CANADIAN FABRY ASSOCIATION

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NEWSLETTER

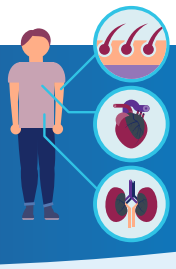
in this issue:

My Fabry App

PATIENT EMPOWERMENT QUESTION: Q. How many people in Canada have been diagnosed with Fabry?

SANGAMO GENE THERAPY

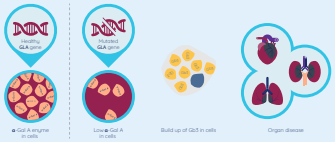
Gene Therapy Approaches for Treatment of Fabry Disease



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 α-galactosidase A (**α-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.[1]



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.[2]

Multiple gene therapy approaches are being studied as a one-time treatment that may provide stable, continuous production of **α-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

STAAR 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
• *In vivo* • Using a vector called an AAV, a healthy copy of the gene responsible for the production of the **α-Gal A** enzyme is delivered into the body • The AAV is administered through an intravenous infusion and targets cells in the liver • Once inside the liver, the new working gene is expected to instruct liver cells to make the **α-Gal A** enzyme • Liver cells are then expected to secrete the **α-Gal A** enzyme via the bloodstream for delivery to other organs • No pre-conditioning is administered • The patient is monitored for a minimum of 5 years	• *In vivo* • Using a vector called an AAV, a healthy copy of the gene responsible for the production of the **α-Gal A** enzyme is delivered into the body • The AAV is administered through an intravenous infusion with a primary objective of targeting the heart • Once inside the heart, the new working gene is expected to instruct heart cells to make the **α-Gal A** enzyme • Heart cells are then expected to secrete the **α-Gal A** enzyme via the bloodstream for delivery to other organs • No pre-conditioning is administered • The patient is monitored for a minimum of 5 years	• *Ex vivo* • 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 **α-Gal A** enzyme • Before the cells are injected, a pre-conditioning chemotherapy agent is required to avoid rejection • Modified stem cells are administered back to the patient • Once inside the body, modified stem cells are expected to produce the **α-Gal A** enzyme • The **α-Gal A** enzyme is delivered via the blood stream to other organs • The patient is monitored for 15 years

For more details on the STAAR study visit: www.staarclinicalstudy.com

Sangamo THERAPEUTICS

References

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

This educational resource was created by Sangamo Therapeutics
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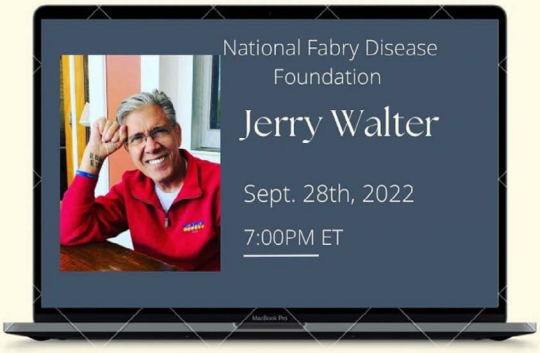
WEBINAR

PATIENT STORY

JOURNEY OF A HEART TRANSPLANT



Thank you Jerry Walter from the NFDF for sharing his story with the CFA for cardiac Month. To watch Jerry's webinar, find it at 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

Julia Alton
Executive Director

CFA CLOTHING LINE

Looking for the perfect gift this Holiday Season? Check out our clothing line at www.fabrycanada.com

PATIENT EMPOWERMENT ANSWER:

A. 605

OUR INTERNET PRESENCE

Our Website: www.fabrycanada.com
Facebook: Canadian Fabry Association
Instagram: Canadian Fabry Association
Twitter: @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.

Amicus Therapeutics
AVROBIO
Chiesi
idorsia
SANOFI GENZYME Empowering Life
Takeda

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 Hawkridge Dr.
Thunder Bay, ON
P7J 1H2
or register online by visiting our website:
www.fabrycanada.com

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