The Barth Syndrome

ADVOCATE

2026 Newsletter



Barth Syndrome Foundation®

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From the Desk of the CEO

Dear Barth syndrome community,

It was wonderful to see so many of you at Conference in July.

Conference is a cornerstone event that brings our families, researchers, volunteers, and healthcare providers together in person, and that togetherness is the heart of the Barth Syndrome Foundation and our global community.

Together we are synergistic, creating knowledge, generating energy, and building vital resources. Together, we help each other to build a world where everyone affected by Barth syndrome can live the life they choose.

Our Fall 2026 BSF Newsletter aims to share our most important updates with you: a celebration of Conference; exciting news about our patient registry and repository, BarthINSIGHTS; our partnership with Mito Foundation to support Australian families; and our newly unveiled three-year strategic plan.

We are honored to do this work and proud to share it with you.

-Emily Milligan















Coming Together

for the 2026 Conference

In July, we gathered for the biennial Barth Syndrome International Scientific, Medical, and Family Conference, which we lovingly refer to simply as "Conference."

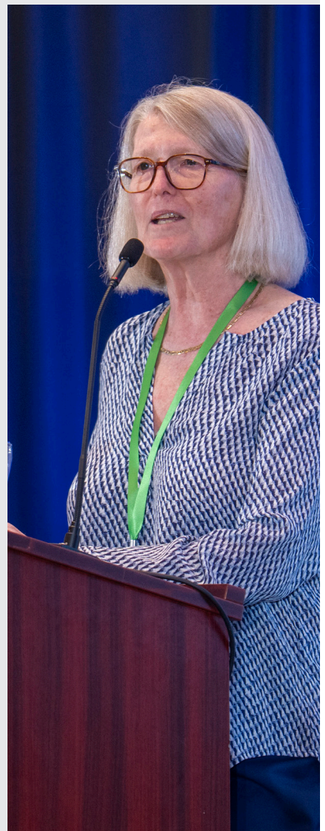












Emily Milligan, BSF's Chief Executive Officer, opened this year's Conference for the 300+ attendees with an intention for the week ahead: "We hope this week brings you replenished energy, new ideas and foundational knowledge, and the opportunity to connect with our beautiful community."

Conference is the only gathering of its kind. It is unique because it brings together critical groups of people: affected individuals and their families and caregivers, researchers and healthcare providers, and the industry professionals who help bring treatments all the way to patients. We believe that when these groups learn from each other, they create progress not just for families affected by Barth syndrome, but more broadly for the rare disease community, where each step forward is hard-earned and each milestone is celebrated to its fullest.

This year, Conference was particularly special: it marked the first gathering since the FDA's approval of FORZINITY™, the first-ever treatment for Barth syndrome.

"This Conference, more than any other, has given me a little more hope for a better future for [my son] Wyatt," said Amanda, a mom who attended her eighth Conference this year.

We heard from Dr. Emil Kakkis, founder and CEO of Ultragenyx Pharmaceutical and a pioneer in rare disease drug development, for our keynote address. He detailed the ways in which regulators can support rare disease drug developers to advance more treatments for conditions like Barth syndrome over the finish line.

We celebrated Dr. Hilary Vernon, Director of the Mitochondrial Medicine Center at Johns Hopkins Hospital, and awarded her with BSF's highest honor, the 2026 Varner Award for Pioneer in Science and Medicine for her dedication to our community.

Programming is as varied as our community itself. Multiple tracks run side-by-side, so that every attendee can find the sessions that matter most to them. Families find their people in our sessions on care management. Wellness programming gives everyone a break. Camp Barth lets kids be kids while their families are in session. Affected individuals can easily participate in on-site research and clinical studies that would be challenging to travel for otherwise, and their participation advances science for everyone.

More than anything, Conference is about being together.

"At times, this journey can feel lonely, but it doesn't need to be," said Tracy Brody, a mother of two individuals with Barth syndrome. 2026 was the Brody family's sixth time attending Conference. "We are so grateful to have incredible people walking alongside us throughout every stage, every age, every struggle, and every triumph."

Becoming Part of the Fabric of the Community:

a Researcher's Perspective

By Sree Venigalla, Researcher, University of Minnesota

I've been studying about how Barth syndrome affects the heart cells in Dr. Christina Pacak's lab at the University of Minnesota since 2021.

At the 2026 Barth Syndrome Conference, my work with Barth syndrome took a whole new view: I became part of the family that is the (figurative) heart of the Barth syndrome community. From my vantage point at the research table, I spent five days right in the middle of families, clinical research, activities, connections, and learning.

While I helped affected individuals get to their appointments and made sure the clinicians had everything they needed for their studies, I watched the community welcome new families and catch up with one another and researchers like they were greeting old friends.

I watched how the work I do in our lab every day has the ability to transform patients' lives.

The air buzzed with conversation, excitement, connection. Over breakfast, researchers uncovered connections between projects that, for one week, were happening right down the hall from one another. We talked about the extracellular matrix and mitochondria – sparking questions and ideas that are energizing our research in new ways.

I attended my first Conference in 2024, where I stayed only for two days while I presented my poster. This year, I showed up to the fullest opportunity – as a volunteer and a researcher, I felt like part of the fabric of the community. I stepped way out of my comfort zone at the Friday night social, singing my paper's abstract in front of the whole room to the tune of Twinkle Twinkle Little Star, raising $200 in Conference donations as part of the fundraising "dare board."

These seven days at the Conference strengthen the Barth syndrome community and expand the science.

I can't wait for 2028.



"*I watched how the work I do in our lab every day has the ability to transform patient lives.*





We Were Never Meant to Do This Alone

By Claire Sandahl, Barth Syndrome Mom

When Rilo was born, everyone told us he was healthy. Then, without warning, he went into heart failure and ended up on extracorporeal membrane oxygenation (ECMO) – life support. Genetic testing revealed Barth syndrome, and our medical team at Stanford moved fast getting him into a compassionate use program [for elamipretide] and coordinating excellent care.

But even with great doctors, I still felt alone. No one in our everyday lives had heard of Barth syndrome, and well-meaning advice from friends and family rarely applied to our reality—especially around feeding and nutrition, where even professionals offered textbook guidance for a kid who didn't exist. I wanted real-world experience from parents who'd stood where I was standing.

By the time the 2026 Barth Syndrome Conference came around, I was exhausted and desperate for community. I was also scared—joining a room full of families who live your worst fears and deepest hopes isn't easy.

A pre-Conference Zoom for first timers helped me know what to expect, but there's no real way to prepare for that moment.

And yet, from day one, my overwhelming feeling wasn't fear. It was belonging.

Everyone we met—staff, longtime families, other first-timers—was kind and unpretentious. I finally hugged, in person, a family Stanford had connected us with months earlier by text. We met more families with young kids in Rilo's age range and started a group text that's become one of the most powerful outcomes of the Conference: a place to ask if anyone else has gone through this, share appointment anxieties, and celebrate small wins only another Barth syndrome parent would understand.

Experts like Drs. Hilary Vernon and Stacey Reynolds sat with us, listened, looked at our kids, and told us to email them anytime. I'd expected layers of referrals; instead, I found brilliant people willing to step into our messy reality with us.

The emotional heart of the Conference was sitting in a room full of mothers facing the same rare condition. Repeatedly, someone would ask,

"

Has anyone else felt this way?

—and the answer was always yes. It didn't fix anything, but it lifted a weight I didn't know I was carrying.

A Message to Other Families

If you're scared to go, go anyway. You'll cry, you'll see hard things, but you'll also find people who hold your story with you and tell you the truth. If money is holding you back, ask about support—the experience is worth far more than the cost.

To the donors, staff, and longtime families who make this possible: thank you. You've turned what could have been an unbearably lonely journey into one we don't have to walk alone.







Together

26 Years & 10 Conferences

By Kevin Baffa, Affected Individual

2026 was my tenth Barth Syndrome Conference.

In 2000, I was a kid meeting other Barth syndrome kids for the first time as our parents talked through the beginnings of what would become the Barth Syndrome Foundation. In 2002, we hosted the first formal Conference, and we've come together every other year ever since except 2020 and 2022, when COVID made it unsafe for us to travel and gather in a large group. Personally, I've only missed these events twice in 26 years.

I'm a regular on the bi-weekly Barth syndrome adult guys Zoom call, which gives me the opportunity to spend time virtually with some of the Barth syndrome community. But Conference is the only time we are together in person, and it is the only time that our entire community comes together.

That makes all the difference. These moments in person, while we learn about the latest research or catch up at breakfast, turn familiar names and faces into lasting friendships. These moments with our researchers – many of whom, like Dr. Taylor and Dr. Cade, I've known for decades – remind me that there are people devoting their professional careers to helping me and my Barth syndrome family live longer, fuller lives.

Every Conference has its own character, shaped by its location and the people who attend. When I was younger, the pool was the nightly gathering place. This year, some of my favorite memories came from relaxed evenings hanging out with the other Barth syndrome adults —playing poker and catching up without an agenda.

Conferences bring us together, strengthen our community, and remind us that no one has to navigate Barth syndrome alone.

Don’t Wait:

What Our First Barth Syndrome Conference Taught Us About Family

By Jamie Loe, Barth Syndrome Mom

To any family on the fence, especially if you’re newly diagnosed: yes, it will be overwhelming, but it’s balanced with knowledge and connection. Don’t put it off. *You’ll find your family.*

When our son Grayson was diagnosed with Barth syndrome during the COVID-19 pandemic, survival and stability became our only goals. Traveling to Conference in 2024 felt out of reach, so we stayed home. We watched photos roll in and wondered if it was worth the risks and hassle of traveling with a young child with medical needs.

This year, we finally said yes, and walked into a reunion for a family we hadn’t known we were part of.

Barth syndrome families came from all over the world and brought different backgrounds, yet everyone there got it: the fatigue, the hospitalizations, the small victories that only the Barth syndrome community understands.

We were able to cut out the part of the conversation where we explain the basics of Barth syndrome and Grayson’s limitations, which can feel like they’re invisible to people who haven’t experienced Barth syndrome. He can run at full speed and look “fine” – but no one sees him crash afterward. At Conference, we never had to explain that. Other families already knew.

As first-timers, we were paired with a mentor family who we ended up meeting in the elevator on the way in. We shared meals, our kids swam together, and when Grayson had a reaction to a bug bite, they checked in. What could have been overwhelming became warm and manageable.

We participated in age-based classes that were part support group, part strategy sessions. Parents of children around Grayson’s age talked openly about medications, school accommodations, and navigating the healthcare system. No one minimized the hard parts, and no one let us face them alone.

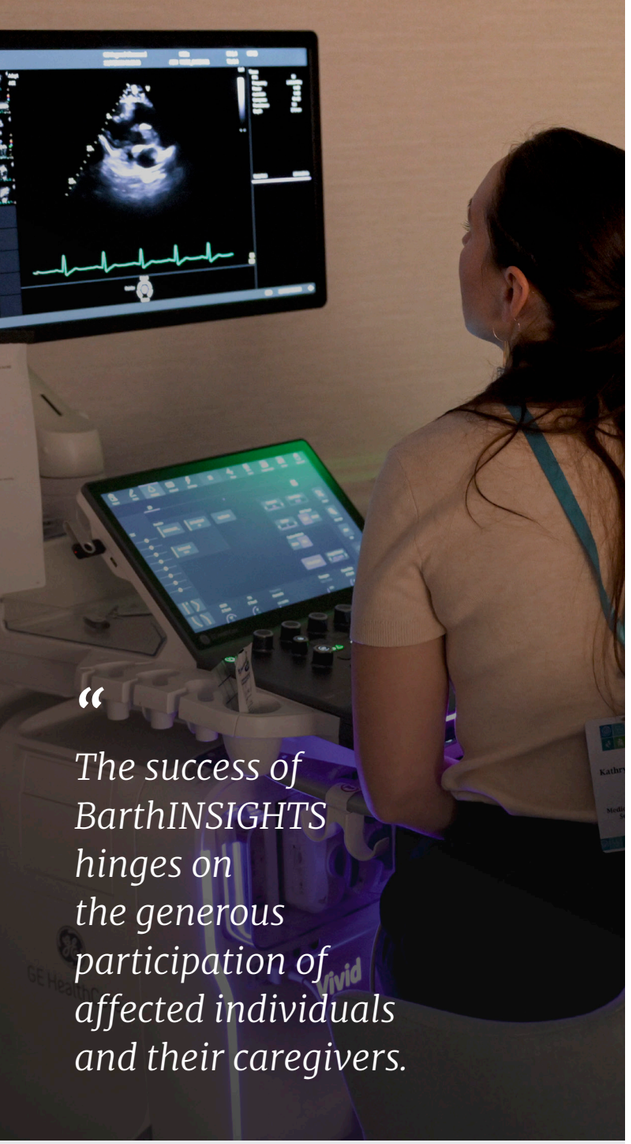
Our daughter Avery is twelve and carries more emotional burdens than many adults. At Conference, she had space to be a kid. While she stayed close to me during the first day, the dedicated sibling spaces gave her peers who understood what it’s like to love fiercely while sometimes feeling invisible. By the second day, she was running off to sibling sessions, saying, “See you later, Mom.”

At Conference, we saw how much the scientists working on Barth syndrome research care about the people and families affected by this disease. When we read about Conference research from home, these researchers and clinicians felt distant and removed from the day-to-day of our lives. But at Conference, we met them and heard from them firsthand.

Seven-year-old Grayson started seeing himself as part of something bigger. After meeting his “Barth buddies” who were already participating in studies, he was inspired. He asked to join a heart-monitor study and start G-CSF shots, decisions we’d held off on until he was old enough to weigh in himself. We had the privilege of watching him choose to help himself and others.







“The success of BarthINSIGHTS hinges on the generous participation of affected individuals and their caregivers.



Barth INSIGHTS
Registry and Repository

The Barth syndrome community has a new way to stay connected to medical and scientific insights: the BarthINSIGHTS Registry and Biorepository.

BarthINSIGHTS is the new name and home for the Barth Syndrome Registry & Repository, the most comprehensive global patient registry and biorepository for Barth syndrome. It collects data about our community’s experiences, symptoms, and disease progression in a secure database. When viewed together, families’ collective experiences can shape the research about them, inform regulatory pathways for drug development, and understand how their symptoms fit into the broader context.

This is not the first time the Barth Syndrome Foundation has created a virtual space for individuals with Barth syndrome to share their lived experiences or donate blood and tissue samples that can be used for research, rather it is the next evolution.

The success of BarthINSIGHTS hinges on the generous participation of affected individuals and their caregivers. To date, over 180 affected individuals have helped advance our understanding of Barth syndrome by contributing to the registry and biorepository. To honor their commitment to the registry, we’ve been working tirelessly to make the experience smoother and more accessible and to ensure participants feel their time has been valued.

“We recognized both the value that a registry like this could provide to our community and the challenges we faced with our former version, the Barth Registry and Repository 2.0. BarthINSIGHTS lives on a new technology platform with more advanced capabilities, all with the primary goal of making it easier for families and researchers to use the tool,” shared Melissa Huang, Barth Syndrome Foundation’s Research Associate, who oversees BarthINSIGHTS.

Now, surveys can be completed in small chunks and participants know ahead of time how many questions each survey will be. Users can see how their own data trends over time. Eventually, many users will be able to directly connect their electronic medical records should they choose to do so. This allows the scientific community to view patient voice and clinical experiences all in one dataset. All of these features also make it a little easier for individuals with Barth syndrome and their caregivers to maintain their BarthINSIGHTS records and see the value for themselves. Since its new launch, 60 affected individuals have already enrolled onto the new platform.

Enroll Today

BarthINSIGHTS has been made possible by a five-year, $800,000 grant from the Biohub (formerly Chan-Zuckerberg Initiative) Rare As One program.

About Biohub

Biohub builds the technology to help scientists around the world use AI-powered biology to study how cells operate, organize, and work as part of systems to understand why disease happens and how to correct it. With unprecedented scale of computing AI research and engineering, and state-of-the-art technology for measuring, imaging, and programming biology, Biohub is leading the first large-scale scientific initiative combining frontier AI with frontier biology.

Barth Syndrome Foundation®

Strategic Plan

2026–2028

The Barth Syndrome Foundation board and staff are excited to share the new mission and vision that were recently adopted as part of the 2026–2028 Strategic Plan.

For the first time, there is an approved therapy for Barth syndrome. This changes what our Foundation can do and what we see as possible. This landmark calls for language that reflects our growth. BSF remains the same at our core, but we are entering a new chapter.

Three things about the FORZINITY™ approval are true at the same time:

1. Barth syndrome is now among the 5% of rare diseases with an approved treatment – a major milestone for our community and for the Foundation.
2. Barth syndrome is a complex disease that presents in different ways for different people, and BSF is committed to building a world where individuals affected by Barth syndrome can choose from a robust portfolio of treatments to address aspects of Barth syndrome that most affect them.
3. We need a consistent voice in the policy and regulatory spaces to continue this track record of success.

These truths guided our conversations with the community as we gathered input for our new mission and vision.

OUR VISION

The future we're building:

A world in which everyone impacted by Barth syndrome can thrive and live the life they choose.

OUR MISSION

The reason we exist today:

To collectively and tenaciously work toward saving and improving the lives of those impacted by Barth syndrome.





Over the next three years, we'll pursue our mission and vision by focusing on five things:

1
Protecting the Heart of the Community

Our community is growing globally and spans more generations, life stages, and disease presentations than ever before. We'll meet families and affected individuals with information they can trust and connections that cut through isolation. We will do this by launching a new BSF website, a family services model built to evolve as our community grows and changes, and hosting our next Conference in 2028.

2
Driving Toward Treatments and a Cure

The first drug approval for Barth syndrome showed what's possible for an ultra-rare disease when patient communities, regulators, and industry work together. We are building on that momentum — supporting the follow-on work from FORZINITY's 2025 approval, pushing for label expansion to younger patients, helping U.S. families navigate reimbursement and access, and continuing to advance the next generation of potential therapies.

3
Investing in Research

A strong research pipeline is our best long-term bet to build a future where people with Barth syndrome can live the life they choose. We'll keep funding the seed grant program that launches new ideas off the ground, work deliberately to close gaps in our scientific understanding, and ensure the tools — including our new BarthINSIGHTS registry and biorepository — that researchers depend on are there when they need them.

4
Advocating for the Community

Our relationships with regulators, legislators, and patient advocacy coalitions have produced real results, and we're not stopping. We'll keep those relationships strong and build new ones, develop the next generation of advocates, and keep showing up for families navigating access barriers.

5
Never, Ever Giving Up

Tenacity carried BSF from a handful of families looking for answers in 2000 to a global community with real influence. Getting to what's next takes real investment in the fundamentals — an effective fundraising program, smart use of new tools and technology, and strong volunteer leadership to carry this work forward for decades to come.

This plan is ours because you built it with us.

Thank you for the conversations, the input, and the tenacity that got us here.

We can't wait to show you what's next.





Partnerships

Across the Globe

The Barth Syndrome Foundation (BSF) is committed to supporting every person affected by Barth syndrome, no matter where they live. Through our partnerships with BSF affiliates in Canada, France, Italy, and the UK, we continue to strengthen our global community and provide geographically relevant support to families.

As part of this commitment, we are proud to announce a new partnership with the Mito Foundation, Australia’s leading umbrella organization for the mitochondrial disease community. For families affected by Barth syndrome in Australia, the rarity of the condition can feel especially isolating without an established local support network.

This partnership represents an important step toward expanding access to resources, fostering meaningful connections, and ensuring families in Australia feel supported and connected to the broader Barth syndrome community.

Our partnership with the Mito Foundation creates that connection. Together, BSF and the Mito Foundation will provide Australian families with trusted information, Australian-tailored resources, peer support, and new opportunities to take part in research and advocacy. The Mito Foundation’s local expertise and on-the-ground support, paired with our deep expertise in Barth syndrome, will bring Australian Barth families one step closer to community and critical resources.

Our CEO, Emily Milligan, explains, “Barth syndrome affects families around the world, and the Barth Syndrome Foundation is called by our mission to support every one of them to the best of our abilities. Mito Foundation has built exactly the kind of trusted presence Australian families need. We’re grateful for their leadership in the mitochondrial disease community, and proud to work alongside them to serve our patient community in Australia.”

As the Mito Foundation CEO, Sean Murray, adds, “Families impacted by Barth syndrome in Australia should not have to face this rare condition alone. By working with the Barth Syndrome Foundation, we can connect families with trusted Barth syndrome knowledge and provide support that reflects the Australian health, disability, and research systems. This partnership helps us listen to families, respond to their needs, and advocate for improved access to clinical trials and new treatments in Australia.”

For Donna, who lives in Australia and whose son is affected by Barth syndrome, the news lands close to home: “When your family is affected by something as rare as Barth syndrome, it can be very isolating and feel like no one around you really understands what you are facing. Knowing that Mito Foundation and Barth Syndrome Foundation are working together gives us confidence that Australian families will have somewhere to turn for information, support, and connection. It helps us feel part of both a local community and a global Barth syndrome community.”

Families, clinicians, and researchers in Australia who want to learn more about Barth syndrome support and connection opportunities can contact the Mito Foundation for more information.

Mark Your Calendars

for Giving Tuesday

Our biggest fundraising event of the year!

The BSF Fundraising Team invites you to make a difference this Giving Tuesday by creating a personal fundraising page, making a donation, or rallying your friends, family, and community to support those affected by Barth syndrome.

Last Giving Tuesday, 43 community fundraisers raised more than $125,000 for BSF. This year, we're building on that success with a goal of bringing together 50 fundraisers to raise $135,000!

Your generosity makes a tangible difference. A $10 gift can help provide a Research ID card for someone affected by Barth syndrome, $100 can help send a care package to someone hospitalized, and $500 can help an advocate travel to Rare Disease Week. Larger gifts can help support a young scientist attending our International Scientific, Medical & Family Conference ($1,000) or provide hotel accommodations for a family attending ($3,000). Every contribution helps advance research, strengthen advocacy, and connect families with critical support and resources.

There's a way for everyone to participate! Create a fundraising page or make a gift through Givebutter using Venmo, Cash App Pay, Apple Pay, Google Pay, or a credit or debit card. Scan the QR code to get started, explore ways to give, or connect with the BSF Fundraising Team.

And there's no need to wait until Giving Tuesday! Set up your page or make a donation today and help us build momentum toward December 1.

Prefer to give by mail?

Please send donations to:

> Barth Syndrome Foundation
> PO Box 419264
> Boston, MA 02241-9264









Create Your Fundraising Page for Giving Tuesday











Barth Syndrome Foundation®

