

ISSUE 07

FABRY AWARE

Education • Empowerment • Innovation • Community • Together



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

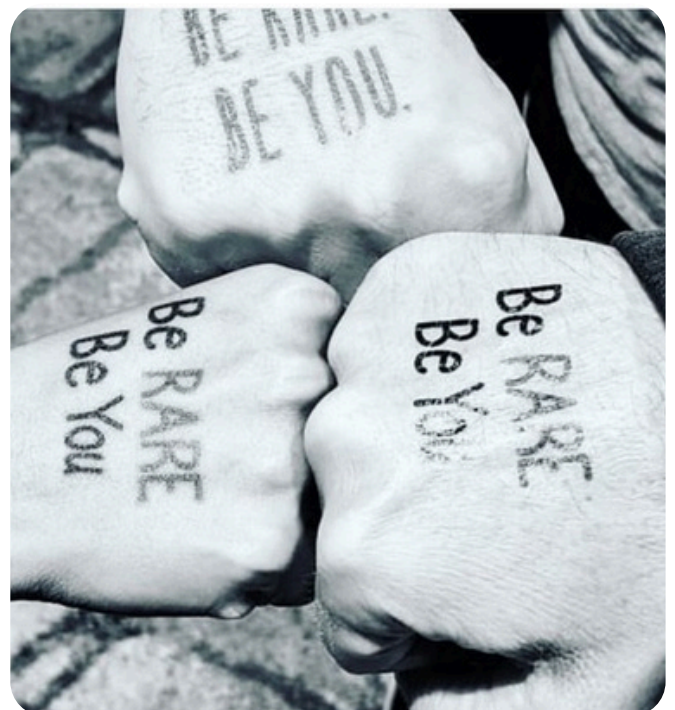
Community Events + News

CFA Updates

Announcements

Sponsor Corner

Closing



FIN Fabry Expert Meeting

In every country, on every continent, there are individuals and families living with Fabry.

The FIN meeting served as a reminder that we are not alone in this work. Instead, we are part of a growing, global community united by shared purpose and compassion.

Throughout the meeting, we explored critical issues: from clinical advances and multidisciplinary care to mental health, menopause, and youth engagement. We learned about creative advocacy initiatives, emerging therapies, and ways to better support families across all stages of the Fabry journey.

Action Step for the CFA

Dawn Laney presented on the intersection of menopause and Fabry disease, which is a critical and under-discussed topic for women living with Fabry. This is a topic the CFA would like to continue learning about to share with our community.



**Thank you to Fabry International Network for 20 years
of dedication, advocacy, and leadership!**

Prairie province patient empowerment meeting and youth & young adult summit



What I Took Home From The Summit

You reminded us that while our paths differ,
we can still choose how we move through
them.

- Fabry Patient



REFLECTIONS:

“Dr. Khan made me feel hopeful for the first time in a long time, I believe better treatments are coming and that someone truly understands what we go through.”

— First time participant, Fabry patient

“It wasn’t just information. It was empowerment. I learned how to ask better questions and advocate for myself with my care team.”

— Young adult living with Fabry

“Being with others who just get it, that alone is powerful. I left feeling more connected and more informed.”

— Parent of a young adult patient

“It felt so good to talk about mental health openly. Sometimes we focus so much on the physical side — I needed this reminder that my emotional well-being matters too.”

— Fabry patient, Alberta

Help Us Solve the A143T and Fabry Disease Mystery!



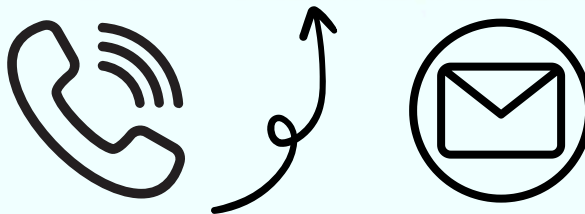
The PAgE Study

Pathogenicity of the c.427G>A (p.A143T) *GLA* variant in male Fabry patients: a multi-center study

The Emory University Department of Human Genetics in Atlanta, GA is currently enrolling interested men 18 years of age and older who have the A143T change in their *GLA* gene in the “Pathogenicity of the c.427G>A (p.A143T) *GLA* variant in male Fabry patients: a multi-center study”

This study involves collection of urine and blood, a physical exam, cardiac testing, skin biopsy, brain MRI, questionnaires, and other tests as indicated. These tests can be done at a medical center near you **OR** you have the option of coming to Emory for a one-day visit. The purpose of the study is to investigate the health impact of living with an A143T change in the *GLA* gene. Study related tests/procedures not covered by insurance and travel expenses will be paid for as part of the study.

If you are interested in participating or would like more information about the study please contact Dawn Laney via phone (404-778-8518) or email (dawn.laney@emory.edu).



Patients and Families living in BC

**Patient Empowerment Meeting
coming soon!**



**SAVE
THE DATE**

**November 22nd
2025**

- Learn from experts in the Fabry field.
- Connect with others going through similar challenges.
- Hear from Dr. Seema Kanwal who shares a functional medicine perspective.
- Get more tools in your toolbox to live well with Fabry.



Empowering Health Through Functional Medicine:

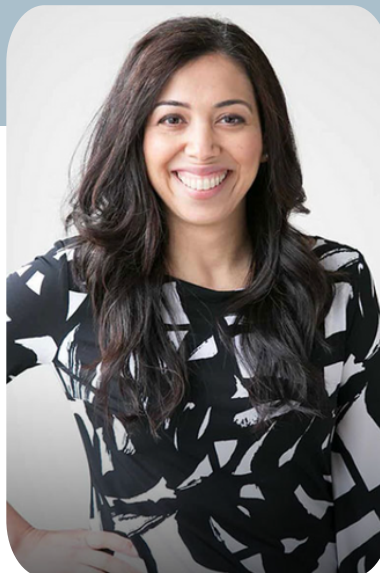
The Canadian Fabry Association hosted a transformative 10-week wellness series led by Dr. Seema Kanwal, a Naturopathic Doctor with a deep understanding of chronic illness and rare disease care.

Through this intimate and engaging virtual program, participants explored essential dimensions of whole-person health — from hormonal balance and anti-inflammatory eating, to sustainable lifestyle strategies that support energy, sleep, and stress management. Each session was grounded in science but delivered with compassion, meeting Fabry patients where they are.

One of the most impactful elements of the series was its interactive and personalized approach. Participants were invited to bring their Fabry-specific questions, allowing Dr. Kanwal to tailor guidance to individual needs and concerns. This format not only provided education, but also created a safe, collaborative space for reflection and empowerment.

We're grateful to Dr. Kanwal for her time, expertise, and thoughtful care. As we continue to center holistic support in our community offerings, we look forward to building on the success of this series.

**We are offering
this 5-part
workshop to
the Fabry
community for
\$25.00**



FABRY PODCAST

A PLACE FOR IMPORTANT CONVERSATIONS

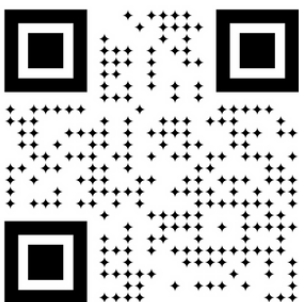


Episode 12: A Modern Era in Cardiac Care: Fabry Disease AI, & the Power of Partnership – Dr. John Jefferies

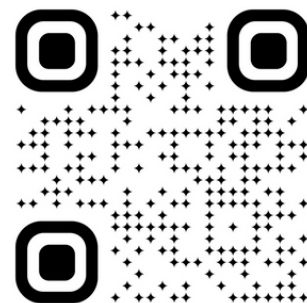
Episode 11: EMDR Therapy & Fabry – Linda Quaranta

Episode 10: Fabry & Mental Health – Christine Leighland

Episode 9: Decision Fatigue – Jennifer Seidman



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

GET IN TOUCH!



GET CONNECTED

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



IN PARTNERSHIP WITH



National
FABRY DISEASE
Foundation



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](tel: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:

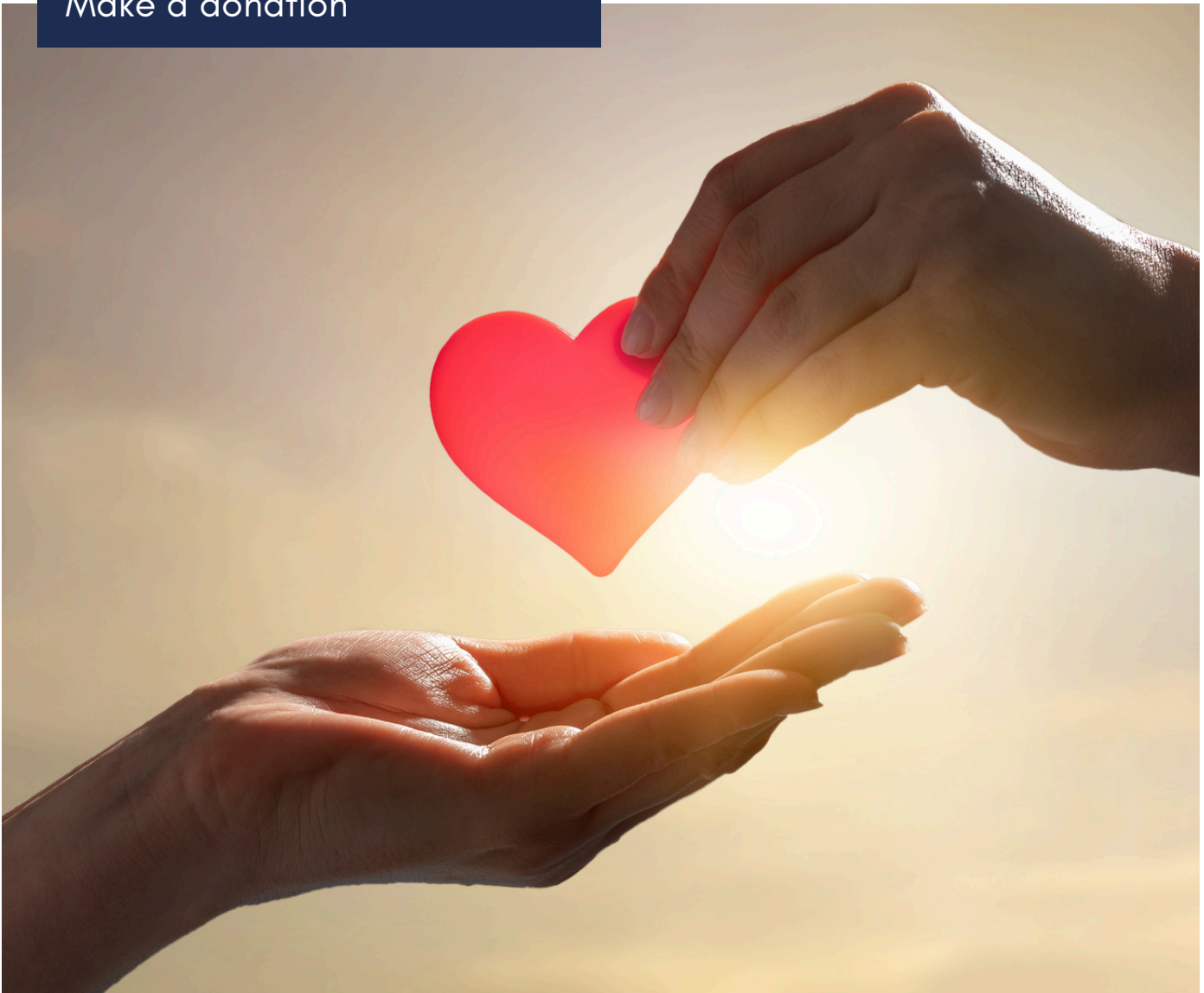
<https://www.youtube.com/watch?v=BvU5NKdOGnI&t=192s>

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

Sangamo Therapeutics Announces Positive Topline Results From Registrational STAAR Study in Fabry Disease

June 24, 2025

STAAR study demonstrated positive mean annualized estimated glomerular filtration rate (eGFR) slope at 52-weeks across all dosed patients in the study, which U.S. Food and Drug Administration (FDA) has agreed will serve as primary basis of approval

Isaralgagene civaparvovec showed a favorable safety and tolerability profile

Sangamo intends to submit a Biologics License Application (BLA) in 2026

RICHMOND, Calif.--(BUSINESS WIRE)--Jun. 24, 2025-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced positive topline results from the registrational Phase 1/2 STAAR study evaluating isaralgagene civaparvovec, or ST-920, a wholly owned investigational gene therapy for the treatment of adults with Fabry disease.

Following a single dose of isaralgagene civaparvovec, a positive mean annualized eGFR slope of 1.965 mL/min/1.73m²/year (95% confidence interval (CI): -0.153, 4.083) at 52-weeks was observed across all 32 dosed patients in the study, which the FDA has agreed will serve as an intermediate clinical endpoint under the Accelerated Approval pathway. Furthermore, a mean annualized eGFR slope of 1.747 mL/min/1.73m²/year (95% CI: -0.106, 3.601) was observed for the 19 patients who have achieved 104-weeks of follow-up.

As recommended by the FDA, Sangamo plans to compare the annualized mean eGFR slope of isaralgagene civaparvovec with approved treatments for Fabry disease by performing a meta-analysis of published studies. According to observational studies, estimated mean annualized eGFR slopes for other marketed treatment options range from -2.2 to -0.4 mL/min/1.73m²/year for treatments such as Replagal (agalsidase alfa)¹, Fabrazyme (agalsidase beta)² and Galafold (migalastat)³.

We believe these data support the potential for isaralgagene civaparvovec as a one-time, durable treatment for Fabry disease that can improve patient outcomes and will form the basis for an anticipated BLA submission under the Accelerated Approval pathway as early as the first quarter of 2026.

The STAAR study enrolled male and female patients who were either on enzyme replacement therapy (ERT), were ERT pseudo-naïve (defined as having been off ERT for six or more months), or were ERT-naïve. The median age of patients enrolled in the study was 42, with a median duration of follow-up of 24 months and the longest treated patient having achieved 4.5 years of follow-up.

Key secondary endpoints in the study were also positive. Elevated expression of alpha-galactosidase A (α-Gal A) activity was maintained for up to 4.5 years for the longest treated patient. All 18 patients who began the study on ERT have been withdrawn from ERT and all remain off ERT as of today. Plasma lyso-Gb3 levels in these patients remained generally stable following ERT withdrawal. A stabilization in cardiac endpoints was also observed.

Patients demonstrated a range of other clinical benefits, including improvements in disease severity reported in the Fabry Outcome Survey adaptation of the Mainz Severity Score Index (FOS-MSSI) age-adjusted score and statistically and clinically significant improvements in the short form-36 (SF-36) quality of life scores, including role-physical +14.8 (95% CI: 7.3, 22.4, p=0.0003), vitality +9.6 (95% CI: 3.9, 15.2, p=0.0017), bodily pain +9.0 (95% CI: 2.3, 15.7, p=0.0104), social functioning +7.8 (95% CI: 2.0, 13.6, p=0.0100), general health +7.4 (95% CI: 2.0, 12.8, p=0.0091), and physical component scores +4.2 (95% CI: 1.8, 6.6, p=0.0014), at week 52 compared to baseline. Statistically significant improvements in the gastrointestinal symptoms rating scale (GSRS) compared to baseline were also observed. Furthermore, following a single administration of isaralgagene civaparvovec, additional clinical benefits were observed in some patients, such as the reduction or elimination in pain medication usage and the resumption of sweating, that has enabled these patients to perform physical tasks and exercise.

Isaralgagene civaparvovec demonstrated a favorable safety and tolerability profile in the study, without the requirement for preconditioning. The majority of adverse events were grade 1-2 in nature. The most common treatment-emergent adverse events (TEAEs) were pyrexia (60.6% of participants), COVID-19 (36.4%), headache (33.3%) and nasopharyngitis (33.3%). All TEAEs resolved in response to clinical management and there were no safety-related study discontinuations.

"Fabry disease is a debilitating and multifaceted condition, for which there is a serious unmet medical need," said Nathalie Dubois-Stringfellow, Ph. D, Chief Development Officer at Sangamo. "We are thrilled to see these compelling topline STAAR study results, including the positive mean annualized eGFR slope at both 52 and 104 weeks, alongside notable improvements in a range of secondary endpoints. Taken together these data demonstrate the potential for a single dose of ST-920 to provide meaningful clinical benefits above current standards of care and to treat the underlying pathology of Fabry disease. We want to thank the patients and investigators who participated in this study and look forward to sharing these data with health authorities."

Isaralgagene civaparvovec has been granted Orphan Drug, Fast Track and RMAT designations from the FDA, Orphan Medicinal Product designation and PRIME eligibility from the European Medicines Agency and Innovative Licensing and Access Pathway from U.K. Medicines and Healthcare products Regulatory Agency.

Analyses of the full dataset from the STAAR study are ongoing and additional data will be presented at an upcoming medical meeting. Sangamo is advancing BLA preparation activities for isaralgagene civaparvovec, while continuing to engage in business development negotiations for a potential Fabry commercialization agreement.

To read
the full
article



click
here!



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.

The Sanofi logo, featuring the word "sanofi" in a dark blue, lowercase, sans-serif font. The letter "i" has a small blue dot above it.