ISSUE 09

FABRY AWARE

Education • Empowerment • Innovation • Community • Together

CFA ACF
CANADIAN FABRY ASSOCIATION
L'ASSOCIATION CANADIENNE DE FABRY

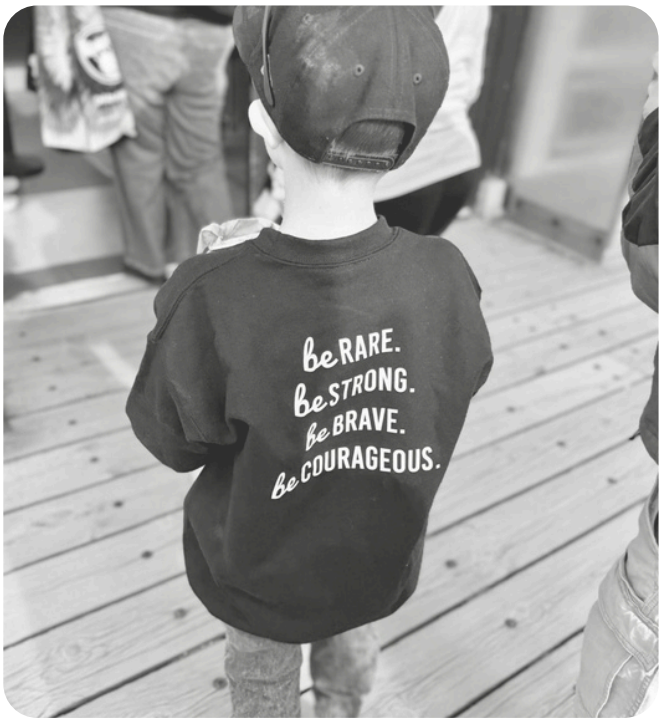
WWW.FABRYCANADA.COM

Summer e-magazine

Contents

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

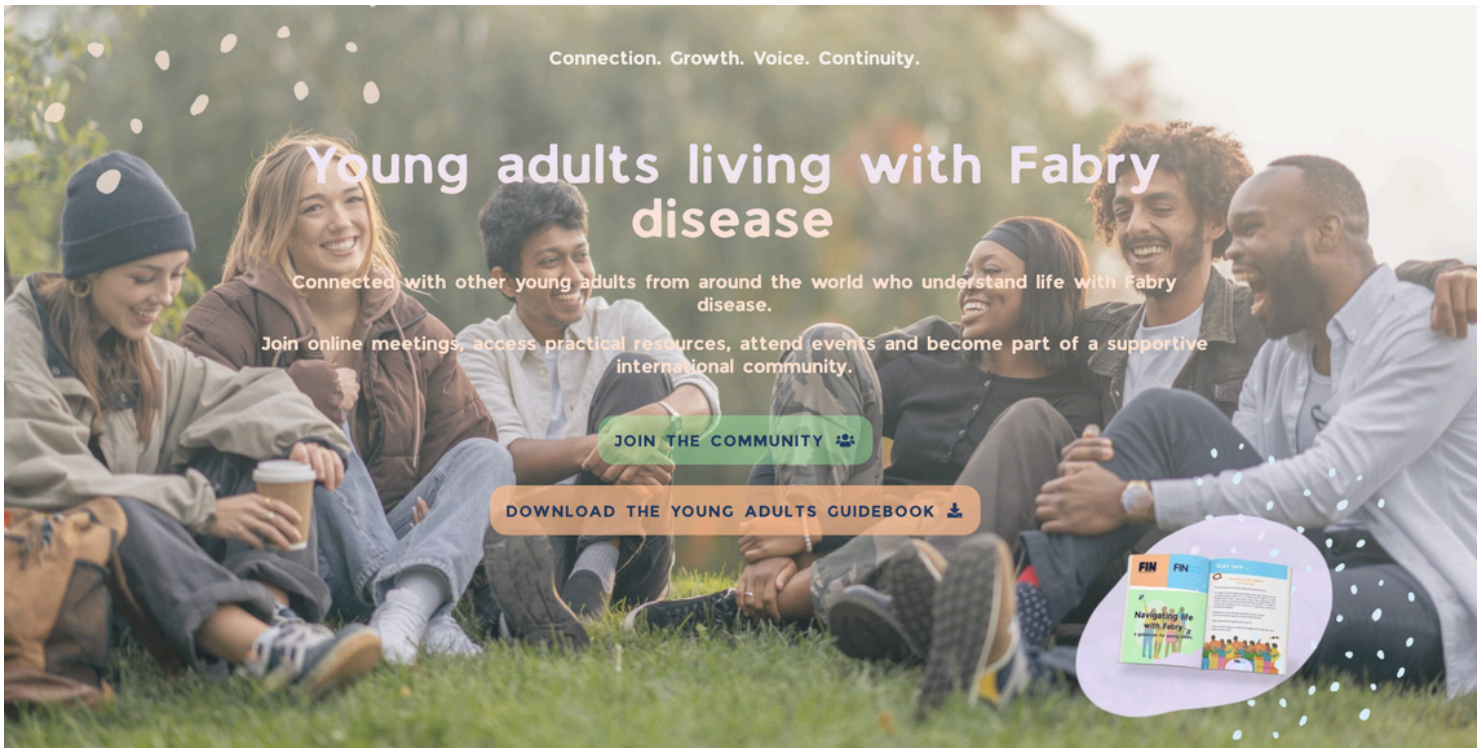
Sections



Quebec Reflection: Rare Together

Our Patient Empowerment meeting brought together passionate voices, inspiring speakers, and plenty of great conversations. The connections made in this room will carry well beyond the event. This is what community looks like in action!

Our Youth and Young Adults meeting brought everyone together for an open, heartfelt conversation. From shared experiences to honest emotions, this group showed up for each other in the best way.



The guidebook explores topics including healthcare, treatment, education, work, relationships, mental wellbeing, family planning, self-advocacy and more.

Combining practical information with lived experiences, it aims to support young adults as they navigate the opportunities and challenges of adulthood with Fabry disease.

To download the guidebook - click here:

Link

Thank you for the overwhelming interest in the 2026 Women's Retreat.

Registration is now full.

We look forward to seeing you in PEI.

The Canadian Fabry Association was grateful to participate in this year's FIN meeting, which brought together organizations from around the world to share knowledge, strengthen relationships, and explore opportunities to better support the global Fabry community.

This gathering provided a valuable opportunity to learn about the initiatives, challenges, and successes of Fabry organizations internationally, while also highlighting the importance of collaboration in advancing patient education, advocacy, and community support.

CFA Board Takeaways from the Fabry International Network (FIN) Expert Meeting 2026

Women with Fabry Disease Need Equal Recognition

Dr. Kobayashi challenged the outdated belief that women experience only mild Fabry disease. Due to random X-chromosome inactivation, women can experience the full spectrum of disease severity and deserve the same comprehensive monitoring as men. The emotional and psychological burden of living with Fabry disease was also emphasized.

Takeaway: Never let your symptoms be dismissed because you are female.

Hearing and Balance Shouldn't Be Overlooked

Research showed hearing and balance problems are far more common than many realize. Up to 80% of patients experience hearing loss at some frequency, and many are unaware until formal testing.

Takeaway: Ask about routine hearing assessments, and seek immediate medical attention for sudden hearing loss, new tinnitus, or severe vertigo.

Fabry Disease Affects the Whole Body

Experts reinforced that Fabry disease is not limited to one organ. Even people with cardiac-predominant variants may experience hearing, balance and other systemic complications.

Takeaway: Comprehensive, multidisciplinary care remains essential.

Be Prepared for Medical Appointments

With limited time during appointments, experts encouraged patients to arrive with a plan.

Takeaway: Bring your top questions, share trusted information, and work collaboratively with your healthcare team.

Know Your Risks

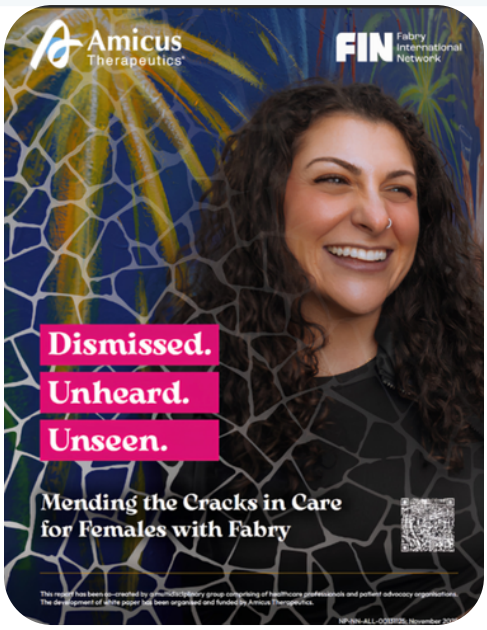
The meeting highlighted the increased risk of stroke in Fabry disease, the high prevalence of gastrointestinal symptoms, and the importance of recognizing symptoms early, where pain and GI issues often appear before significant organ damage.

To read the full Fabry International Network (FIN) Expert Meeting 2026 Report, visit:

Link

Dismissed. Unheard. Unseen.

Mending the Cracks in Care for Females with Fabry



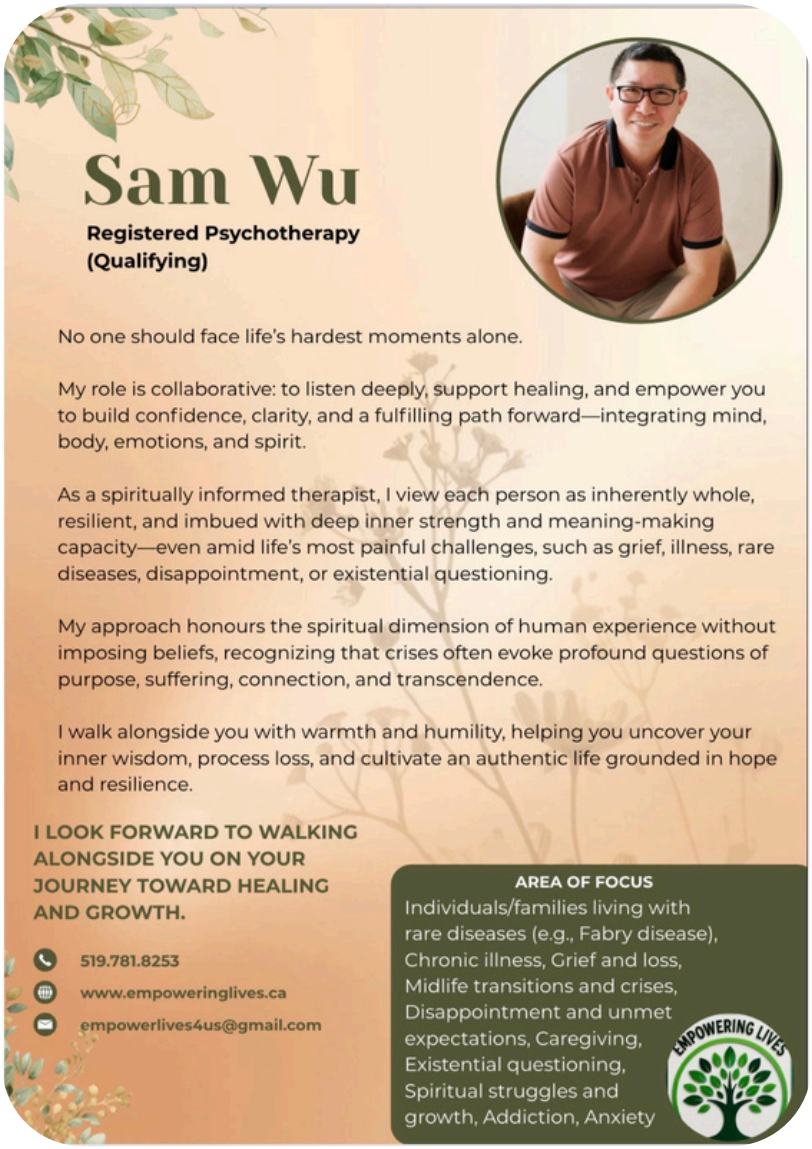
We are proud to share, **Dismissed, Unheard, Unseen: Mending the Cracks in Care for Females with Fabry**, a new white paper developed in collaboration between the Fabry International Network and Amicus Therapeutics. Grounded in lived experience and clinical insight, the report is a global call to action to address the persistent gaps that leave women and girls with Fabry undiagnosed, overlooked, and underserved.

This is a pivotal moment. Momentum is building to reshape care, research, and understanding of Fabry in females so that we can meet the unique needs of women and girls with Fabry. We encourage you to read, share, and use this report as a tool for advocacy, education, and change.

Together, we can help ensure no woman with Fabry is ever dismissed, unheard, or unseen again.

Click here to read more

CFA Resilience Program



Many of you have already had the opportunity to meet Sam during our Patient Empowerment Meetings across Canada. His willingness to travel, connect in person, and listen deeply has made a meaningful impact. He has become a genuine source of support and light for so many in our community.

Sam brings not only professional expertise, but also a grounded, empathetic presence that resonates with individuals and families navigating the complexities of Fabry disease.

We are offering:

5 sessions covered by CFA

We're proud to be able to share this opportunity for continued support to both patients and caregivers. Offered both in person (Ontario GTA) or over Zoom.

Get in touch with Sam by the contact information provided in the poster. He is honoured to walk alongside you in this journey

Acquisition Complete

BioMarin + Amicus Therapeutics

The Canadian Fabry Association recognizes the completion of BioMarin's acquisition of Amicus Therapeutics as a significant milestone within the Fabry community. As this new chapter begins, we would like to express our sincere gratitude to the Amicus team for their longstanding partnership and unwavering commitment to supporting individuals and families affected by Fabry disease.

Over the years, Amicus has played an important role in advancing patient education, advocacy, and community initiatives, and we are thankful for the meaningful collaborations we've shared.

We also look forward to continuing our work with BioMarin as we collectively strive to improve the lives of Canadians living with Fabry disease. By keeping patients and families at the heart of everything we do.

FABRY PODCAST

A PLACE FOR IMPORTANT CONVERSATIONS

Episode 16: - When Your Body Speaks: Holding Space for Both Fabry and Mental Health - Nadia Ali

Episode 15: A Rare Story of Love & Loss - Donna Strauss

Episode 14: A Life Shaped by Fabry - Jerry Walter

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

SCAN THE QR CODE
TO LISTEN

CFA Rare Collection

For 20 years, we've stood together, through challenges, resilience and hope.

The **CFA RARE Collection** honours our community, and every purchase directly supports education, connection and care for patients living with Fabry across Canada.

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

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.

Glafabra THERAPEUTICS

The Canadian Fabry Association is encouraged to see continued innovation in the field of Fabry disease. The FDA granting Orphan Drug Designation to Glafabra's investigational cell therapy represents an important milestone in the development pathway for potential new treatments. While this designation does not mean the therapy is approved or available yet, it reflects ongoing progress in the scientific community's efforts to explore new approaches for treating Fabry disease.

We remain hopeful as research continues and look forward to learning more as development advances.

Click here to read more:

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

IN PARTNERSHIP WITH

National FABRY DISEASE Foundation

KGA

CFA CANADIAN FABRY ASSOCIATION

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.

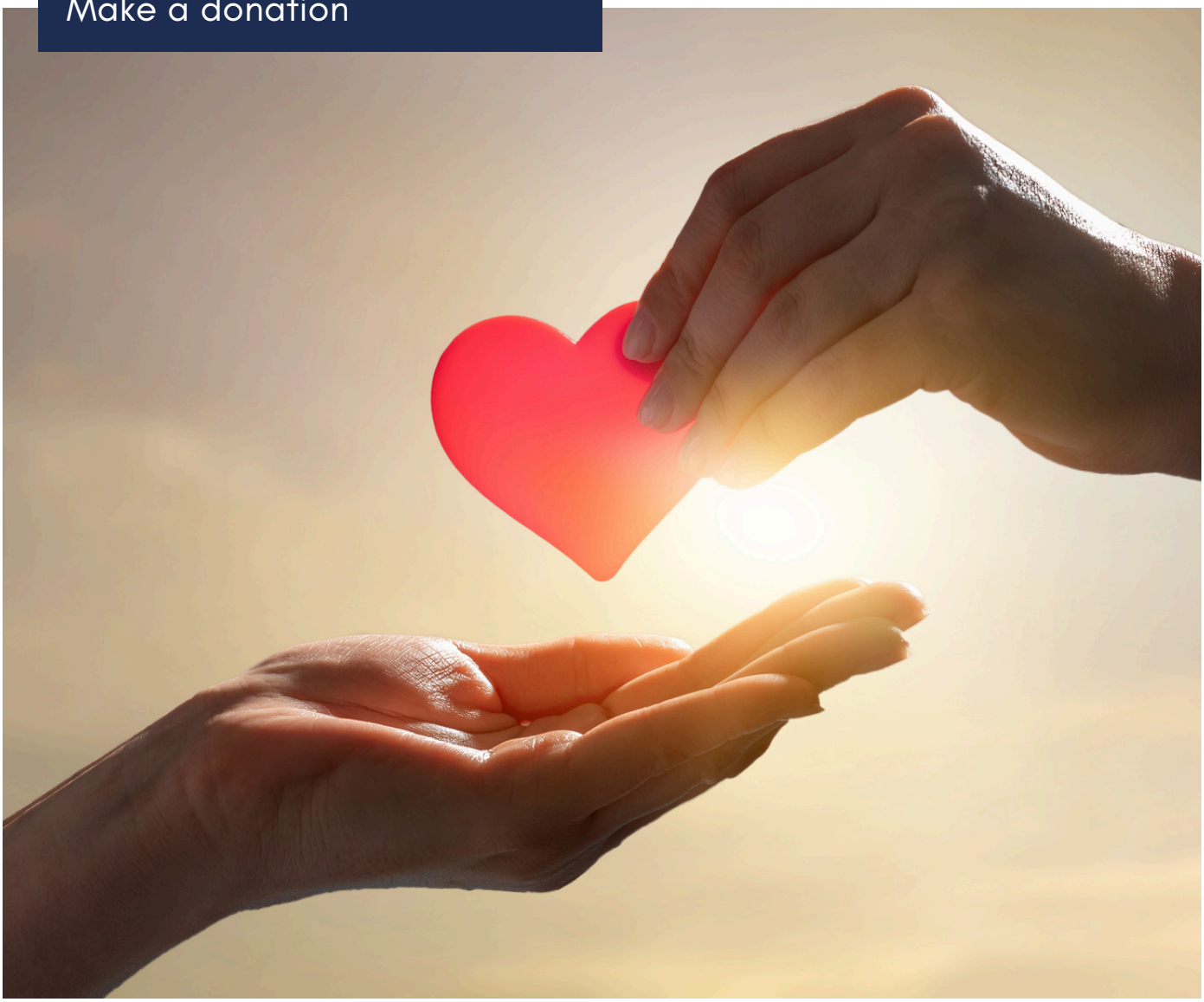
KGA offers mental health, family, work, and home support, referrals, webinars, and other resources explained at:

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

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