Barth Syndrome Foundation of Canada

Canadian Newsletter

www.barthsyndrome.ca

May 2026

President's Report *by Susan Hone*

Welcome Spring!

I would like to update you on a few of the things Barth Syndrome Foundation of Canada have been participating in since the last newsletter.

In August of last year, we combined our annual planning meeting and family outreach into one weekend. Families gathered at a cabin in Northern Alberta on Friday afternoon. On Saturday, the Board and Executive reviewed our previous year, created the budget and brainstormed on program goals for 2026 which include:

- Support for affected individuals and Canadian researchers to attend the July international conference
- New family and information resource support
- A possible Family Outreach meeting in Ontario
- Increased research grant funding
- Continued fundraising, hopefully including some personal fundraisers

A recent research grant that BSF Canada is partially funding is showing some exciting possibilities. Canadian researcher Dr. Jason Moffat along with Dr. Blomen from the Netherlands have separately discovered that "turning off" a gene called ABHD18 may actually prevent Barth syndrome symptoms. The goal of the current research is to evaluate a class of drugs that may effectively block or "turn off" ADHD18 as a potential therapy for Barth syndrome. Dr. Moffat will speak at the Barth Syndrome Conference in July to discuss this research.

The drug Elamipretide now marketed as Forzinity, manufactured by Stealth BioTherapeutics, was conditionally approved on September 19, 2025, by the United States Federal Drug Agency. This is a major achievement in the pursuit of a treatment for Barth syndrome. As part of the conditional approval, more clinical studies will be done worldwide on Forzinity over the next two years. Canada will hopefully have a site or two chosen to participate in the clinical trials.

Our Annual General Meeting was held on May 21, with elections of our Board of Directors for terms of two years. Our current Board of Directors is:

Susan Hone	President
Lynn Elwood	Board Member
Marilyn Pridgen	Board Member
Christiane Hope	Board Member, Treasurer
Laurel Pridgen	Board Member

Jasmine Champagne has retired from the board. She has been a strong voice, an enthusiastic volunteer supporter and will be missed. We wish Jasmine all the best and welcome the new and returning members of the board.

To all our families, friends and supporters, I thank you and wish you all the best. Enjoy the upcoming warm weather.

Clinical Trial *by Jasmine Champagne*

Caleb is nine years old, yet he still cannot walk down a set of stairs on his own. When he goes up stairs, he must be accompanied because he becomes exhausted so quickly and often loses his balance. He cannot swim without wearing a lifejacket — not because he lacks ability, but because his fatigue is so severe that he needs the added safety when his body simply cannot continue.

Caleb cannot play tag with his friends. He is still learning how to run, and he cannot keep up. He has never been enrolled in organized sports because he does not have the stamina to last through a game, an inning, or even a short shift. He does not have the strength to hold a bat, throw a ball, or kick with force. Family walks or hikes are only possible if the path is wheelchair-accessible or if a family member can carry him in a backpack.

Barth syndrome has taken away many of the everyday experiences that most children take for granted.

Recently, a new medication called Forzinity was approved by the United States Federal Drug Agency (FDA), and individuals with Barth syndrome are beginning to see improvements in energy, fatigue, and muscle strength. Unfortunately, the medication is not yet available for children of Caleb's size or approved to be prescribed in Canada. His family has been discussing the possibility of enrolling Caleb in a clinical trial that would allow younger boys with Barth syndrome to access this treatment. The trial could last up to 18 months, requires daily injections, and there is a chance Caleb would receive a placebo for the entire duration.

After his parents explained everything as openly and honestly as they could, Caleb shared his own reasons for wanting to participate:

"There are two reasons I should do the trial. Number one — because it could help me. It could change me. I could play tag at recess. Number two — because I need to do it for my baby Barth brothers who might need it one day."

Barth syndrome has taken so much from this little boy — yet Caleb is already thinking about how he can do something big, not only for himself, but for every child who will walk this road after him.

Rare Disease Day *by Laurel Pridgen*

This year's Rare Disease Day was especially meaningful for our family, as I was invited to share our story at the Calgary event. Hosted by the Rare Disease Network of Alberta, I had the opportunity to speak with clinicians, researchers, and families about our journey from diagnosis to where we are today in navigating care and family life. The focus of the day was on how personal stories help shape better care in medicine.

It was incredibly encouraging to see such a well-established and passionate rare disease community in our city, including professionals who were already familiar with Barth syndrome.

Each time I share our story, I'm reminded that opportunities to connect and engage with others often create a ripple effect which sparks curiosity, conversation, and new possibilities. After my talk, several people approached me with questions and reflections that will likely lead to future collaboration and connection. I've even been invited to return and speak again, which feels like a meaningful and practical way for me to continue advocating in my world and sphere of influence. I am grateful to have had the opportunity and look forward to seeing what comes of the day!

Bringing passion together with fundraising

by Lynn Elwood

In the summer of 2025, Pamela and Lynn decided they wanted to help raise funds for the organization. For each of them in isolation it seemed a daunting task, but together they knew they could do it.

Over the summer and fall, Lynn made t-shirts with mental health sayings on them and cup holders to replace Tim's cardboard sleeves. Pamela worked on Christmas decorations and her educational books, lettering and sign language guides.

In late fall, we went to a local sale where we set up a table and did our best to sell our items. Thankfully selling is one of Pamela's many gifts. Thanks for her, our family and friends, and the local people who came to the sale, we were able to raise just under $400 as well as some awareness.

Pamela and Lynn's Fall Fundraiser.

Outreach 2025 *by Jasmine Champagne*

When you live with a rare disease or love someone who does as a parent, sibling, or grandparent, life can feel lonely, overwhelming, and at times, terrifying. It can feel as though no one fully understands what you are carrying or how deeply the disease shapes every part of your daily life.

Barth syndrome is a rare mitochondrial disease that affects approximately 300 individuals worldwide. Here in Canada, only 16 families are living this reality. As the mother of a 9-year-old boy with Barth syndrome, the opportunity to connect with others impacted by this devastating disease is something I struggle to put into words.

This past August, our Barth Foundation gathered for a summer outreach at Gull Lake, Alberta. From the west coast to the east coast, a handful of affected families came together for three days of connection and laughter at a beautiful home near the lake.

As parents of children with rare diseases, isolation often becomes part of life. The people around us love us, but they don't always see the full extent of how our lives have changed. Being together with other Barth families gives us the chance to share experiences that might help our children's specific needs. It gives us the space to cry about struggles that others may never witness, to laugh about G-tube mishaps and medication-travel chaos that only someone living this life would understand, and to celebrate victories that might seem small to others but are enormous to us.

Watching my son Caleb interact with his "Barth brothers," as he calls them, was something I will never forget. He has met some of them before, but now at nine years old he is far more aware of how his syndrome impacts his life and often separates him from his peers. For three days, he was surrounded by boys who are tube-fed like he is, who get regular injections and blood work, and who spend so much time in hospitals that it almost feels like a second home, just like him.

At one point, I stood in the kitchen watching Caleb and his Barth brother Jonathan snuggled together on the couch playing video games. They were casually talking about recent hospital admissions. At just seven and nine years old, I heard Caleb say, *"Oh, me too. I had an infection. What were you in for?"* As if it was a perfectly normal conversation.

For a little boy who carries such heavy things every single day, who tries to explain them to friends who can't fully understand, being able to talk freely with someone who truly knows what he is going through was absolutely beautiful.

Over those three days we played board games, did science experiments, drove tractors around the yard, played at the park, and went for boat rides. But more than anything, we connected with our Barth family.

And for that, we are deeply grateful.

Outreach 2025 *continued*

Jonathan and Caleb at Outreach 2025.

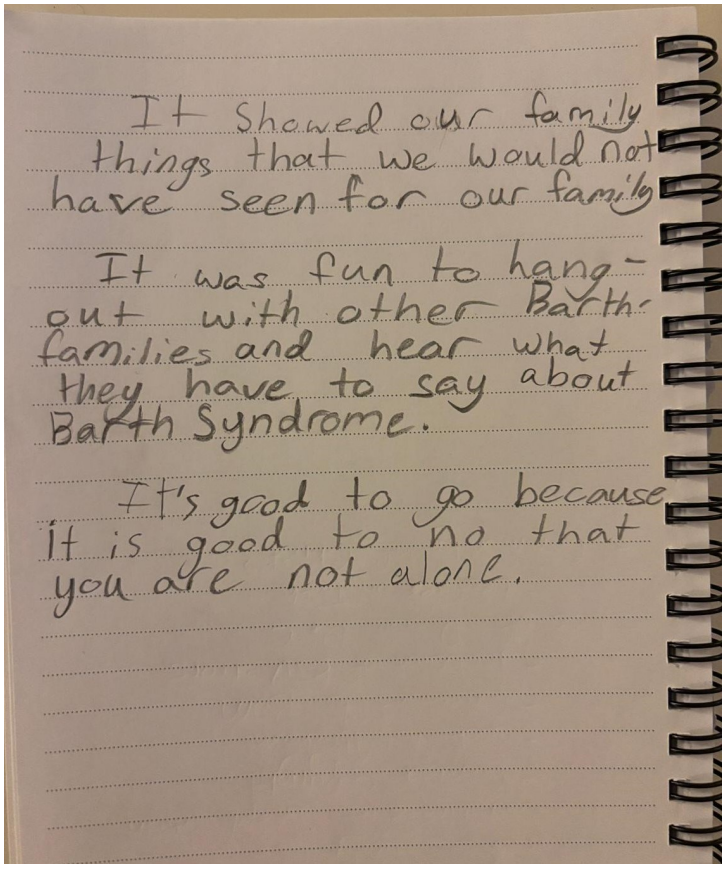
Outreach 2025.

Barth Syndrome 2026 Conference

The 2026 conference is fast approaching. People from across the globe come to this event, showing us how much stronger we are together. It is the one time affected individuals, caregivers, researchers, clinicians, and healthcare leaders get together in one place at one time.

For more information about the conference and registration information, please go to 2026 Barth Syndrome Conference. https://barthsyndromeconference.org/

Attending this conference is extremely helpful to those looking for answers and support, and we encourage Barth affected individuals to attend and participate wherever possible. To assist resident Canadian Barth individuals' attendance, BSF Canada is offering help with travel and lodging costs. For guidelines and information, please contact us at **info@barthsyndrome.ca.**



Memories of her first conference by H. Pridgen.

Previous Conference Pictures

Canadian Contingent at the 2024 Conference.

International Affected Individuals at the 2024 Conference.

Volunteers Wanted: Help Support our Community

We are looking for new volunteers who want to make a meaningful difference. Whether you're interested in learning more about Barth Syndrome, contributing professional expertise, or helping our organization grow, we would love to hear from you.

We are especially interested in individuals within Canada who have backgrounds in: **medicine, grant writing, fundraising, accounting, government advocacy or legal services**, as well as those who may be interested in **serving on our Board of Directors**.

Time commitments are flexible, and we are happy to find a role that fits your availability and interests. For those considering board involvement, the bi-monthly meetings are typically online, with one annual planning meeting that may be in person. The time commitment is approximately two hours a month.

If you are interested in getting involved or learning more, please contact us at **info@barthsyndrome.ca**.

The Barth Syndrome Foundation of Canada is dedicated to honouring our commitment to our donors and to Canadian individuals and their families affected by Barth syndrome by supporting much needed research, advocacy, education and responding to families' needs.

Because BSFCa is a volunteer-based organization, we know how to do a lot with a very modest budget. However, we still need your financial contribution to help us do our work. Whether you contribute $5 or $5,000, every single dollar moves us closer to more answers, more treatments, and especially more time for families.

Thank you for your generosity!

For donations, please visit

www.barthsyndrome.ca/donate

What is Barth Syndrome?

Barth syndrome is a rare, genetic disorder primarily affecting males around the world. It is caused by a mutation in the *tafazzi*n gene which results in an inborn error of lipid metabolism. This error causes, in various combinations and varying degrees: cardiomyopathy (disease of the heart muscle), neutropenia (an abnormally low count of a type of white blood cell that helps fight off infections), underdeveloped skeletal musculature and muscle weakness, and severe growth delay and exercise intolerance.

While much progress has been made in treating Barth syndrome, unfortunately, it still remains all too often a fatal disorder.

Going Green!

If you have any concerns or are interested in receiving our newsletter via email, please contact us at info@barthsyndrome.ca