National FABRY DISEASE Foundation

A better understanding can lead to a better future.

End of Year Newsletter ❄️ December 2025

Letter from the President

When I look back on 2025, I see it as a year full of initiatives to improve the understanding and management of Fabry disease. So many individuals and organizations, along with us, have worked hard to improve education and awareness of Fabry disease and have provided programs and resources to help people with Fabry disease live better and longer lives. It seems like the countless people vested in making life better for people with Fabry disease are in an endless relay race. Each time a new study or research paper is completed, a new educational webinar is produced, a new program is implemented, the baton is passed to someone with a new idea to develop and implement. As more questions are answered and more problems are solved, more questions arise. And the race goes on. We sincerely thank all those working diligently on behalf of people with Fabry disease!

On a parallel path, people with Fabry disease and their families are on their separate journeys to live their best lives possible. It takes tremendous time, energy, strength, courage, perseverance, resilience, faith, and sometimes luck to overcome the challenges of living with Fabry disease. I know from experience that surprises, good and bad, come often. Fabry disease is a progressive disease. Even with good management and effective treatment, optimal health is a moving target. Best wishes to our community members as they work to overcome the challenges they face.

I am inspired by the scientific progress that has been made and what is yet to come, including increased early diagnoses, improved management, and better treatments. I am inspired by the growth and mutual support in our community. I am inspired and thankful for the opportunities for children and adults to avoid some of the devastating effects of Fabry disease when treatment can be started earlier in life and with improved management.

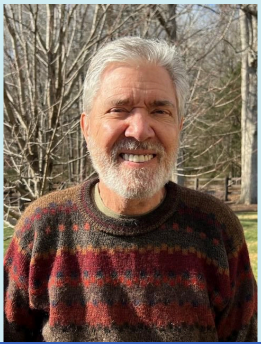
I remain sad for the families who still experience premature loss of loved ones as progress is made, and for those with health challenges that take away their enjoyment of life. But I find comfort in knowing that the prognosis for people with Fabry disease overall is brighter.

Also, thank you to my NFDF team members, Casey McKenna and Hannah Prince Sawyers, for their expertise, creativity, hard work, service to our community, and dedication in all the things we have accomplished. We have a great team!

As we say goodbye to 2025, we are excited about the possibilities of 2026.

We wish everyone a Happy New Year and much joy, happiness, and success in the coming year!

Best Wishes,
Jerry Walter



National FABRY DISEASE Foundation

Looking Back at 2025

WORLD Symposium

Jerry Walter and Casey McKenna attended the WORLD Symposium, where they attended lectures about Fabry disease research, met with community advocates, and members of industry from around the globe. Several members of the NFDF's Medical Advisory Board were also at the WORLD meeting, and in some cases, platform speakers: Dr. Robert Hopkin, Dr. Eric Wallace, Dr. Nicolas Abreu, and Dawn Laney, MS, CGC. We are working hard to stay up-to-date about advances in the management and treatment of Fabry disease.

Fabry Disease Awareness Month

Thank you to everyone in the community for participating in Fabry Disease Awareness Month activities! Whether you read our emails, liked our social media posts, or shared with your community, we hope our "Did You Know?" campaign was engaging and educational.

April was proclaimed Fabry Disease Awareness Month by the majority of state governors! The NFDF coordinates these efforts, but the real legwork is undertaken by the individual volunteers in each state who submit the requests. A special thank you to every community volunteer for your hard work!

Photos of proclamations and community members are located here: https://tinyurl.com/FDAM2025

NFDF 20th Anniversary

The National Fabry Disease Foundation was established in June 2005 by Jerry Walter. Many years and numerous successes later, we have a thriving, multifaceted program that supports the Fabry community in many ways. This summer, we celebrated 20 years of service, supporting individuals with Fabry disease and their families. In the next 20 years, we will continue to develop new resources to empower individuals with Fabry disease to live better and longer lives!

Look at how far we've come, together:

2005	to	2025
One FDA approved Fabry specific treatment	⟶	Three FDA approved Fabry specific treatments
Incidence 1:50,000 males with Fabry	⟶	Incidence 1:5,000 births with GLA gene variants
Females were considered carriers of Fabry	⟶	Females are AFFECTED with Fabry disease
Gene therapy is a far-off possibility	⟶	Multiple gene therapy trials are ongoing
The NFDF employs its first staff member	⟶	The NFDF employs three staff members
The NFDF provides its first resources	⟶	The NFDF provides hundreds of resources

The Fabry Market

For years, the National Fabry Disease Foundation (NFDF) has provided T-shirts for attendees of our annual conference and camp. We are pleased to make the classic *COURAGE*, *RESILIENT*, and *I Define Me* T-shirts available to everyone in the wider Fabry disease community! This platform also allows us to offer an extended catalog of NFDF items, including bags, hats, and cold-weather clothing

Visit The Fabry Market here: https://tinyurl.com/FabryMarket

Fabry Family Education Conference

The National Fabry Disease Foundation hosted its 15th Annual Fabry Family Education Conference in Greensboro, NC, on October 16-17, 2025! We were able to host 159 adults and children with Fabry disease and their families.

On Thursday, attendees participated in focus groups, community activities, and a large exhibition of representatives from healthcare, patient support services, and industry. Community members were able to select their Fabry disease awareness materials, including a preferred t-shirt, symptoms presentation, playing cards, awareness ribbons, wristbands, wallet cards, and more. There were interactive exhibits such as the "Where are you from?" map, temporary tattoo station, and selfie station!

On Friday, we started bright and early with a Fabry disease overview presentation by Dr. Hopkin. Next came Dawn Laney, MS, CGC who presented about early childhood symptoms of Fabry disease, followed by Dr. Staci Kallish who presented about managing your health in adulthood by Dr. Staci Kallish. In the afternoon, Dr. Jefferies presented the cardiology lecture, Dr. Mehta presented the nephrology lecture. Representatives from Sanofi, Chiesi, Amicus, Sangamo, and Uniqure provided community updates. Adults participated in a community sharing session led by Jerry Walter, and children participated in “chat groups” led by trained genetic counselors.

Thank you to our healthcare and industry speakers, community and industry volunteers, our conference childcare workers from Jovie, the Embassy Suites hotel, and our tremendous sponsors Sanofi, Chiesi, Amicus, Sangamo, Uniqure, and other exhibitors!

Conference presentations from healthcare providers are available to view on our YouTube channel: https://www.youtube.com/thenfdf. Conference photos are available to view online on Facebook page and our conference website.

Fabry Family Camp at Victory Junction

The National Fabry Disease Foundation then hosted its 14th annual Fabry Family Weekend Camp at Victory Junction Camp in nearby Randleman, NC!

We were pleased to host 32 families with Fabry disease, completely filling Victory Junction's cabin allotment! There were over 50 volunteers across the Fabry community, healthcare, and industry. Families sang and danced after breakfast and dinner, caught (and kissed) many fish, shot archery, went bowling, made arts and crafts, got makeovers at the Fab shop, and even went on the zip line! On Saturday evening, Victory Junction hosted a talent show, and Fabry community members absolutely rocked the stage!

The camp photos are available to view on Facebook and our 2025 conference website. The Victory Junction video is viewable on our YouTube page. Fun fact: The dancing zebra in the camp video is our long-time camp sidewalk consultant, Dr. Rob Hopkin. Go, Dr. Rob! Dr. Staci Kallish joined Dr. Rob as a sidewalk consultant this year. Don't miss the photo of her kissing a fish at the camp's Catch, Kiss, and Release Marina.

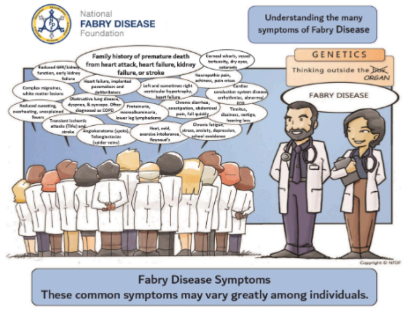
NFDF Webinar Series

The NFDF hosted five virtual education meetings in 2025.

1. Understanding 504 Plans with Katherine Graves
2. Family Planning Options with Kristen Nunez, MS, CGC
3. Cell Therapy with Glafabra with Chris Hopkins, PhD
4. Amicus Fabry Champion Panel with Sherrita Dorsey, MSN
5. Chiesi Total Care Program with Mike Clavadetscher

Videos are published on our YouTube channel once edited and approved: https://www.youtube.com/thenfdf

Other Educational Resources



The National Fabry Disease Foundation continues to add to its extensive library of YouTube recordings (www.youtube.com/thenfdf) and its robust educational website (www.fabrydisease.org).

The NFDF is pleased to continue its partnership with Bionews, Inc., the parent company of Fabry Disease News, to help further the mission of ensuring that patients and caregivers get the support, assistance, and information they need to help them in their daily lives. In case you missed it, Jerry Walter published three new articles in his column for Fabry Disease News, called Fabry: A Road Less Traveled.

The United States Fabry community is fortunate to have two complementary support organizations providing a diverse set of programs and services. Learn more about the Fabry Support & Information Group (FSIG) and their resources: https://fabry.org/

The US Fabry community is also supported by educational opportunities from the UCLA Core Kidney team. They host monthly "kidney chats" with expert speakers and community interaction every month in the evenings. The next meeting is January 10, 2026. Learn more: www.uclahealth.org/programs/core-kidney

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Support Resources

Kathleen Greer Association (KGA) offers cost-free counseling, referrals, education, and support services to U.S. and Canadian Fabry communities through a new NFDF/CFA collaboration. The 100% confidential services available to individuals with Fabry and their immediate family members include 24/7 access to a network of experts at 800-648-9557 for mental and emotional health and family, home, and work support resources.

When using KGA services, use "**fabry**" as the company code. Learn more at: https://www.my.kgalifeservices.com/

Industry Updates

sanofi	Sanofi recently completed a study evaluating its enzyme replacement therapy, Fabrazyme, infused over a shorter period of time in its SHORTEN study. Sanofi continues to research the use of Venglustat, an oral substrate reduction therapy, in adults with Fabry disease. The PERIDOT study focuses on pain and the CARAT study focuses on heart disease. Neither is recruiting.
Chiesi global rare diseases	Cheisi recently announced the approval of its enzyme replacement therapy, Elfabrio, for use in adults with Fabry disease in Canada by Health Canada. Chiesi continues to research the use of Elfabrio in children and adolescents with Fabry disease in the FLY study, which is currently recruiting.

	In 2025, Chiesi requested approval from the European Medicines Agency for an alternative dosing schedule of Elfabrio, with infusions every 4 weeks. The EMA rejected the proposed regimen in October, and Cheisi later announced plans to challenge the ruling.
Amicus Therapeutics	Amicus' oral chaperone therapy, Galafold, was approved for use in children age 12 and up in Europe. In the United States, Amicus has completed its ASPIRE study to evaluate its use in children ages 12 and up, but has not yet been approved for use in children by the FDA. Amicus has an ongoing clinical trial assessing the use of Galafold in very young patients aged 2-12. Amicus has recently announced that it has entered into a definitive agreement to be acquired by Biomarin. The full press release is available HERE. Your contacts at Amicus will remain the same.
Sangamo THERAPEUTICS	Based on the results of its STAAR study, Sangamo has initiated the rolling submission of their Biologics License Application (BLA) with the FDA seeking accelerated approval for its investigative one-time gene therapy, ST-920. This study is not currently recruiting.
uniQure	UniQure is currently recruiting adult males with Fabry disease to participate in a clinical trial of its investigative one-time gene therapy, AMT-191.
4DMT	4DMT continues to evaluate its one-time gene therapy, 4D-310, in adults. This study is not currently recruiting.
Glafabra Therapeutics	Glafabra is developing a repeatable-dose cell-based gene therapy for Fabry disease using Live-cel™ technology. Based on results from the Canadian FACTs trial, Glafabra aims to develop a treatment that needs to be administered every 3-5 years. Email Dr. Chris Hopkins at chris@glafabra.com to learn more.

There are many ongoing clinical trials assessing the effectiveness of a proposed treatment in improving the health of individuals with Fabry disease. If you are interested in participating in a clinical trial, please talk to your Fabry specialist. Learn more at www.clinicaltrials.gov

Looking Forward to 2026

2026 Webinar Series

We plan to continue our virtual educational meetings in 2026! Topics will include: Fabry disease management, women's issues, biomarkers, and more. This year, we plan to incorporate new publications to update the community about emerging topics.

https://sites.google.com/fabrydisease.org/nfdfwebinars/home

Planning for Medical Expenses

Are you worried about out-of-pocket medical costs in 2026? The NFDF is in contact with the five primary financial assistance programs available to individuals with Fabry disease that may be able to help.

We have compiled a chart comparing eligibility requirements and available services across programs. Programs may be able to assist with out-of-pocket costs for insurance premiums, medication copays, specialist visits, travel, and more if related to the management of your Fabry disease. Income limits vary, and can be as high as 700% of the federal poverty limit.

This resource details 2025 program information and will be updated once 2026 details become available. https://www.fabrydisease.org/images/ReferencePDFs/2025-Assistance-Programs-5-28-2025.pdf

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Fabry Disease Awareness Month

April is Fabry Disease Awareness Month! The National Fabry Disease Foundation aims to have a representative from every state request a proclamation of April as Fabry Disease Awareness Month from their governor. Please email cmckenna@fabrydisease.org to learn more or volunteer!

Do you have any plans to raise awareness about Fabry disease in April? Please reach out, we'd love to support you!

Save the Date for 2026 Conference & Camp

We are pleased to announce that the 16th Annual Fabry Family Education Conference will be held on September 17-18, 2026, once again at Embassy Suites in Greensboro, NC, with travel on Wednesday, September 16th, for many.

We are pleased to announce that the 15th Annual Fabry Family Camp at Victory Junction Camp will take place on September 18-20, 2026. Applications will be made available in the Summer of 2026 for families with a child with Fabry disease who will be age 6-16 on the date of camp. Priority is given to families who are first-time attendees.

Our Supporters

Industry Support

Thank you to our Pharmaceutical Industry and other business sponsors who, through their charitable giving programs, enable the National Fabry Disease Foundation to provide resources, programs, and services to people with Fabry disease and their families. Industry contributions provide the starting point of our annual budget to accomplish our mission.

In the order of the amount of their generous support are:

Overall annual program, annual Fabry family education conference, and annual Fabry family weekend camp.
- Sanofi
- Chiesi
- Amicus

Annual Fabry Family Conference
- Sangamo
- Unique
- Glafabra
- Data Registry Services
- PAN Foundation

Thank you also to the hundreds or individuals and smaller organization donors who add to the larger industry/company sponsor's support to make all our programs achievable and successful. We will provide a more detailed report of our 2025 donation soon.

We are grateful for your tremendous support to give people with Fabry disease a chance to live better and longer lives along with better family lives!

Community Support

While we are very grateful to our industry sponsors for providing the bulk of our annual funding, we are especially thankful to our community members and other non-corporation supporters for helping to fund our programs and services and to help meet our IRS compliance requirements as a nonprofit charitable organization.

As a nonprofit organization that serves a geographically dispersed rare disease community, the IRS requires that the NFDF fundraise 10% of its annual funding from the public, which is a high bar for us to meet. We need help from the community not only to provide our many programs and services to the Fabry community, but also to maintain our nonprofit status!

Unfortunately, due to insufficient funding this year, we had to decline 40 individuals' annual conference applications in October. With additional funding, we hope to accept all 2026 applications and continue our many other Fabry community programs.

Please consider the National Fabry Disease Foundation in your charitable giving plan throughout the new year. We can’t do it without everyone's help! Please see our Ways to Give handout for more information.

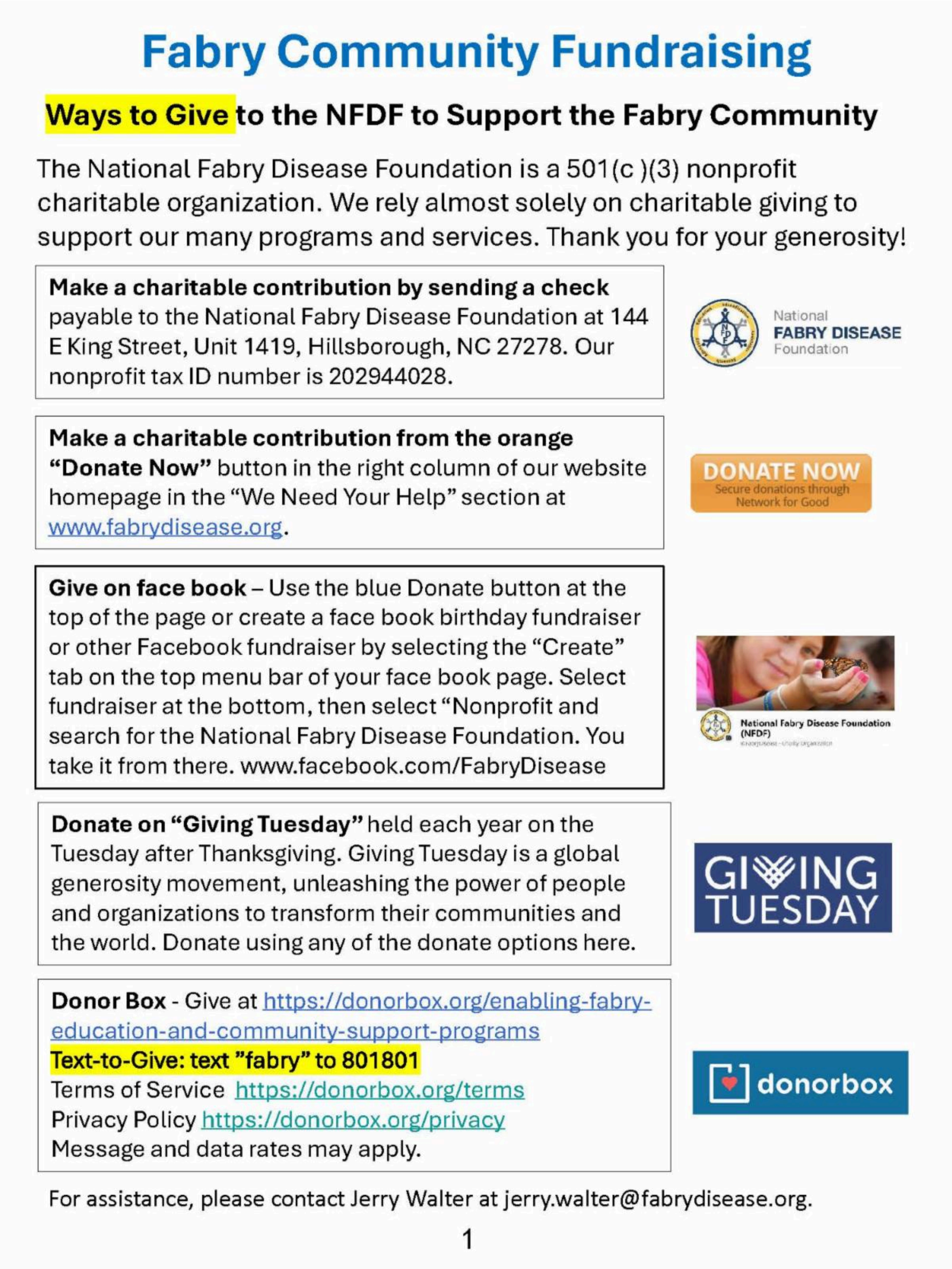
If you reside outside the U.S., please make charitable contributions to the Fabry support organization in area!

Thank you for your consideration!

Shining Star Campaign


Star light, star bright, The first star I see tonight; I wish I may, I wish I might, Have the wish I wish tonight
Whatcha doin'?
Making a wish!
For what?
For a future!
Why?
Because I have **Fabry Disease**
What happens to kids with Fabry Disease?
A lot of stuff happens! We have a lot of pain, and we don't sweat when we get really hot.
And we have a really hard time doing physical activity or being in hot tempuratures.
And we have bad stomach problems. And we get high fevers for no reason...
And sometimes we miss a lot of school... And for the rest of our lives we have to get an infusion with a needle every two weeks that takes a long time. I don't like it very much but it helps.
That sounds really bad! What happens when you get older?
We don't always get to be really old like other people.
Written by Jerry Walter
A lot of the time, when we are 30 or 40 or 50 years old, we have worse pain and fatigue, and we have kidney failure, heart attacks, strokes, and lung disease, and we lose our hearing, and a lot of people die before they're very old.
It makes me scared to get old, but it's not much fun being a kid either!
I'm reall sorry! How can you get a better future?
Illustrated by Michael Johnson
We really need help to improve recognition and diagnosis of Fabry Disease so people havea chance to get treatment to live longer and better lives.
And we need help to find a cure!
I sure hope You can find some help!
There are millions of stars out there. Maybe we can find a bright star for me and for everyone with Fabry Disease.
That sounds great! Then we can be friends for a really long time.
Are you a shining star to help us give people with Fabry Disease a better life and a brighter future?
Please make a charitable controbution to the National Fabry Disease Foundation at **www.fabrydisease.org**
With treatment available, kids shouldn't have to live a poor quality of life and adults shouldn't die young because of Fabry Disease!
Be the Brightest Star!
Please give generously.

Ways to Give



Fabry Community Fundraising

Ways to Give to the NFDF to Support the Fabry Community

The National Fabry Disease Foundation is a 501(c)(3) nonprofit charitable organization. We rely almost solely on charitable giving to support our many programs and services. Thank you for your generosity!

Make a charitable contribution by sending a check payable to the National Fabry Disease Foundation at 144 E King Street, Unit 1419, Hillsborough, NC 27278. Our nonprofit tax ID number is 202944028.

Make a charitable contribution from the orange "Donate Now" button in the right column of our website homepage in the "We Need Your Help" section at www.fabrydisease.org.

Give on face book – Use the blue Donate button at the top of the page or create a face book birthday fundraiser or other Facebook fundraiser by selecting the "Create" tab on the top menu bar of your face book page. Select fundraiser at the bottom, then select "Nonprofit and search for the National Fabry Disease Foundation. You take it from there. www.facebook.com/FabryDisease

Donate on "Giving Tuesday" held each year on the Tuesday after Thanksgiving. Giving Tuesday is a global generosity movement, unleashing the power of people and organizations to transform their communities and the world. Donate using any of the donate options here.

Donor Box - Give at https://donorbox.org/enabling-fabry-education-and-community-support-programs
Text-to-Give: text "fabry" to 801801
Terms of Service https://donorbox.org/terms
Privacy Policy https://donorbox.org/privacy
Message and data rates may apply.

For assistance, please contact Jerry Walter at jerry.walter@fabrydisease.org.

Connect with Us

TheNFDF

The National Fabry Disease Foundation

We've made it easier to access NFDF resources online!

Visit our LinkTree for quick links to many of our key programs, connect with us on social media and sign up for our email newsletter!

https://linktr.ee/thenfdf

- Facebook
- Instagram
- TikTok
- Twitter (X)
- YouTube
- LinkedIn

- NFDF Website
- Sign up for our Newsletter!
- NFDF's YouTube Video Index
- Educational Symptoms Presentation
- Fabry Facts Playing Cards - Answers to FAQs
- U.S. Primary Financial Assistance Programs
- Please help us to find people with undiagnosed Fabry... YouTube · TheNFDF
- NFDF Family Assistance Program (Code: fabry)
- Mental Health Resources
- Sponsored (cost-free) U.S. Genetic Testing
- 2025-2026 NFDF Webinar Meeting Registration
- The Fabry Market
- Jerry Walter's Fabry Disease News Column

View the 2025 Conference and Camp website, including this year's photo album:
https://sites.google.com/fabrydisease.org/familyconference/home

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