>Once inside the body, modified stem cells are expected to produce the <math>\alpha</math>-Gal A enzyme</li> <li>The <math>\alpha</math>-Gal A enzyme is delivered via the blood stream to other organs</li> <li>The patient is monitored for 15 years</li> </ul>

For more details on the STAAR study visit: [www.staarclinicalstudy.com](http://www.staarclinicalstudy.com)

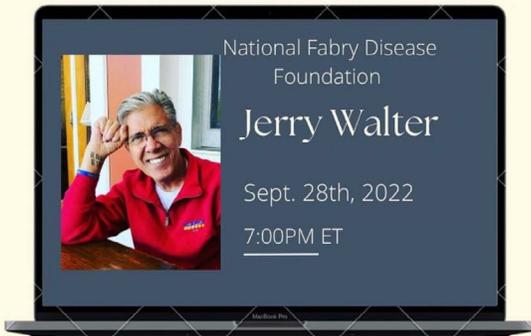
Sangamo<sup>™</sup> THERAPEUTICS

#### References

- Fabry International Network | Fabry International Network. What is Fabry? Retrieved from <https://www.fabrynetwork.org/what-is-fabry/>
- American Society of Gene & Cell Therapy. Fabry Disease and Gene Therapy. Retrieved from <https://asgct.org/global/documents/patient-ed-infographics/sep15-launch-website-e-material/fabry-disease-and-gene-therapy.aspx>

This educational resource was created by Sangamo Therapeutics V1.0, US and Canada - January 2022

## WEBINAR PATIENT STORY JOURNEY OF A HEART TRANSPLANT



Thank you Jerry Walter from the NDFD for sharing his story with the CFA for cardiac Month. To watch Jerry's webinar, find it at [www.fabrycanada.com](http://www.fabrycanada.com)

Julia Alton  
Executive Director

## WOMEN'S RETREAT Thunder Bay



Thank you to everyone who joined the CFA Women's Retreat in Thunder Bay. A weekend full of empowerment, learning, sharing, and true connection.

Julia Alton  
Executive Director



Donations to the CFA are greatly appreciated

# FABRY & FEMALE SURVEY

Please take a moment to help us gather insights to Females living with Fabry disease. Share what your needs are, what you're experiencing in clinic, and how we can better understand the disease manifestations for females. We appreciate your time and willingness to contribute. Link: [www.surveymonkey.co.uk/r/TWSXDR7](http://www.surveymonkey.co.uk/r/TWSXDR7)

Julia Alton  
Executive Director

## CFA CLOTHING LINE

Looking for the perfect gift this Holiday Season? Check out our clothing line at [www.fabrycanada.com](http://www.fabrycanada.com)



## PATIENT EMPOWERMENT ANSWER:

A. 605

## 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.