ISSUE 07

FABRY AWARE

Education • Empowerment • Innovation • Community • Together

CFA ACF
CANADIAN FABRY ASSOCIATION
L'ASSOCIATION CANADIENNE DE FABRY

WWW.FABRYCANADA.COM

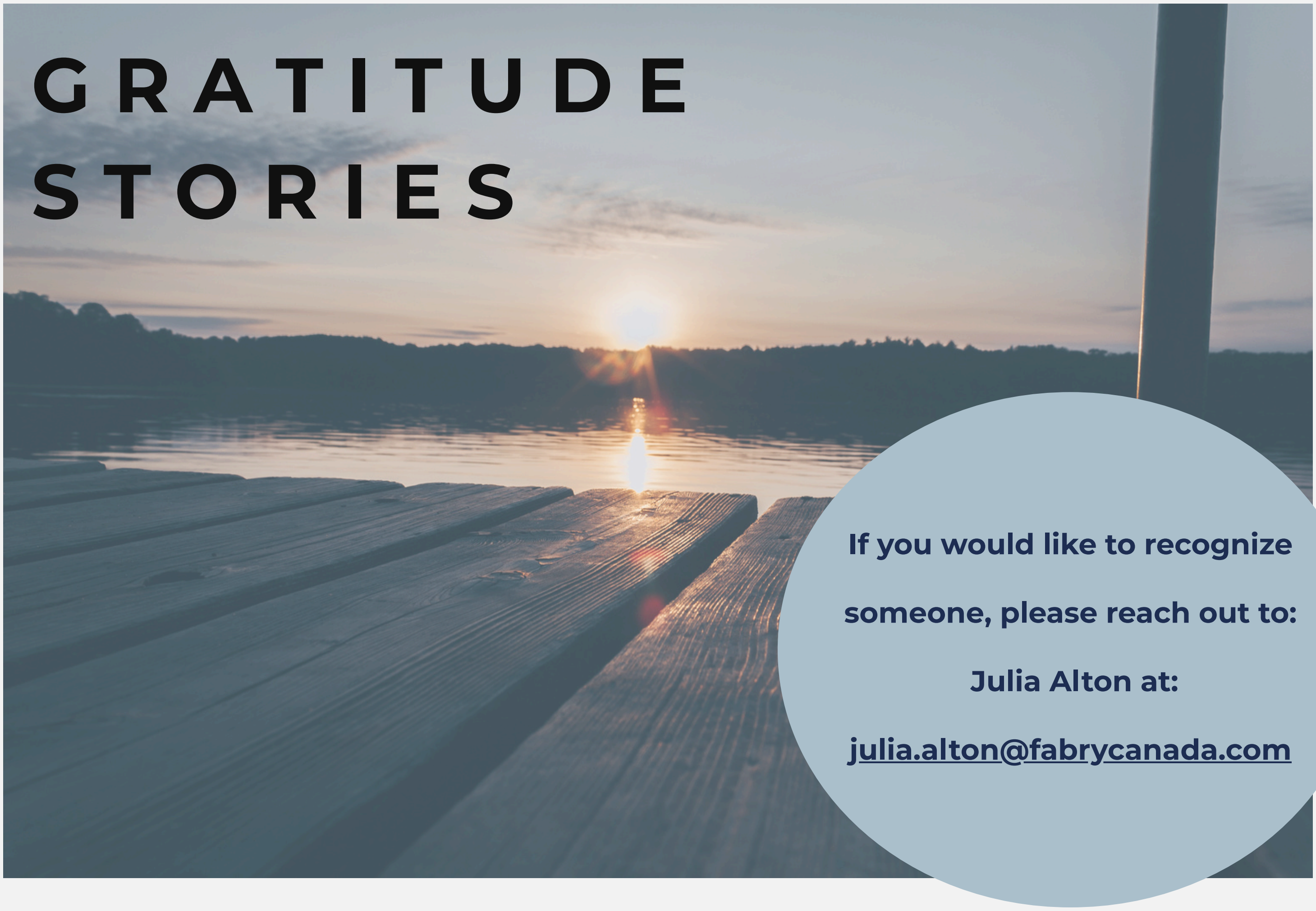
Winter e-magazine

Contents

Designed to keep you informed and inspired on your Fabry journey.

Sections

GRATITUDE STORIES



If you would like to recognize someone, please reach out to:

Julia Alton at:

julia.alton@fabrycanada.com

As part of our **20th anniversary** celebrations, the Canadian Fabry Association is collecting gratitude stories recognizing healthcare professionals who have made a meaningful impact in the Fabry community. If there is a healthcare professional - physician, nurse, or care team member who has made a meaningful difference in your Fabry journey, we would love to hear their story.

These gratitude stories will help us highlight the **compassion, dedication, and excellence** that exist within Fabry care across Canada.

An empowering day of connection, learning, and shared experience in British Columbia.

In November 2025 we held our BC & Yukon Patient Empowerment Meeting and it was nothing short of inspiring. Bringing together patients, families, caregivers, and the broader fabry community , the day was filled with meaningful conversations, practical learning, and a powerful sense of connection. The educational sessions provided valuable insights into medical, emotional, and day-to-day realities of Fabry disease.

Patient Perspective:

"I often feel like I am navigating Fabry disease on my own, being in a room with people who understand what it's like both physically and emotionally was incredib;e validating. I walked away from the breakout session feeling hopeful, inspired, and can put to use tips I learned from others."

FIN Youth Retreat: Emma’s Reflection

“Going to Antwerp for the FIN Youth Weekend Retreat was one of the best experiences of my life. We had the opportunity to do some amazing activities, learn from some very intelligent individuals and we got the chance to bond. I met some of the most amazing and inspirational people I have ever met in my life, Fabry youth from all over the world. I have made bonds that can never be broken, we aren’t just a group of people with the same disease, we are a family.”

“I am so happy they opened their arms and welcomed me in. I learned so much from every single one of the youth, I will hold all the memories with me for the rest of my life. Having Fabry is hard but being around this group of people can easily make you forget how hard it can be and just make you grateful that it's the thing that brought you all together. I am so honoured that I got this opportunity and I will remember it for the rest of my life. Team Panda Forever.” -Emma Thornhill

Patient Experience & Care Survey

We thank you in advance for your time and thoughtful input - if you would like to enter your name in for a draw to win a $50 Amazon GC please e-mail julia.alton@fabrycanada.com

Your Experience Matters!

Your lived experience with Fabry disease is essential to improving care. This survey is designed to help the Canadian Fabry Association better understand what patients experience day to day, what is and isn't discussed in clinic, and what healthcare teams are doing well. Your responses will guide advocacy efforts, education for clinicians, and future patient programs. Participation is voluntary and confidential, and you may skip any questions you prefer not to answer.

Thank you for taking the time to share your perspective - your voice matters.

To participate in the survery

click here!

Sangamo
THERAPEUTICS

Sangamo Therapeutics, has initiated a rolling submission of the Biologics License Application (BLA) to the FDA seeking accelerated approval of isaralgagene civaparvovec, or ST-920, a wholly owned investigational gene therapy for the treatment of adults with Fabry disease.

Rolling submission allows for completed modules of the BLA to be submitted and reviewed by the FDA on an ongoing basis rather than waiting for the entire BLA to be submitted at once.

Click here to read the full news release:

Link

Live-cel™ Therapy - One Treatment for Years of Relief

Contact:
Chris Hopkins
Dm +1 801 631 9114
chris@glafabra.com

Glafabra
Therapeutics

A clinical-stage biotherapeutics development company

Company Overview

- **Lead asset for Fabry in clinic - Safe, Effective and Durable.**
- **Seasoned operators from the cell and gene therapy industry.**
- **Live-cel(TM) technology - a cell-based gene therapy approach.**
- **Targeting Fabry and other enzyme deficiency disorders.**
- **Ex vivo autologous - patient's own immune cells engineered to provide missing enzyme.**

Problem

Current standard of care in Fabry treatment is enzyme replacement therapy (ERT) and requires high frequency protein infusions - patients need to get the jab-for-the-bag every other week. They need to build their lives around tedious clinical visits. Our Live-cel infusions use patient cells and last 130x longer. With Live-cel, patients only need to repeat an infusion procedure every 3-5 years. Patients get all that time back with their family and can now go away easily, for extended-stay trips.

Highlights

- Glafabra has recruited Uncommon Cures to help guide us on clinical trials design and regulatory submissions.
- Glafabra has signed LOI with University of Utah to be our main clinical trials and cell manufacturing site.
- Glafabra has signed Exclusive Options Agreement with the Medical College of Wisconsin for patent covering our cell engineering technology.

Go-To-Market Strategy

Glafabra's go-to-market strategy is a repeat demonstration of the Fabry therapy under a phase 2 clinical trial in the US. Also, we plan to demonstrate the same core technology platform can be applied to Gaucher and Pompe diseases. Demonstration that our approach is effective in more than one disease will indicate our approach is broadly applicable to 100s of other genetic diseases. Multi-indication demonstration will drive a $1-$4 billion exit when we achieved multi-indication phase 2 efficacy data before 2030.

What Makes Us Special

- Clinical stage startup - Our lead asset clinically demonstrated in Canadian clinical trial.
- Filing an IND in early 2026, in collaborating with Uncommon Cures.
- Therapy can be re-dosed as needed every 3-5 years
- Team is comprised of seasoned experts in biopharma development.

Financial Info

Raising
$0.5 M

Valuation
$5 M

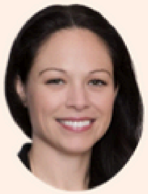
Location
Park City, UT, USA

Business Stage
pre-seed

Business Type
Healthcare, Biotech, Biopharma development

Meet the Team

Chris Hopkins
CEO



Elizabeth Wagner
COO

Krista Casazza
Reg. Sci.

Dwayne Barber
Clin. Ops

Amicus Therapeutics

BIOMARIN

Important News from Amicus Therapeutics:

On Dec. 19th 2025, Amicus Therapeutics, the maker of Galafold, announced it has agreed to be acquired by BioMarin Pharmaceuticals. There are no immediate changes for patients.

If you are taking Galafold, your therapy will continue without interruption, and your Case Manager, Patient Education Liaison, and all Amicus Assist services will remain the same, including how you access your medication. Your health care provider will also be informed.

Click the link below to read more:

Link

FABRY PODCAST

A PLACE FOR IMPORTANT CONVERSATIONS

Episode 13: Fabry & the Kidneys with Dr. Michael West

Episode 12: A Modern Era in Cardiac Care: Fabry Disease AI, & the Power of Partnership with Dr. John Jefferies

Episode 11: EMDR Therapy & Fabry with Linda Quaranta

Episode 10: Fabry & Mental Health with Christine Leighland

SCAN THE QR CODE
TO LISTEN

CFA Merchandise

01 Sweatshirts

CFA Blue Ultra Long Sleeve Tee (Stronger Together)
$25.00

CFA Ultra Long Sleeve Tee (Stronger Together)
$25.00

She is Crewneck Sweater
$30.00

We Can Adult Hoodie

CFA Crewneck Sweater (Be Brave)

CFA Crewneck Sweater (Be Rare Be You)

CFA Crewneck Sweater (Be Rare)
$30.00

CFA Crewneck Sweater (Be You)
$30.00

CFA Crewneck Sweater (Rare)
$30.00

02 Youth selection

We Can Toddler Hoodie (Grey)
$30.00

We Can Youth Hoodie
$30.00

CFA Toddler Hoodie
$30.00

We Can Toddler Hoodie (Navy Grey)
$30.00

CFA Youth Crewneck Sweatshirt (Be Brave)
$30.00

CFA Youth Crewneck Sweatshirt (Be Rare Be You)
$30.00

CFA Crewneck Sweater (Be Rare)
$30.00

CFA Crewneck Sweater (Be You)
$30.00

CFA Crewneck Sweater (Rare)
$30.00

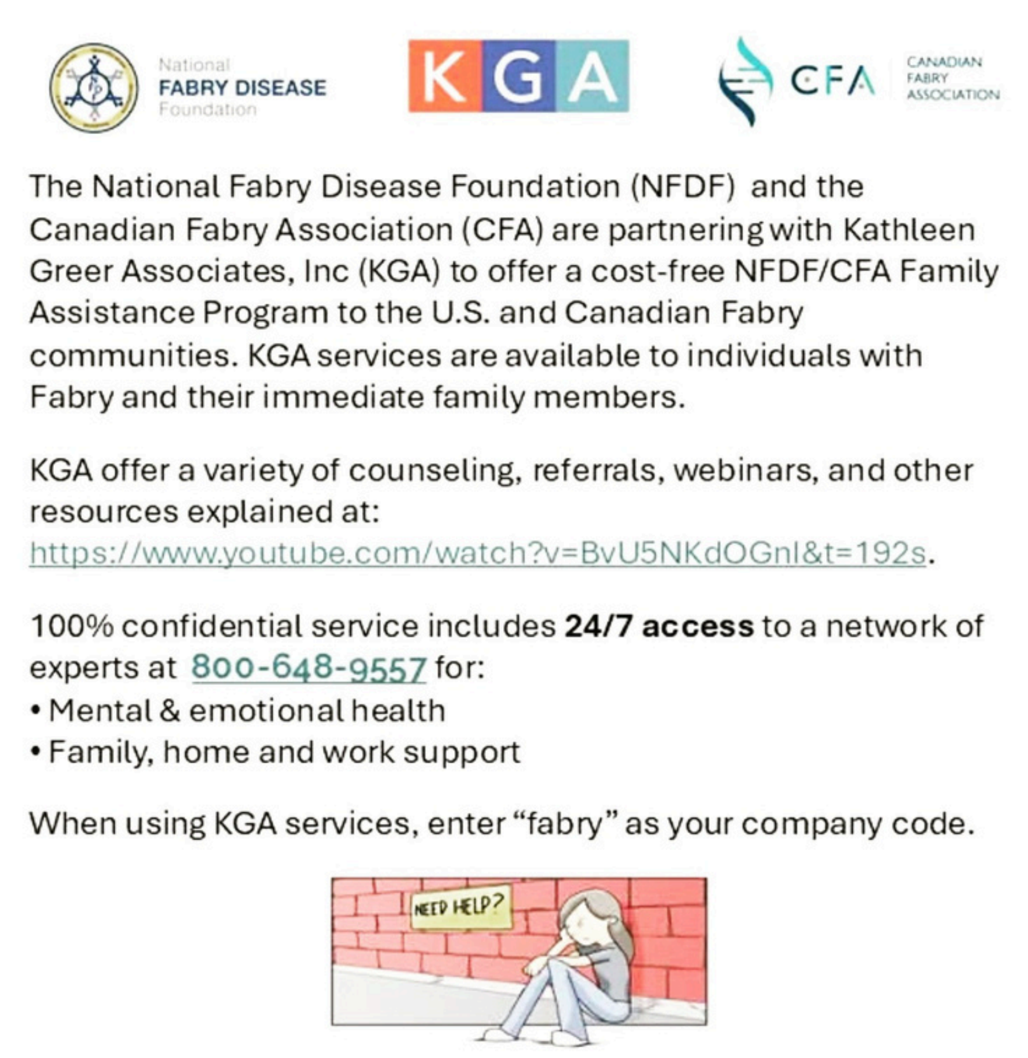
CFA | CANADIAN FABRY ASSOCIATION

Français

GET CONNECTED

Website: www.fabrycanada.com
Facebook: Canadian Fabry Association
Instagram: Canadian Fabry Association
X: CdnFabry
LinkedIn: Canadian Fabry Association

IN PARTNERSHIP WITH



The National Fabry Disease Foundation (NFDF) and the Canadian Fabry Association (CFA) are partnering with Kathleen Greer Associates, Inc (KGA) to offer a cost-free NFDF/CFA Family Assistance Program to the U.S. and Canadian Fabry communities. KGA services are available to individuals with Fabry and their immediate family members.

KGA offer a variety of counseling, referrals, webinars, and other resources explained at: https://www.youtube.com/watch?v=BvU5NKdOGnI&t=192s.

100% confidential service includes **24/7 access** to a network of experts at 800-648-9557 for:

- Mental & emotional health
- Family, home and work support

When using KGA services, enter “fabry” as your company code.

KGA offers mental health, family, work, and home support, referrals, webinars, and other resources explained at:

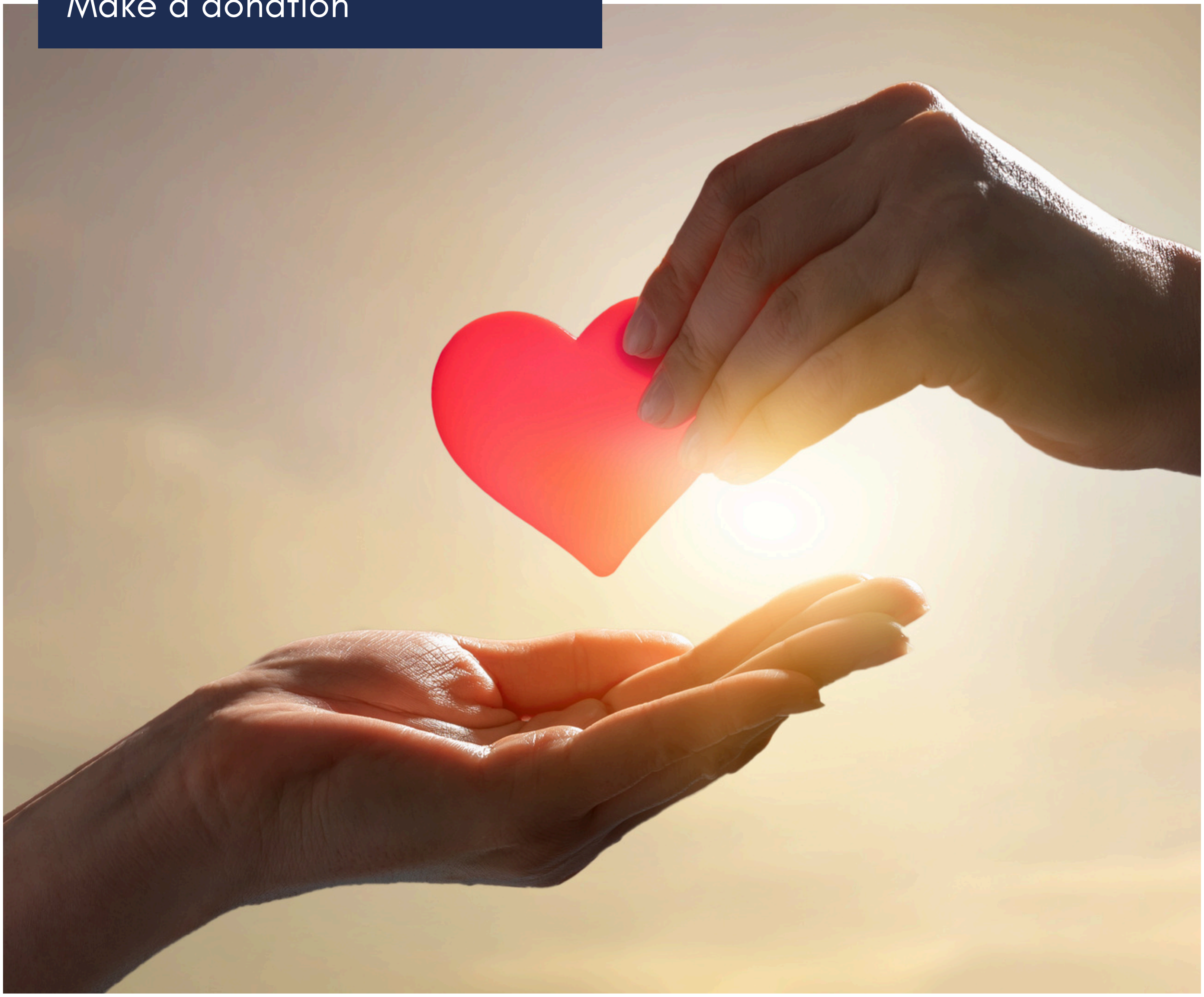
https://www.youtube.com/watch?https://www.youtube.com/watch?v=BvU5NKdOGnI&t=192s

Please note that the video was recorded before our CFA partnership so the CFA is not mentioned in the video, but the entire program applies to the Canadian Fabry community also.

****Please use the webinar link above rather than the inactive link in the image below.**

The NFDF and CFA teams

Make a donation



Make a Donation

Thank you for making an impact, it makes a difference.

Your generosity directly contributes to the events and initiatives that bring patients and families education, support, and a sense of community. It enables the CFA to continue to advocate, bring awareness, and support research.
To make a donation to the Canadian Fabry Association visit www.fabrycanada.com.

Or send a cheque to the address below:

The Fabry Charity Association
1964 Hawkridge Dr.
Thunder Bay, ON.
P7J 1H2

Sponsor Corner

Thank You

for your support

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

We would 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.

We would also 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.

Thank you to our **industry leaders** for your support and collaboration. We receive financial support from Pharmaceutical companies who are currently committed to advancing medical research, developing groundbreaking treatments, ensuring access to essential medications, and continuing to create hope for Fabry patients.

All your support **empowers** us to achieve our mission and **create positive change** in the world.

sanofi

Takeda

Amicus
Therapeutics®

Chiesi
global rare diseases