



Barth Syndrome  
Foundation



# 2020

# ANNUAL REPORT

Saving lives through education, advances in  
treatment, and finding a cure for Barth syndrome

# Welcome

# A Message to Our Community

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Dear BSF Friend,

What will you remember about 2020? Never before have we collectively – globally – experienced so many “firsts.”

The first time we faced a novel virus that caused a pandemic. The first time our kids attended school on Zoom and stayed home for weeks (months!) without contact with other children. The first time a face mask became a normal and necessary accessory.

While these “firsts” resonate with many people, there are some aspects of 2020 that were not new for those living with Barth syndrome. People with Barth syndrome have always experienced some degree of social isolation. Our families often stay home due to the risk of life-threatening infection caused by neutropenia, a hallmark characteristic of the disease. Barth syndrome causes people to miss school or work, miss games or outings, avoid crowded places and be forced to plan multiple contingencies. After 2020, you probably have either experienced or heard about the debilitating fatigue that is associated with a coronavirus infection. Our children and adults with Barth syndrome experience often debilitating fatigue all the time, swiftly compromising their ability to live to their full potential. Our global new norm has always been the norm for people with Barth syndrome.

And yet, despite this, we at BSF never give up. As a global community, we stood together in solidarity more in 2020 than ever before. Rather than quit, we pivoted. Our longstanding and much anticipated international conference had to be postponed, so we hosted the first-ever Virtual Barth Syndrome Scientific and Medical Symposium, attracting more than 350 researchers, clinicians and family members from around the world. We got to know one another through weekly virtual roundtables, hearing from both expert speakers as well as members of our community about living with and managing Barth syndrome. We submitted a petition to FDA containing more than 4200 signatures and hundreds of powerful testimonials requesting access to potential therapies that may alleviate symptoms for some affected individuals. We learned that together we are “#BarthStrong,” and that together we really can change lives and change the world.

I hope that you and your family and friends have weathered the challenges of 2020, and I thank you for your generosity and support of BSF. You are a part of our global family, and everything that we do is made possible because of your unwavering commitment to our shared cause. We look forward to your continued support as we drive progress in 2021 across advocacy, research and cross-sector partnerships that will advance therapies for Barth syndrome.

In health and hope,

Emily Milligan,  
Executive Director



## OUR MISSION

Saving lives through education, advances in treatment and finding a cure for Barth syndrome

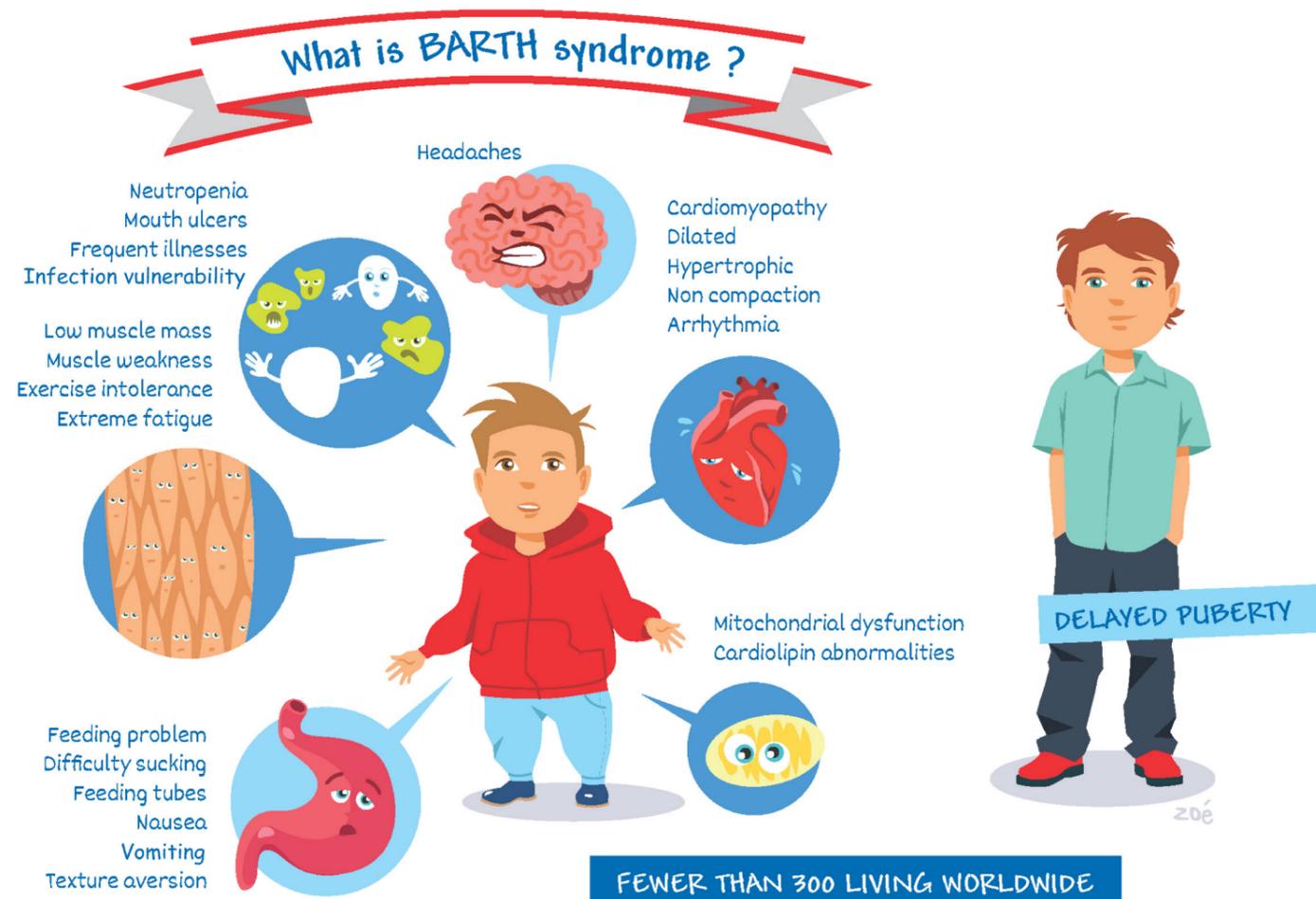
## OUR VISION

A world in which Barth syndrome no longer causes suffering or loss of life

## OUR CORE VALUES

Credibility | Integrity | Inclusion | Professionalism | Compassion

# What Is Barth Syndrome?



**Barth syndrome** is a rare, life-threatening, genetic mitochondrial disorder primarily affecting males. Affected people may suffer from heart failure, muscle weakness, and infection (caused by neutropenia). Additional characteristics of the syndrome commonly include growth delay, impaired lipid metabolism, extreme fatigue, and cardiolipin deficiency. In some people affected by Barth syndrome, the symptoms can be very severe, sometimes resulting in heart transplant, potentially lethal infections, and even death.

# Who We Are

**Barth Syndrome Foundation (BSF)** is the only global network of families, healthcare providers, and researchers solely driven by the mission to save lives through education, advances in treatment, and finding a cure for Barth syndrome. To achieve this goal, BSF has invested \$5.6M USD in research, which has translated into \$23M in follow-on funding from other agencies and catalyzed another \$6.7M USD for clinical trials research.



**Research & Development**



**Family Services**

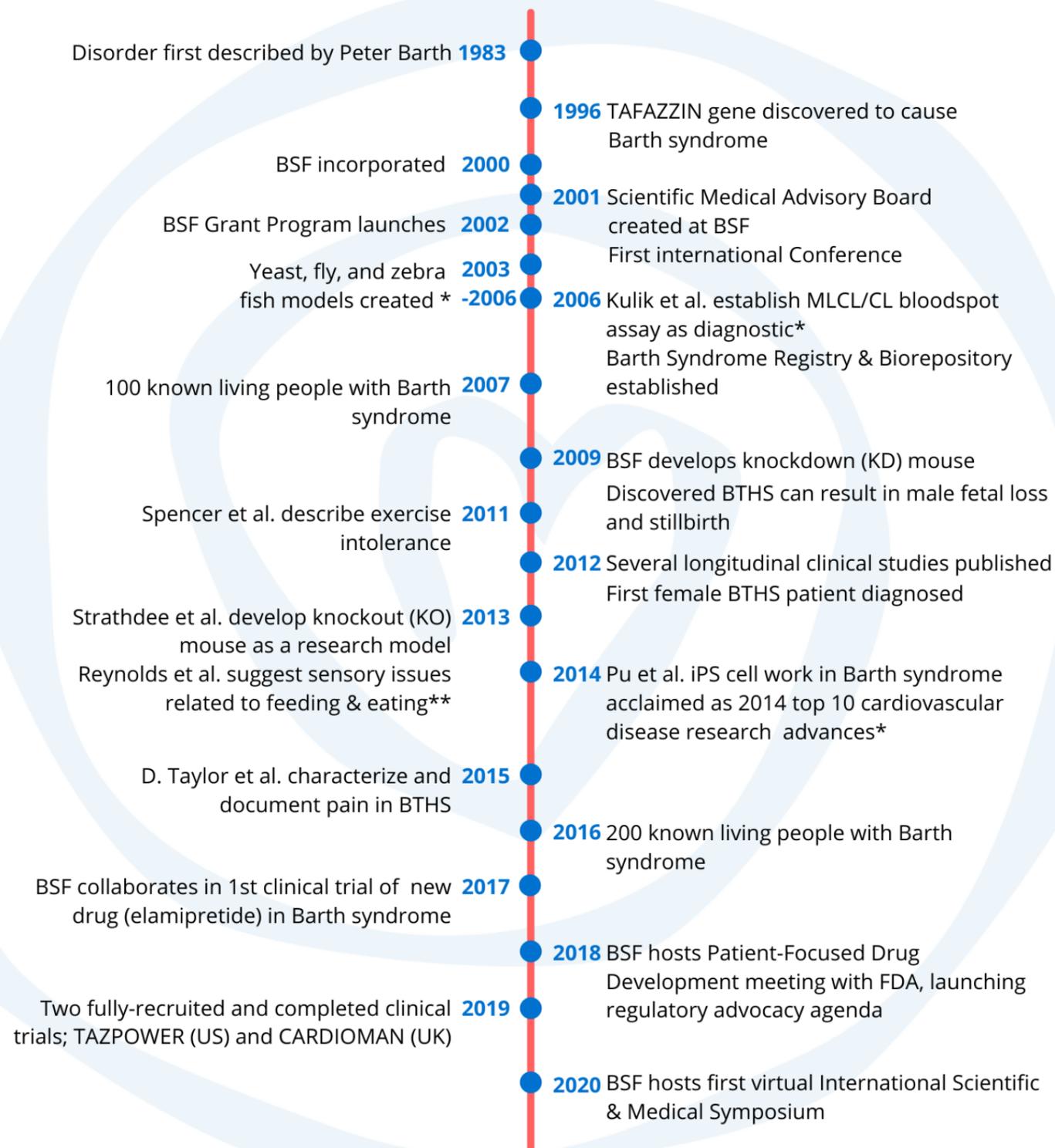


**Advocacy**

**BSF provides a lifeline** to families and individuals living with Barth syndrome around the world, offering 24/7 individualized support, educational conferences, a robust patient registry, and collaborations with specialist healthcare providers to define standards of care, treatment, and rapid diagnosis.

# BSF Through the Years

# Our Global Community



\*grant support from BSF

\*\* grant support from BSF affiliates

## BSF AFFILIATES

### BARTH SYNDROME FOUNDATION OF CANADA

Phone: 1-888-732-9458 or 905-873-2391  
www.barthsyndrome.ca

### SYNDROME DE BARTH (FRANCE)

Phone: +33 6 15 58 02 32  
www.syndromedebarth.fr

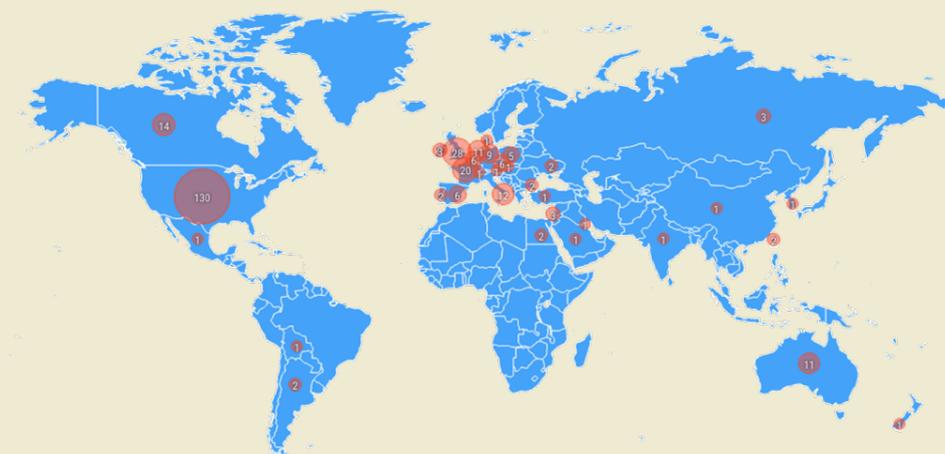
### BARTH SYNDROME UK

Phone: +44 1794 518 785  
www.barthsyndrome.org.uk

### BARTH ITALIA ONLUS (ITALY)

Phone: +390392023777  
www.barthitalia.org

## 292 Individuals Living With Barth Syndrome Worldwide\*



\*As of December 2020

# Research & Development

In order to meet the needs and challenges faced by our community of affected individuals, **BSF's Research and Development Program** is driven to advance treatments, foster collaborative research, and serve as scientific ambassadors to engage the partners essential to achieve our vision of a world in which Barth syndrome no longer causes suffering or loss of life



**115 Research Grants Awarded**  
Since 2002

**Totaling \$5.6M**  
in funding from BSF and affiliates

**To 67 Principle Investigators**

**At 49 Institutions**  
Around the world

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**Catalyzing >\$23M**  
In follow-on funding and  
**\$6.7M in Clinical Trials**

## Community Voices: Meet Steve



Steve is 34 years old and currently works in the field of auditing and coding. Steve is 6'2" now but was 4'11" going into his senior year of high school. By the time he entered college at Northern Kentucky University, he grew to be 6'2" during two large

growth spurts. "My brother and his friends called me Big Steve when I was in high school, even though I was the shortest person in my class. That stuck, and they still call me Big Steve. I guess it started as an oxymoron, but I grew into the name."

Shortly after Steve was born, he experienced seizures, heart problems, and difficulty with eating. He was treated for an unspecified muscular dystrophy after they ruled out Duchenne or Becker MD.

Steve's journey to a Barth syndrome diagnosis began about five or six years ago after his mom

watched an episode of "Mystery Diagnosis" featuring Barth syndrome. Steve and his mother brought it up to the doctors, but they didn't think he needed to go through genetic testing since it wouldn't change his treatment.

In 2019, Steve reached out to the genetics department at Cincinnati Children's hospital. The genetic counselor immediately thought it was Barth syndrome. Steve's Barth syndrome diagnosis was confirmed through a dilated cardiomyopathy genetic panel.

In his free time, Steve always enjoyed sports but couldn't play them. He follows the Cincinnati Reds, Bengals, UC Bearcats and is a season ticket holder with his dad for the NKU Norse basketball team.

When asked about growing up not knowing he had Barth syndrome or any other condition, Steve says "I guess you learn to listen to what your body is telling you because that's all you have to rely upon. You know to do what you can."

# Grant Awards

In consultation with BSF's Scientific and Medical Advisory Board, and with the support of the international affiliates - Barth Syndrome Foundation of Canada, Barth Syndrome UK, Association Syndrome de Barth France, and Barth Italia Onlus - our strategic seed-funding approach has resulted in BSF's original research investments translating into nearly \$30M USD in follow-in funding and clinical product development for Barth syndrome.

**The 2020 research grant recipients** demonstrated innovative and scientifically-rigorous approaches to addressing knowledge gaps in two key areas: Barth discovery science and development of therapeutic opportunities.



## Development of mitochondria-targeted peptide compounds as Barth syndrome therapeutics

Nathan Alder, PhD, Associate Professor, University of Connecticut, Storrs, CT

Awarded to Associate Professor Nathan Alder of University of Connecticut, this project will first ask how Szeto-Schiller compounds (of which elamipretide is a member) impact mitochondrial function. These findings will then allow Dr. Alder and his team to focus on developing Szeto-Schiller compounds tailored to treat Barth syndrome. The potential impact of this project stems from Dr. Alder's ideal expertise in cell-free model systems, alongside our increasing knowledge of the safety and efficacy (or positive impact) of elamipretide in our affected individuals. This project's funding was made possible by the generous support of the Will McCurdy Fund for Advancement in Therapies for Barth Syndrome.



## Investigation of a new nutraceutical for treatment of Barth Syndrome

Robin E. Duncan, PhD, Associate Professor, University of Waterloo, Waterloo, ON, Canada

Awarded to Associate Professor Robin Duncan of University of Waterloo, this project will assess the therapeutic potential and activity of a nutraceutical (a possible supplement therapy that is available without prescription) in preserving the viability of Barth syndrome cells. Following up on early results that this product has the ability to help Barth syndrome cells survive at the same levels as normal cells, Dr. Duncan and her team will try to understand what is the process that helps preserve these cells and further expand her research into the TFAZZIN knockout (KO) mouse model. As an early stage research effort (aka preclinical study), this research aims to provide the foundational understanding of this nutraceutical and its impact on Barth syndrome. This project's funding was made possible by generous contributions from our affiliates Barth Syndrome Foundation of Canada and the Barth Syndrome UK.



## Structural and Biophysical Studies of Tafazzin

Steven Glynn, Associate Professor, Stony Brook University, Stony Brook, NY

Awarded to Associate Professor Steven Glynn of Stony Brook University, who is a trained crystallographer (someone who purifies and generates protein crystals for structural studies), this project will apply his experience towards purifying normal and variant versions of tafazzin from yeast. If successful, he will determine what normal tafazzin looks like and how variant versions look and function differently. Since protein structure dictates function, this important discovery science project will shed light on how some of the TFAZZIN variants we know of and track, impact tafazzin activity. This project's funding was made possible by a generous contribution from our affiliate Association Syndrome de Barth France.



## Cardiolipin Requirement for Mitochondrial Calcium Import

Vishal Gohil, PhD, Associate Professor, Texas A&M University, College Station, TX

Awarded to Associate Professor Vishal Gohil of Texas A&M University, this discovery science project aims to understand how levels of calcium, mature cardiolipin (CL), and energy generation play a role in Barth syndrome. By using the highly modifiable yeast system, Dr. Gohil will probe what happens with reduced levels of mature CL (which is what happens in Barth syndrome) and its subsequent impact on the amount of calcium inside and outside of the mitochondria. Studying this relationship may further shed light on why Barth syndrome mitochondria produce lower levels of energy.



## Essential activities of Tafazzin that are independent of cardiolipin remodeling

William T. Pu, MD, Professor, Boston Children's Hospital, Boston, MA

Awarded to Professor William Pu of Boston Children's Hospital and a member of the BSF SMAB, this project aims to understand the functions of mouse tafazzin beyond generating mature cardiolipin (CL). By utilizing the TFAZZIN knockout (KO) mouse model, Dr. Pu and his team found that replacing the gene with different versions of TFAZZIN, resulted in different levels of rescue for the KO mice. By using these versions tafazzin as bait for new interacting proteins, Dr. Pu and his research team hope to identify new proteins and functions for TFAZZIN independent of its CL-modifying function.

# Scientific & Medical Symposium

Although we had planned an in-person conference in 2020, the COVID-19 pandemic made our effort impossible. With the support of our research community, BSF quickly went to planning a meaningful and accessible convening. On July 23-24, we proudly launched our first ever virtual Scientific and Medical Symposium.

With an audience of 160 unique viewers each day, and an agenda that featured 15 presenters across a host of topics, this event demonstrated the global scope of the Barth community of patients, families, researchers, and clinicians.

## Session Topics

**CARDIOLIPIN & METABOLISM**  
**BTHS CARDIOMYOPATHY**

**NEUTROPENIA IN BTHS**  
**PATHOPHYSIOLOGY**

In addition to the research presentations, two individuals with Barth syndrome, Andrew and Cameron, shared their firsthand experience with cardiomyopathy and neutropenia, respectively. Their stories are a testament to BSF's continued effort to make central and elevate the patient's voice.



Cameron



Andrew

## 14 Countries Represented

Australia  
Austria  
Belgium  
Brazil  
Canada  
Denmark  
France

Germany  
Italy  
Japan  
Netherlands  
Russia  
United Kingdom  
United States

"The Barth Syndrome Foundation is highly supportive of young investigators in the field, and this has undoubtedly led to the furthering of our understanding of BTHS and therapeutic development for this rare disease."

-Christina P

## WE THANK OUR PRESENTERS

## FOR THEIR RESEARCH AND DEDICATION



**Jose Bozelli Jr., PhD**  
McMaster University



**Christoph Maack, MD**  
University Clinic Wuerzburg



**Mauro Corrado, PhD**  
University of Freiburg



**Charles McCall, MD**  
Wake Forest School of Medicine



**Deena Damschroder,**  
PhD Candidate  
Wayne State University



**Peter Oates, PhD**  
Stealth BioTherapeutics



**Yu Li, Medical Student**  
University of Pittsburgh



**Christopher Park, MD, PhD**  
New York University



**Colin Phoon, MD**  
New York University



**Chris Rice, PhD**  
University of Bristol



**David Sykes, MD, PhD**  
Massachusetts General Hospital



**Carolyn Taylor, MD**  
Medical University of South Carolina



**Reid Thompson, MD**  
Johns Hopkins University  
School of Medicine



**Suya Wang, PhD**  
Boston Children's Hospital



**Hilary Vernon, MD, PhD**  
Johns Hopkins University  
School of Medicine

All presentations and abstracts are viewable at [www.barthsyndrome.org/research](http://www.barthsyndrome.org/research), and we graciously thank all of our speakers for their willingness to share their work and progress with the broader BTHS community.

# Publications

Since the launch of the BSF research grant program 20 years ago, our field has fostered and experienced the tell-tale history of scientific progress, from incremental findings to breakthrough achievements. Supported by BSF funding over the years, our research community's publications this year demonstrate this level of success and have expanded our understanding of Barth syndrome in our affected individuals, cardiolipin's role in metabolism and energy generation, and the curing of the Barth syndrome mouse via gene replacement therapy.

## Gene Replacement Therapy in the Tafazzin Knockout Mouse

Driven by Dr. Suya Wang and led by Dr. Bill Pu, this pivotal research effort not only characterized the value and utility of the TFAZZIN knockout (KO) mouse, but also demonstrated the ability of gene replacement therapy to rescue and reverse Barth syndrome symptoms and manifestations (phenotype). Experiments utilizing the full KO (complete absence of TFAZZIN), Dr. Pu's team demonstrated that the KO resulted in increased newborn deaths compared to controls, which could be rescued by providing a new copy of TFAZZIN via adeno-associated virus delivery (AAV). Utilizing a cardiac-specific knockout of tafazzin (only absent in heart cells, CKO), his team showed that the levels of AAV-TFAZZIN delivered correlated with the ability to rescue the cardiomyopathy associated with Barth syndrome, wherein if 70% of cardiac cells get a new copy of TFAZZIN, cardiac dysfunction and fibrosis could be prevented. Of value for the age range of affected individuals with Barth syndrome, delivering high levels of AAV-TFAZZIN in older mice was found to reverse levels of cardiac fibrosis and improve mitochondrial structure.

*AAV Gene Therapy Prevents and Reverses Heart Failure in A Murine Knockout Model of Barth Syndrome.* Wang et al, *Circ Res.* 2020 Apr 10; 126(8): 1024-1039.

## The Integral Role of Cardiolipin in Metabolism

Dr. Miriam Greenberg and her research team continue to expand our understanding of how the loss of the lipid cardiolipin contributes to the varied manifestations of Barth syndrome. In the first of two publications, Dr. Greenberg builds upon her previous findings that iron-sulfur biology is influenced by cardiolipin levels and how loss of cardiolipin results in a chain reaction that ultimately lowers that levels of generated glutathione. Given glutathione's antioxidant role in protecting the cell by absorbing reactive oxygen species (ROS), Dr. Greenberg provides another clue in cardiolipin's critical role in maintaining cellular health. Secondly, her research team delved more deeply into how loss of cardiolipin results in iron-sulfur biology, ultimately connecting their initial finding to the transport and maturation of the protein frataxin (the causative gene of Friedreich's Ataxia, FA). Beyond expanding our understanding of Barth biology, these results also raise the possibility that treatments in the FA field may have therapeutic potential for our affected individuals.

*Loss of the mitochondrial lipid cardiolipin leads to decreased glutathione synthesis.* Patil et al, *Biochim Biophys Acta Mol Cell Biol Lipids.* 2020 Feb; 1865(2): 158542

*Cardiolipin-deficient cells have decreased levels of the iron-sulfur biogenesis protein frataxin.* Li et al, *J Biol Chem.* 2020 Aug 14;295(33):11928-11937

## Amino Acid Metabolism in Affected Individuals

Involving the participation of five of our affected individuals, Dr. Todd Cade and his research team demonstrated that the uptake and distribution of the amino acid arginine differed when compared to healthy controls. Given the reliance of Barth cells on metabolizing proteins and sugar to generate energy, Dr. Cade's results continue to build on his body of Barth metabolic understanding and set the stage for further research into how arginine and other amino acids play a role in the manifestation and symptomology of Barth syndrome.

*Arginine kinetics are altered in a pilot sample of adolescents and young adults with Barth syndrome.* Cade et al, *Mol Genet Metab Rep.* 2020 Nov 4;25:100675

## Altered Metabolism in Barth Syndrome Mouse and Cell Models

Talented researchers and BSF grantees Dr. Renata Goncalves and Dr. Adam Chicco both published tours de force in mouse and cell biology that elucidate how loss of tafazzin and cardiolipin result in altered Barth biology. Dr. Goncalves measured the levels of ROS in tafazzin knockdown (KD) mice and found that loss of tafazzin did not translate into greater capacity to generate ROS. Also using the KD mice, Dr. Chicco found that the tafazzin-deficient heart is limited in its ability to utilize pyruvate and fatty acids as fuel and rather rely on the consumption of glutamate to meet the demands of energy generation.

*Cardiolipin deficiency in Barth syndrome is not associated with increased superoxide/H<sub>2</sub>O<sub>2</sub> production in heart and skeletal muscle mitochondria.* Goncalves et al, *FEBS Lett.* 2021 Feb;595(3):415-432

*Tafazzin deficiency impairs CoA-dependent oxidative metabolism in cardiac mitochondria.* Le et al, *J Biol Chem.* 2020 Aug 28;295(35):12485-12497

## Researcher Spotlight: Iris Gonzalez



Dr. Iris Gonzalez, affectionately known simply as Iris in our community, is the diligent and humble molecular geneticist who was a founding member of our Scientific and Medical Advisory Board (SMAB) and both creator and curator of our "Human TFAZZIN Gene Variants Database." We are truly indebted for her 20 years of service and contributions to our community, from her body of scientific work, to her involvement with patients and ability to explain complex genetic concepts to them and their families, to her diligent efforts curating the Human TFAZZIN Gene Variants Database. Iris has dutifully researched the gene's conservation across species and has tracked, recorded, and classified the genetic history of our human community through the immensely valuable variants database. Beyond being the most knowledgeable individual anywhere about the Barth gene, Iris has been unceasing in her willingness to share her expertise with everyone. She is a generous, thoughtful and compassionate person who will forever be a beloved member of our community.

Rare diseases are often referred to as zebras in a field of horses. Iris is a wonderful unicorn.

# Family Services

**Family Services** works to champion a thoughtful, engaged, well-informed, inclusive community who are collectively invested in achieving the ultimate vision of saving lives and ending suffering caused by Barth syndrome

## Family Services Objectives

**Advance** collaboration among clinicians to improve health outcomes for people affected by Barth syndrome

**Educate** affected individuals and their families to help them understand the various symptoms of this complex disease and navigate its management

**Grow** and nurture a caring and supportive community to help empower affected individuals and their families

**Integrate** healthcare providers into BSF family programs and global community so that there is two-way benefit of increasing knowledge

**Support** individuals, families and physicians in their quest to obtain a diagnosis and throughout the Barth syndrome journey

## Community Voices: Meet Tyler



Seventeen-year-old Tyler is currently a junior in high school. Upon graduation, Tyler plans to enroll in college with the intent to become a priest. "It's been really wonderful since I embraced that decision," Tyler said. "Before, it was like something was missing but since I made the decision that this

is what I wanted to do, everything has been wonderful."

In his free time, Tyler loves to bowl and go fishing. He also enjoys playing video games because, in

his words, "I am just bad at sports in general. I think this goes beyond Barth syndrome. I am just bad at sports." Although he doesn't play sports, he likes watching football and baseball and is "more than happy to watch people who are good at it."

When he's in the mood for some tunes, Tyler listens to 40's, 50's, 60's and 70's music "and that's it." To Tyler, "that is the four decades when music was good." His favorites are Frank Sinatra, Dean Martin, Bobby Darren, Deon and the Belmonets and Paul Anka.

One parting word from Tyler, "if anyone has a PS4 and would like to play a video game or just want talk to someone, I am always available."

# Family Services

## Patient-Centric Approach



Maintain a vast library of Barth syndrome fact sheets to support affected individuals and their families



Facilitate communications amongst the Barth community using an array of technologies including listserv, Facebook groups, virtual meetups, registry, surveys, and polls



Tailor programs and services to specific experiences of affected individuals, parents, siblings, care teams, and researchers



Support individuals and families through their Barth syndrome journey in areas of diagnosis, referrals, disability benefits, and healthcare decisions



Create frequent opportunities for knowledge and experience exchanges to inform crisis responses, care guidelines, and multimedia resources



"Without BSF, we wouldn't have known how to care for our son or give him his best chance at a long full life. I tear up thinking how much they've done for us. Thank you BSF!"

-Laura

## 2020 Webinar Series

BSF Family Services collaborated with our science and medical team to develop a series of webinars for our community designed to deliver updates and outcomes of current research and development relating to Barth syndrome. The webinars listed below were presented throughout the year, bringing together individuals with Barth syndrome, families, and the research and medical communities for valuable information exchange and discussion.

### GENE THERAPY WEBINAR

Barry Byrne MD, PhD, University of Florida  
Christy Pacak PhD, University of Florida  
Todd Cade PT, PhD, Duke University

October 15, 2020 | 12pm-1pm EST

### Assessment of Cardiac Function in Barth Syndrome

Carolyn Taylor, MD  
August 2020



### Barth Syndrome: Cardiac Arrhythmia Concerns

Carolyn Taylor, MD and Lanier Jackson, MD  
Pediatric Cardiology  
Medical University of South Carolina



### Potential Role of Entresto (sacubitril-valsartan) in Barth Syndrome Cardiac Management

With John Lynn Jefferies, MD, MPH, FAAP, FACC, FAHA, FHSA  
Jay Michael Sullivan Distinguished Chair in Cardiology  
Professor and Chief, Division of Adult Cardiovascular Diseases  
Director, Methodist University of Tennessee Cardiovascular Institute  
Director, Methodist Cardiovascular Service Line  
Professor, Pediatric Cardiology, Le Bonheur Children's Hospital  
Professor, Department of Preventive Medicine  
Research Member, St Jude Children's Research Hospital

The recordings of these webinars along with other virtual community events can be viewed by visiting the BSF website at [www.barthsyndrome.org](http://www.barthsyndrome.org)

# COVID-19 Support

BSF recognizes that 2020 was an extraordinary year and that the COVID-19 situation is extremely stressful and frightening for most people in our community. Parents, caregivers, grandparents, teens and adults with Barth syndrome who often experience isolation, felt especially disconnected during the pandemic due to their medical vulnerability. Together with our community, BSF rallied to identify and implement innovative and meaningful methods to continue offering support through these challenging times.

## COVID-19 Resource Library



The COVID-19 Precautions page on the BSF website was created to provide guidance to and answer questions from the Barth community in the age of COVID that would be accessible day or night. Dr. Colin Phoon and Dr. Brian Feingold, BSF medical advisors were instrumental in collecting the content for the resource library. BSF staff continuously worked with our medical community to keep the resource page current, updating content as new pandemic developments arose.

## Support Groups



Beginning in March 2020, BSF offered virtual support groups EVERY WEEKDAY for anyone in the community looking to connect via video chat. The purpose of these gatherings was to allow our global community of affected individuals, parents, and caregivers to share concerns and offer support to each other during the stressful times of quarantine and a global pandemic.

## Community Engagement



In addition to creating opportunities for information and experience exchanges, BSF recognized that the social health of our community was being negatively impacted by the pandemic. In response, we worked with the community to develop opportunities to socialize and decompress in a more relaxed atmosphere. Barth Yoga, quarantines, and Sunday Funday allowed community members of all ages and demographics to participate in virtual mindfulness and social wellness activities.

## Friday Roundtables



The roundtables were developed to be community conversations and provide an opportunity to connect with others while sharing and receiving meaningful information.

Over the year, the community has gathered for over 25 roundtables, with particularly steady attendance over the summer. Discussion topics included Barth syndrome carrier issues, eating/feeding challenges in BTHS, metabolic issues, family planning, and many more, each led by an expert in the respective field.

During the Zoom calls, community members had the opportunity to ask questions and share experiences, which was primary to the design of the roundtables, thereby increasing opportunities to connect. This way, they not only were informative but also helped mitigate isolation in the age of COVID.

**280+ hours of real-time Conversation, Community Building, and Information Exchange!**



[Click the image to watch the recordings](#)

# In Memory...



## Jeremiah

"In loving memory of Jeremiah Sage Caldwell. You'll be in our hearts, always."



## Max

"Max adored Queen, drumming, theater, pasta in brodo and spending time with his brother and friends. This picture was taken during a 2019 hike to a local mountain hut, Cabane Brunet. He was exhausted but so proud he made it to the top. He is forever in our hearts."



## Oliwier

"Oliwier Staniszewski był zawsze uśmiechniętym szczęśliwym chłopcem o tysiącu pomysłach. Zmarł mając zaledwie 6 lat 10.04.20 w Zabrze nie doczekał przeszczepu serca."  
(Oliwier Staniszewski was always a smiling happy boy with a thousand ideas. He died when he was only 6 years old on 10/04/2020)



## Rúnar

"Even with everything he was going through, with all his surgeries and the Berlin heart, he was always happy."



## Tristan

"In loving memory. Tristan Kade Strange March 11, 1996 - June, 18, 2020."



## Vincent

"Vincent was a cheerful boy who never wanted to be bored and loved the moon, trams, flags and his stuffed lion cub. We are grateful for the awesome two and half years we spent with him, and we miss him every day."

# Advocacy

**Advocacy Services** works to develop partnerships that advance BSF's mission, educate healthcare providers, empower people with Barth syndrome, and develop support systems for people with Barth syndrome and their families

## Advocacy Priority Areas

**Partnerships** Cultivate relationships with the greater rare disease community to increase opportunities for collaboration and change

**Policy** Inform the community of legislation and other systemic initiatives that directly impact the health and quality of life of those with Barth syndrome and amplify the Barth community voice

**Awareness** Increase awareness of Barth syndrome and its impacts on affected individuals and their families through education

**Self-Advocacy** Support individuals with Barth syndrome and their network as they advocate for their rights

## Community Voices: Meet Joe



Joe is the father of four loving children and a business owner. He enjoys hunting and fishing and loves being outdoors. "I love the solitude while being out in the woods. I enjoy taking the children out with me as well." When Joe was a kid, he would come home from school and go

rabbit hunting while other kids were out playing sports. He worked with dogs and taught them to go fetch the rabbits. Joe did sometimes play sports though, but he says, "When kids were chosen for the team, I wasn't the one that would be picked first."

Joe worked on a dairy farm milking cows when he was in high school. "I enjoyed working on the farm," Joe says of the experience. "Cows don't argue with you. They are happy to see you."

Joe was initially diagnosed with Marfan syndrome when he underwent a mitral valve repair in 2001.

In 2018, Joe came down with what he thought was a bad cold that progressively became worse and wasn't responding to treatment. He eventually went to the Cleveland Clinic where, after a heart catheterization, the doctor's told him he wasn't leaving until he had a heart transplant. Joe had that transplant on his 44th birthday.

After the transplant, Joe proceeded with genetic testing to confirm Marfan syndrome, as he and his wife wanted a definitive answer out of concern for their children. "I don't think anyone expected the findings to come back as Barth syndrome," Joe said. "Not even the doctors."

"Before I knew I had Barth syndrome, I knew I was different than other kids," Joe reflected back on growing up. "I knew I had a heart problem, now I just have a name to give to it. I was concerned about dying; no one knows what the future holds. Hopefully if we all work together, we can change the future."

# Advocacy

## Rare Disease Week



BSF joined over 900 other rare disease patients and families on Capitol Hill during the February 25-28, 2020 Rare Disease Week. Rare Disease Week is hosted by Rare Disease Legislative Advocates (RDLA) to connect patients, families and rare disease organizations with legislators for education, awareness and advocacy on legislation that directly impacts our communities.

BSF proudly represented our individuals and families during 4 days of meetings with numerous legislators. "Individual rare

disease populations are small in number and often overlooked by policy makers. Coming together with other rare disease advocates to raise our collective voice and be heard was empowering," said Shelley Bowen, BSF Director of Family Services and Advocacy.

As part of the effort, nine members of Congress joined the Rare Disease Congressional Caucus, bringing the total membership to 170. 2020's Rare Disease Week proved to be the largest since it began in 2012.



## Barth Syndrome Community Voice Report



**1400+**  
PEOPLE  
PARTICIPATED

**23,070**  
POSTS  
SHARED

**61,204**  
COMMENTS  
ELICITED



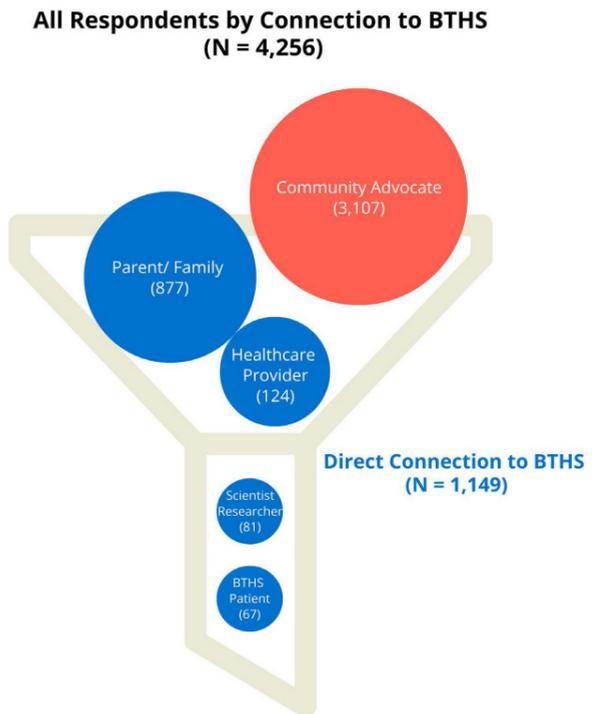
BSF partnered with TREND Community to analyze patient experience data shared from December 2003 to August 2020 via our Facebook group and listserv. The analysis was published as the Barth Syndrome Community Voice Report which can be used to inform medical and support teams, understand and advocate quality of life issues and catalyze research. The full report is available via the BSF website.

## Petitioning for Drug Access

On November 17, 2020 BSF delivered our Elamipretide Community Petition to the FDA. It was signed by over 4200 supporters and accompanied by more than 730 written testimonials.

Elamipretide, produced by Stealth BioTherapeutics, is an experimental drug that has been shown to reduce debilitating fatigue and potentially improve important baseline health measures, including various heart components, in people with the ultrarare disease Barth syndrome.

Given the risk of life-threatening cardiac complications in this population, individuals with Barth syndrome cannot wait for additional studies of elamipretide before receiving access.



You can view the full petition, including all 730+ written testimonials, on the BSF website:

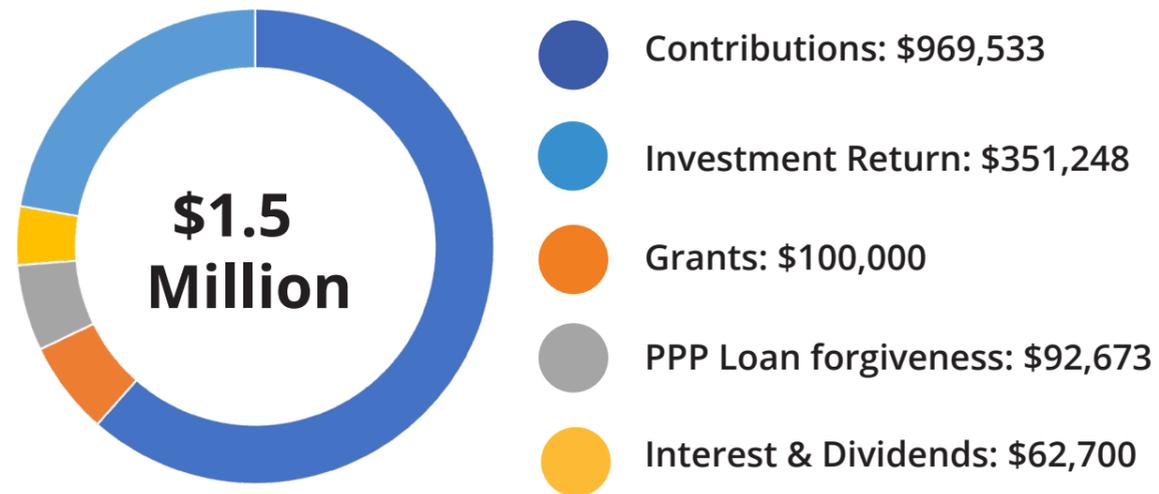
[www.barthsyndrome.org](http://www.barthsyndrome.org)

**#BarthVoices**

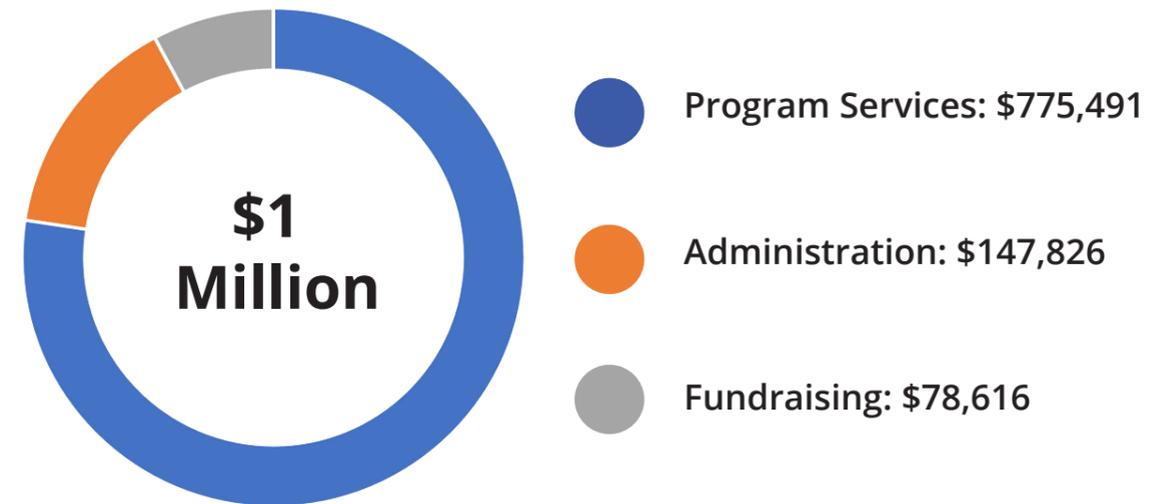
# Financials

BSF remains a financially healthy organization that continues to encourage and initiate research and development of potential new treatments. Thank you to our donors for your continued support in helping us work toward our vision-- a world in which Barth syndrome no longer causes suffering or loss of life.

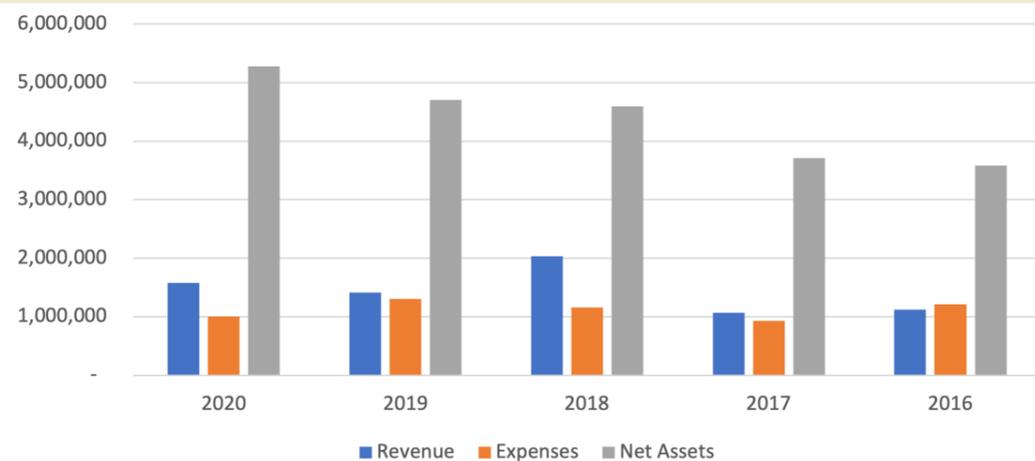
## 2020 Revenue



## 2020 Expenses



## 5 Year Trend



## 2020 Program Services Breakdown

Research Grants	\$210,604
Science & Medicine	\$184,720
Communications & Awareness	\$132,109
Advocacy	\$115,348
Family Services	\$88,804
Barth Registry and Repository	\$43,906

# Looking Ahead

# Why Give to BSF?



## Our Areas of Focus for the Coming Year

**Invest in R&D** We will make smart investments in research and development that can improve our understanding of Barth syndrome, identify possible treatments, and develop a cure.

**Support Barth Families** Through all the ups and downs, we stand with our families and offer the resources and compassion they need to navigate life with Barth syndrome.

**Advocate for Our Community** Barth syndrome is rare, and we do everything we can to make our voice heard and attract support and focus to our cause.

**Never, Ever Give Up!** No matter the obstacles, we remain committed to our vision and the people we serve.

Dear Friends,

2020 was an unprecedented year for us all - for us individually, for our regions and for our world. Despite this, BSF continues to be strong and make great progress for our community.

One of our advantages has been that we have always been a virtual organization, with no bricks and mortar headquarters and a staff and group of volunteers accustomed to operating virtually. Another has been that we are small and can be agile. Recent times have been ones in which the ability to pivot quickly has made a real difference. In early 2020, as the COVID-19 pandemic took hold, our staff and Board made some well-considered but rapid decisions. Though it broke our hearts not to be able to be together for our international conference last July (especially since we do so only every two years), it clearly was the prudent approach for our medically vulnerable population. Instead, we launched an innovative series of virtual meetings for our community – some programs were full of vital content engaging various experts and some were community strengthening, such as yoga. Our Director of Research, Dr. Erik Lontok, additionally organized one of the first virtual Scientific and Medical Symposia in the rare disease community with expanded attendance and to great acclaim.



Remarkably, our financial position also strengthened in 2020. This was due in part to our donors' steadfast support and in part to BSF's ability to quickly reduce our expenses in response to the pandemic. Over the last three years, BSF has accumulated more than \$1.5M in funds to be put toward critical initiatives. This does not mean we can rest on our laurels and simply draw from reserves, but rather that we must continue to raise funds that we will spend strategically and wisely on key projects.

Today, we are in the enviable position as a rare disease of having recently completed two clinical trials for possible Barth syndrome treatments (one for a new drug in the US and another for a repurposed drug in the UK). It is fair to say that neither of these two trials would have occurred without the support of BSF and our global Barth community. Although final regulatory approval is never certain, we continue to advocate, to be hopeful and to push ahead.

This past year also allowed us the important opportunity to take stock of where we are now and where we want to be, and to create an in-depth strategic plan for the next three years. We set priorities that will accelerate the development of new treatments and a cure, while continuing to support those affected by Barth syndrome and their families. With the advice of our expert scientific and clinical advisors, the BSF Board voted to increase investment in several building blocks crucial to further success in our research and development programs. In 2021, while counting on the continued support of our loyal donors, we have purposefully budgeted a deficit reflecting these critical increased strategic investments.

All of these ambitions require the strength and perseverance of our Barth community – our families, friends, researchers, clinicians, and advocates. We could not do all of this without all of you who contribute in so many different ways to this enterprise we call BSF. Thank you! Together, we are #BarthStrong!

With gratitude and hope,

*Kate McCurdy*



# Thank You!

We believe the most efficient way to find a cure for Barth syndrome is by directing as much funding as possible to research, providing patient and family support, and engaging in advocacy. We truly appreciate your continued support.

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Marc and Tracy Sernel

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