



Barth Syndrome
Foundation

Barth Syndrome Foundation 2019 Annual Report

CONTENTS

A MESSAGE TO OUR COMMUNITY	3
WHO WE ARE	4
WHAT IS BARTH SYNDROME	5
BSF'S PROGRAM PILLARS	6
IMPACT AT A GLANCE	7
BSF PROGRESS	8
OUR GLOBAL COMMUNITY	10
FAMILY SERVICES	12
COMMUNITY VOICES- ALFIE	15
RESEARCH	16
COMMUNITY VOICES- TRAVIS	21
ADVOCACY	22
COMMUNITY VOICES- JOHN	25
FINANCIALS	26
IN MEMORY OF	28
LOOKING AHEAD/WHY GIVE?	30
THANK YOU	32
LEADERSHIP/PARTNERS	38



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.

MESSAGE TO OUR COMMUNITY

When reflecting on the accomplishments and efforts of BSF in 2019, I recognize how powerful this year has been. BSF planted strong roots over the past two decades by building worldwide research partnerships, investing in basic science to progress to translational research, and tirelessly galvanizing the patient community in order to better describe and define Barth syndrome. 2019 bore the fruits of many of these unwavering labors, helping bring Barth syndrome to the forefront in the rare disease community and advancing viable therapies more than ever.

- BSF presented the landmark “Voice of the Patient” report to the US Food & Drug Administration (FDA), a compilation of the testimonies and survey responses of individuals affected by Barth syndrome from all around the world in the first-ever report of its kind for this ultra-rare condition. The Voice of the Patient report is critical input for researchers as they submit new and repurposed therapies to FDA for regulatory approval. It will also inform the clinical trial endpoints that are important to our community.
- Strategic alignment of BSF’s Research Grant program occurred throughout the year, culminating in the completion of two clinical trials for Barth syndrome and the first-ever Research Portfolio Review.
- BSF continued to be a leader on the rare disease stage, advising other advocacy organizations through publications, participation on elite panels and innovative partnerships.

As we look at the alignment of two decades of research partnerships and advances toward the development of viable therapies for people with Barth syndrome, the commitment to our community throughout the world remains strong. Advancing these and other potential therapies will require significant additional investment, new approaches, new partners and the continuing patience and support of our community. Nonetheless, in less than 20 years, BSF has achieved what many thought was impossible by advancing the scientific and medical understanding of Barth syndrome. We will never give up.

Thank you for supporting Barth Syndrome Foundation.

A handwritten signature in cursive script, appearing to read "Emily Milligan".

Emily Milligan, Executive Director



WHO WE ARE

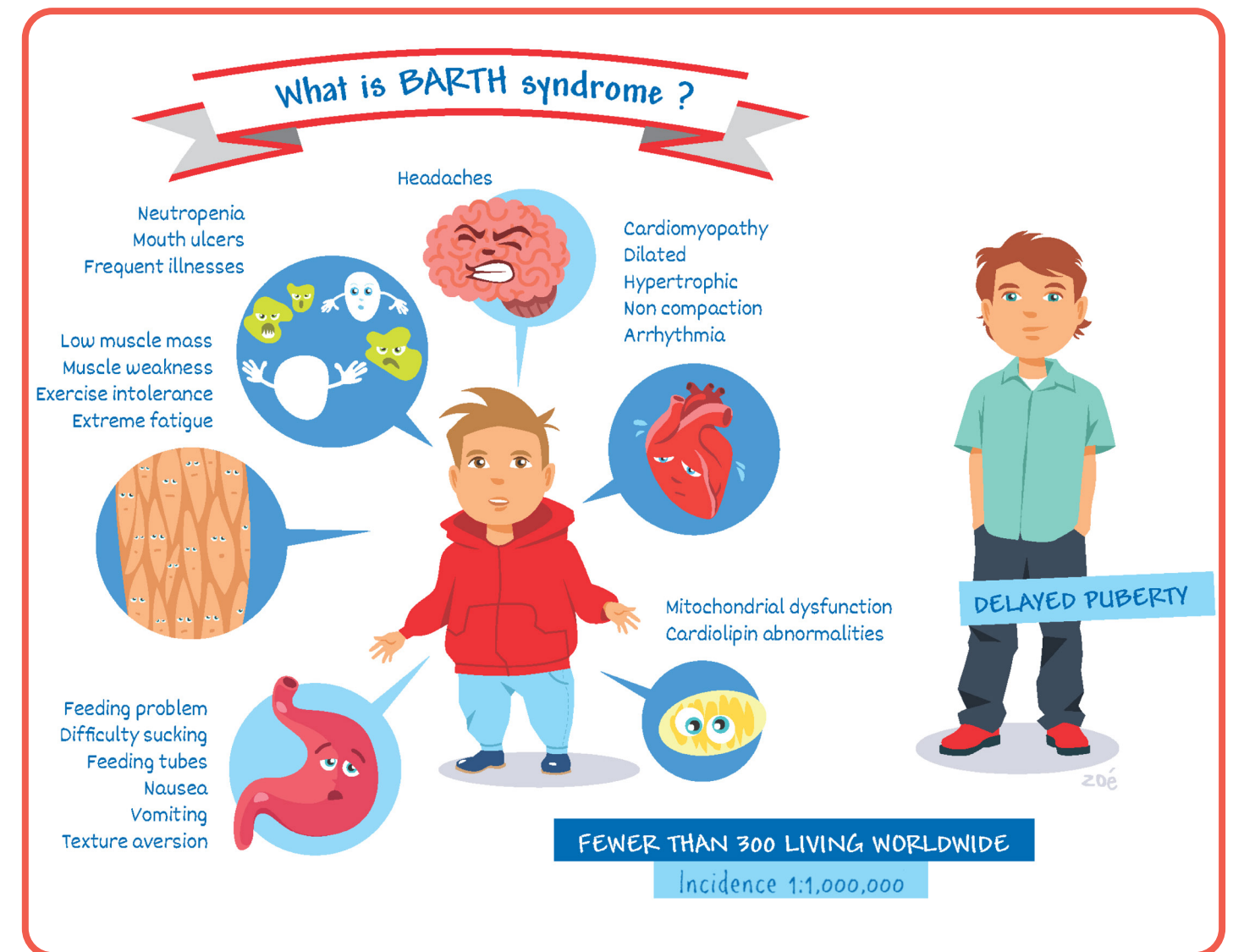
Barth Syndrome Foundation (BSF) is the only global network of families, healthcare providers, and researchers solely driven by the mission to save lives through education, advances in treatment, and finding a cure for Barth syndrome. BSF has funded nearly \$4.9M USD through 2019, catalyzed over \$21M USD in funding from other agencies, as well as \$6.7M USD in clinical trials research, all in an effort to advance global scientific discoveries to end the suffering and loss of life from Barth syndrome.



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 has funded nearly \$4.9M USD to advance scientific discovery since 2002”

WHAT IS BARTH SYNDROME?



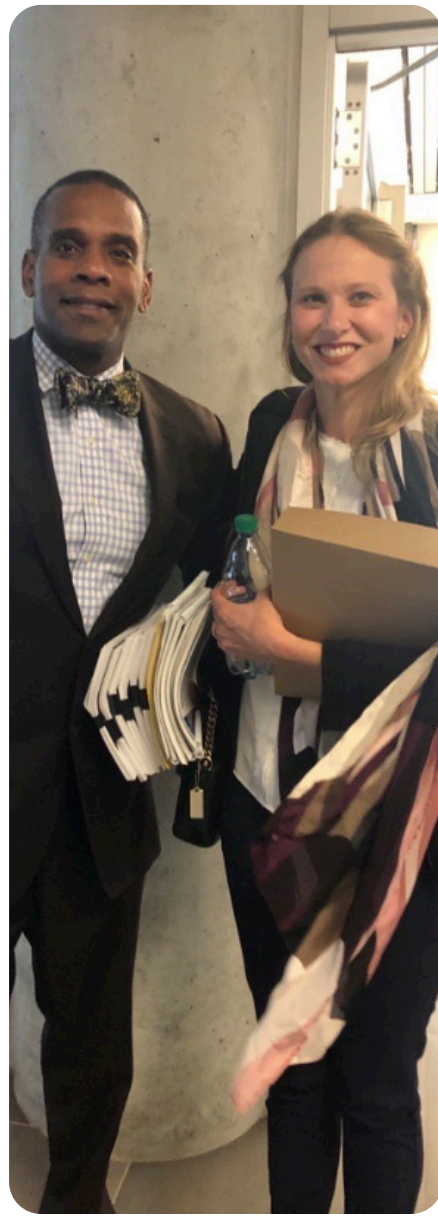
Barth syndrome is a rare, life-threatening, genetic mitochondrial disorder primarily affecting boys. Affected people may suffer from heart failure, muscle weakness, and infection (caused by neutropenia). Additional characteristics of the syndrome commonly include growth delay, impaired lipid metabolism, 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.



1/3 of those with Barth syndrome have been told by their doctor that they may need a new heart at some point in their life.

BSF'S PROGRAM PILLARS

IMPACT AT A GLANCE



416

individuals supported globally since 2002

34

number of stakeholders that took part in BSF's first-ever portfolio review

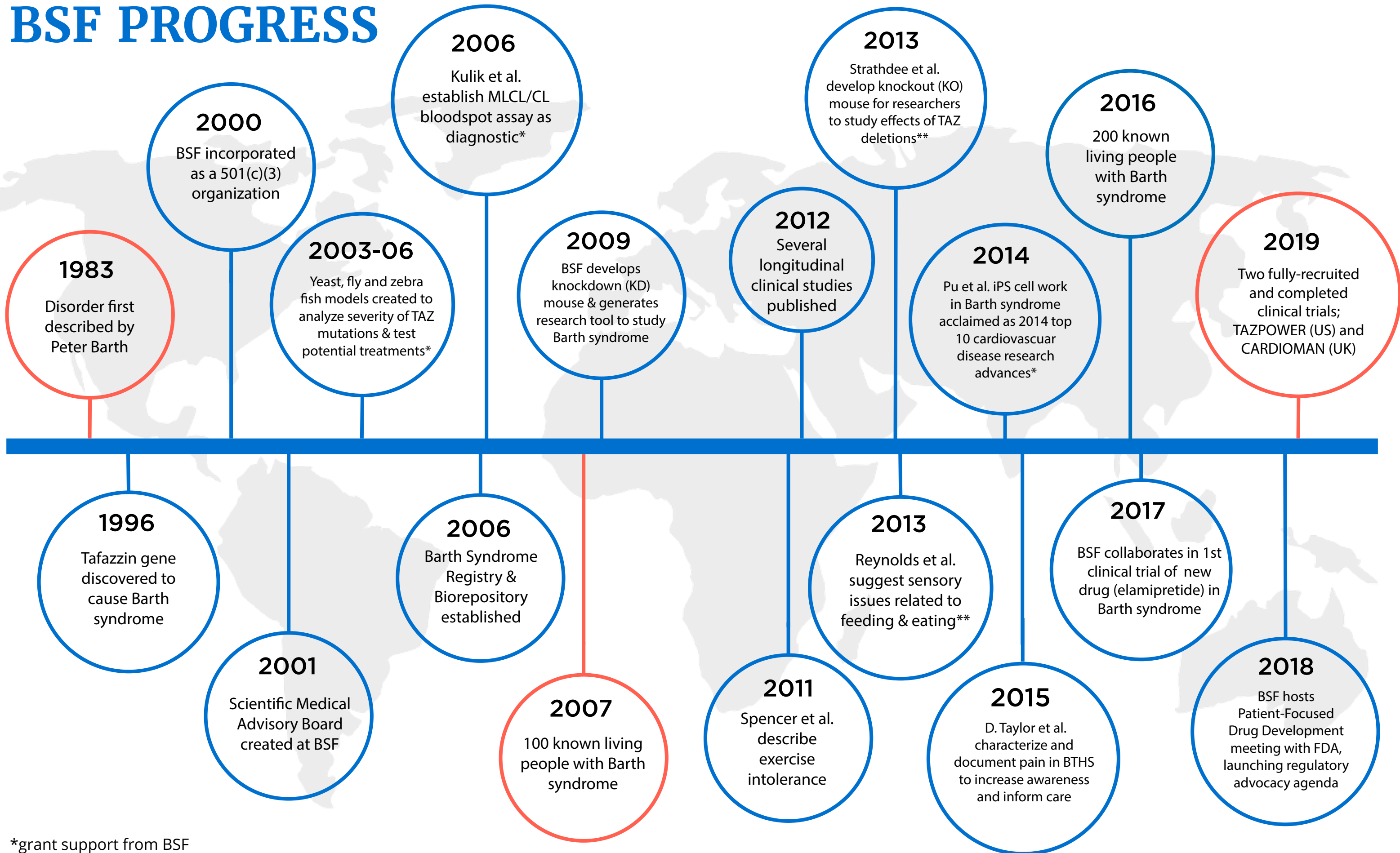
25

percent of the world's affected individual perspectives heard by the FDA during the PFDD

5.7

multiplier effect of BSF's Research program in stimulating additional funds for follow-on research and clinical trials

BSF PROGRESS



*grant support from BSF

** grant support from BSF affiliates

OUR GLOBAL COMMUNITY

BSF AFFILIATES

BARTH SYNDROME FOUNDATION OF CANADA

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

BARTH SYNDROME UK

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

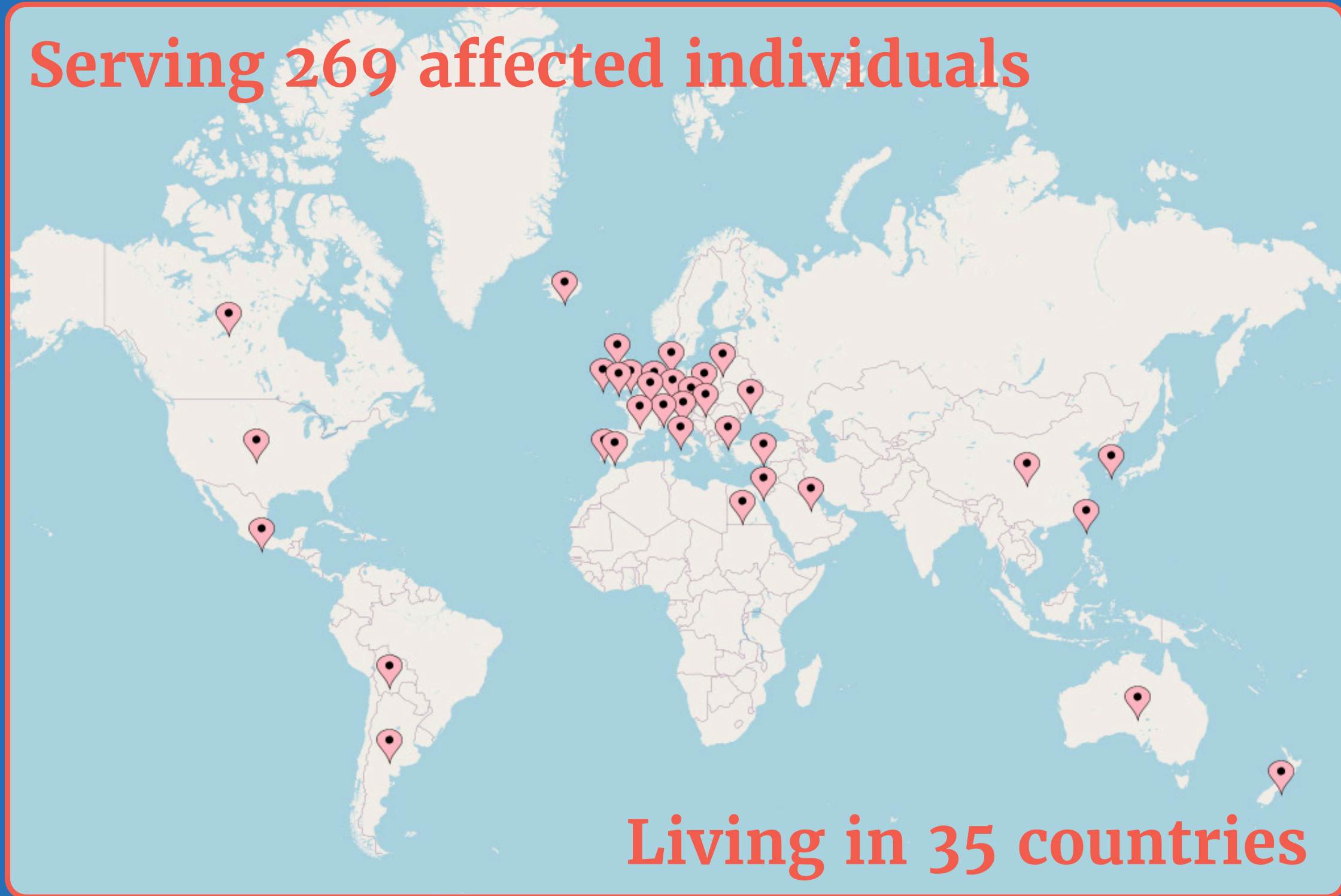
SYNDROME DE BARTH (FRANCE)

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

BARTH ITALIA ONLUS (ITALY)

Phone: +390392023777
www.barthitalia.org

Serving 269 affected individuals



Living in 35 countries

FAMILY SERVICES

BSF's Family Services is designed to support and educate families as well as their caregivers, healthcare providers and community about how to best support the person living with Barth syndrome. New families from around the world are welcomed into BSF's community via an extensive and individualized process. Existing families are supported by ongoing programs which provide advocacy and educational support including areas like disease management, access to insurance and community resources.

Advance collaboration among clinicians

Educate individuals and their families

Grow & nurture a caring & supportive community

Integrate healthcare providers into BSF family programs

Support individuals, families and physicians in their quest to obtain diagnosis & support



PATIENT CENTRICITY

**LISTSERV
&
REGISTRY**

A space for families to share their stories and experiences

**OBSERVATIONS
&
DATA**

Observational data collected through the registry become research priorities

**RESEARCH
THERAPIES**

From bench to bedside, research drives the potential for viable treatments

**IMPROVED
OUTCOMES**

Therapies improve quality of life and longevity for people with Barth syndrome

BSF has long recognized the value of gathering information about Barth syndrome directly from our global patient community. When people living with Barth syndrome offer information about their disease experience, this information helps to determine meaningful endpoints for clinical trials, improves clinical understanding of the disease, and demonstrates areas for further research and collaboration.

FAMILY RESOURCES

CARE MANAGEMENT TOOLBOX

BSF developed a family resource tool to help organize & compile important medical information to provide school and community guidance.

The Care Management Toolbox includes materials and tips to help our Barth syndrome families consolidate and organize complex medical records, manage emergency visits and plan for appropriate school and community accommodations to support the person with Barth syndrome.



These resources are provided as part of BSF's Family Services program, made possible by generous donors around the world.



2/3 of those affected with Barth syndrome report heart failure or **CARDIOMYOPATHY**

COMMUNITY VOICES

19-year-old Alfie is a university student, musician and video gamer from Scotland. He is currently studying chemistry with the intent to be a teacher once he completes his studies. "Going through [secondary] school I loved science," Alfie explained. He wanted to work with children, so teaching was a natural choice as he focused in on higher level sciences courses.



But chemistry isn't the only thing he's learning at university; he's also learning to advocate for himself. He is very open about Barth syndrome, and will talk with his friends and schoolmates because his life could depend on it, as he carries a defibrillator with him. "I explain that I have this medical condition (BTHS), what it is and what people should do if something should happen." But he also makes sure to let everyone know the defibrillator "is not who I am or a part of me, it's just something that goes along with me."

When he's not studying, Alfie plays video games online with his friends, plays the drums and is learning bass guitar. He is also a huge football fan and watches matches any chance he gets, usually in his "man cave" he has setup in his house.

What's one piece of advice Alfie has for younger people with Barth syndrome? "Things can be done in moderation. Don't try to do too much all at once. It's better to pace yourself."



70% of those affected with Barth syndrome have **NEUTROPENIA**, increasing the risk of infection

RESEARCH

Guided by BSF's mission of "Saving lives through education, advances in treatment and finding a cure for Barth syndrome," our research program is built on four pillars:

Focus on Treatment Advances

Fostering Collaborative Research

Ambassadors for the Barth Community

Stewards of the Barth Community



BY THE NUMBERS

Since 2002,
BSF has awarded
111
Research
Awards

Totalling
\$4.9
Million

To
65
Unique
Investigators

At
49
Universities,
Hospitals &
Companies

And this has translated into
\$21.3 Million
of Follow-on Funding

From Governmental Funders

From Philanthropic Partners

\$18.4 Million
National Institutes of Health

\$450,000
United Mitochondrial Disease Foundation

\$1.8 Million
Canadian Institutes of Health Research

\$680,000
American Heart Association

All of which has led to **\$6.7 Million** in Clinical Trials Funding

CLINICAL TRIALS

23 Pioneers for Barth Clinical Research

Research participation is a fundamental step towards bringing new treatments into the clinic, and 2019 was a landmark year with the completion of the first ever clinical trials focused on Barth syndrome. For our global community of 250+ affected individuals, the 23 pioneering participants alongside clinical leaders Hilary Vernon (TAZPOWER Principal Investigator) and Guido Pieles (CARDIOMAN Principal Investigator) helped drive the full recruitment of both trials, with results anticipated in Summer and Winter 2020, respectively.

TAZPOWER

US-based TAZPOWER, which is assessing the impact and safety profile of elamipretide in 12 affected individuals. Elamipretide is a small molecule that efficiently enters the cell, is targeted to the mitochondria, and interacts with cardiolipin.

CARDIOMAN

UK-based CARDIOMAN assessed the impact and safety of the repurposed drug bezafibrate in 11 affected individuals. Bezafibrate is an activator of a class of receptors (PPARs) that help make new mitochondria.



100% of those affected with Barth syndrome experience **FATIGUE**

PORTFOLIO REVIEW

In June 2019, BSF convened members of BSF's Board of Directors, the Scientific and Medical Advisory Board (SMAB), as well as four external experts to strategically evaluate the research challenges and development pathway of efforts with therapeutic potential for Barth syndrome.

With each effort previously supported by the BSF Research Grant Program – made possible by the generous support of our donors – the review meeting focused on the key steps BSF could undertake to foster continued research and therapeutic progress. For example:

Gene Therapy

Drs. Barry Byrne, Christina Pacak & Todd Cade presented their pivotal gene replacement therapy research which spans pre-clinical advancements and clinical endpoints development, for which BSF continues its support via community education and targeted research funding.

Enzyme Replacement Therapy

Dr. Michael Chin presented his pre-clinical research advancements in enzyme replacement therapy, for which BSF remains a supportive foundation partner through submitted NIH proposals.

The Research Portfolio Review was an example of BSF's collaborative approach to research, wherein our Board, SMAB, and external advisors played an active role in driving our basic discoveries further along the product development pathway.



12 different doctors are typically involved in the care of one individual with Barth syndrome

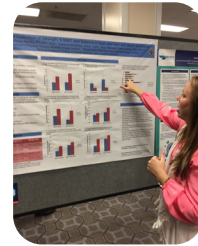
PUBLICATIONS

Research publications are the currency of an academic field, and 2019 was a stellar year in terms of the quality and scope of articles published by BSF's community of researchers. Below are five notable publications that demonstrate our continued drive towards understanding and changing the trajectory of Barth syndrome

Reduced functional exercise capacity, balance, reaction time, and muscle strength in Barth syndrome

Brittany Hornby et al, Orphanet J Rare Dis. 2019 Jan 22;14(1):37

Using tests like the six-minute walk test and the five times sit to stand test to assess functional exercise capacity and leg skeletal muscle strength, Dr. Hornby (see right) found that kids and adults with Barth syndrome walk less and take longer to sit and stand when compared to unaffected individuals. These results correlate well with the fatigue so widely reported by our community. These tests were further used to determine the impact of elamipretide (TAZPOWER) in affected individuals.



Characteristics, risks, and management of neutropenia in Barth syndrome

Colin Steward et al, Curr Opin Hematol. 2019 Jan;26(1):6-15

Neutropenia, or the loss of a certain class of white blood cells, a common symptom of Barth syndrome, can lead to life-threatening conditions from uncontrolled infection. Using the Bristol Barth Clinic data and the Barth Syndrome Patient Registry, Dr. Steward highlights the use of granulocyte colony stimulation factor or G-CSF (Neupogen) to help diminish the dangers from neutropenia and supports the use of this drug to help prevent serious infections.

Individuals with Barth syndrome rely on glucose instead of fat to generate energy for exercise

Todd Cade et al, J Inherit Metab Dis. 2019 May;42(3):480-493

With the participation of 29 Barth guys, Dr. Cade employed numerous tools including the bod pod (see right), heart function monitoring, blood draws, and graded exercise tests to understand the rate of glucose and fat consumption and conversion into energy. He reports that there is limited ability in affected individuals to generate energy from fat and instead they rely on glucose, findings that align with reduced muscle and heart function. Critically, Dr. Cade's suite of tools are key tests that will be employed in clinical trial studies to assess the impact of gene replacement therapy.



Gene replacement therapy's impact on mitochondrial health and function in Barth syndrome animal model and patient-donated cells

Silveli Suzuki-Hatano et al, Hum Gene Ther. 2019 Feb;30(2):139-154 & Int J Mol Sci. 2019 Jul; 20(14):3416

Employing the tafazzin (TAZ) knockdown mouse and cells obtained from individuals with different TAZ mutations, Dr. Suzuki-Hatano found that delivering a fully working copy of TAZ can lead to improved mitochondrial structure and cellular function, as well as improved mouse activity, levels of fatigue, muscle and cardiac function. Alongside Dr. Cade's physiological studies above, Dr. Suzuki-Hatano's pre-clinical findings serve as the underlying basic biology and proof of concept findings that argue for the potential and value of gene replacement therapy for Barth syndrome.

COMMUNITY VOICES

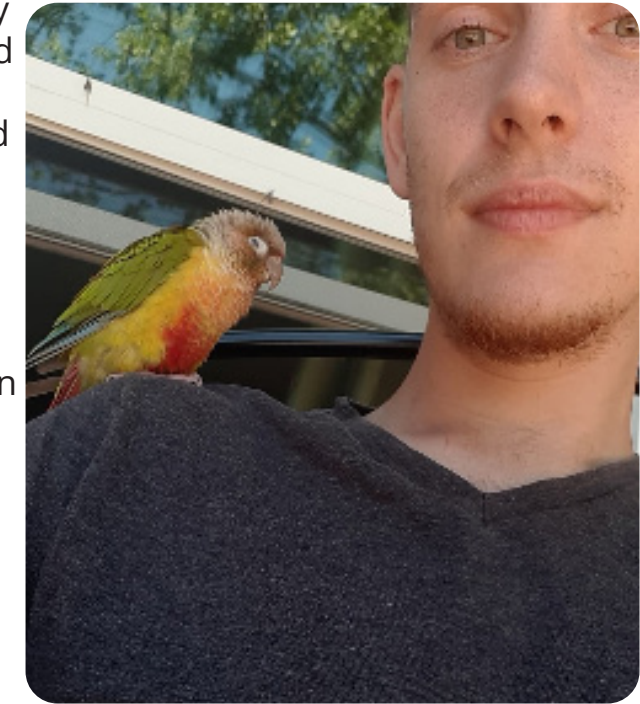
Music has always come naturally to 24-year-old Travis. He's played the guitar since he was six, at first getting lessons from his dad followed by six years of private lessons. He started sharing his talent with others by teaching lessons himself.

School was an on again, off again journey for Travis due to BTHS. He is now interested in studying speech and language pathology as he wants a "career that will support [him] and maybe other people."

Travis likes to stay active, but paces himself, taking cues from Dr. Todd Cade's research and his experience as a youth. "I was sick a lot when I was young," he recalls. "Now I tend to know my limits."

Travis describes Barth syndrome as "that sneaky little devil on your shoulder that taps to remind you it's still there. It's not just one thing; it's the constant reminders that you need to learn how to handle."

Travis's advice to the younger members of our community? "Stay active. Move, just move; even if it's just to walk for two minutes or lift two pounds, keep moving."



"I'm not sure how we would have endured it all without the help, advice and friendship of those involved with BSF. They have been there to support us through good times, bad times and even the very darkest days. BSF is our lifeline. ..." ~ Brie K.

ADVOCACY

BSF built off momentum from the 2018 patient-focused drug development meeting with the US Food and Drug Administration (FDA). In 2019, BSF charted new inroads to become the premier organization advocating for therapies and policies that positively impact the lives of individuals affected by Barth syndrome.

Develop partnerships that advance
BSF 's mission

Educate healthcare providers
on Barth syndrome

Empower people with Barth syndrome

Develop support networks for people with
Barth syndrome and their families



RAISING OUR VOICES



We represented Barth syndrome families at the **Global Genes DIY Registry Workshop** in the summer of 2019, sharing the evolution of the Barth syndrome registry over the years. Shelley spoke of the lessons we learned about enrollment, diagnostics and natural history of disease, while Emily enlightened participants on the business of registries and clinical trial design.

In March 2019, BSF presented the **“Voice of the Patient: Barth Syndrome”** report to the FDA during an in-person meeting designed to educate regulators on the types of therapies that would address the urgent unmet medical needs from Barth syndrome. This milestone continues to provide BSF the platform to engage with drug developers and advocacy representatives by sharing firsthand experience of what it means to have Barth syndrome.



In September 2019, BSF participated in a **FDA stakeholder workshop** to address recent advancements and key challenges facing mitochondrial diseases. This scientific symposium brought together academic physicians, FDA regulatory experts, and experts from related disciplines to exchange ideas on ways to address the challenges and to promote drug development in this field. BSF was one of three advocacy organizations invited to advise on the importance of natural history and patient registry data.

PARTNERSHIPS

GENE THERAPY SPOTLIGHT

BSF continues to be a thought leader in the area of gene therapy for rare diseases such as Barth syndrome. In partnership with Rare Revolution, BSF published guidance based on lessons learned surrounding regulatory, financial, and manufacturing challenges related to gene therapy. BSF's steadfast efforts to build a clinical trial-ready population and two decades of research bring the potential for gene therapy to the forefront of the organization's mission today.

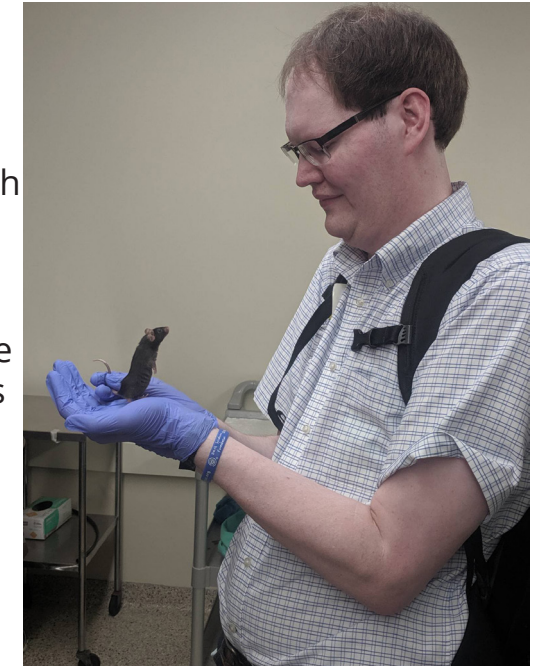
THE POWER OF PARTNERSHIP

BSF was active in establishing partnerships throughout the year, including innovative collaborations with drug developers and academic institutions as well as agencies such as the National Institutes of Health (NIH) and National Health Congress. BSF was invited to participate in the World Orphan Drug Congress, a Rare Disease Day leadership panel at NIH, and an executive think tank of drug developers and advocacy organizations. These partnerships demonstrate the potential benefits of a multi-sector approach to drug discovery which could revolutionize approaches to rare disease drug development.



COMMUNITY VOICES

Growing up, I'm sure it was difficult for my sister to have a brother with special needs. I didn't notice because I was the object of the attention so that worked for me. Seriously though, she's great. I enjoy spending time with my sister, her husband and my niece.

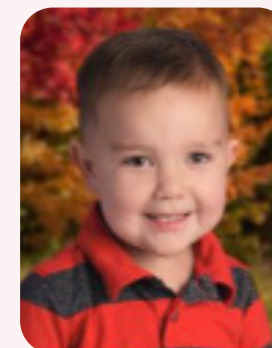


I enjoy football and basketball and am a Nebraska Cornhusker fan. I enjoy going to the games, but I also enjoy listening to the games on the radio while I am tinkering about on my model trains. Sometimes I travel with my family to out of town games, but I stay in the room and watch the game on the television and serve as the color commentator. I fix computers and I volunteer with the Barth Syndrome Foundation. I like flying flight simulators on my computer.

Computer repair comes naturally. My uncle taught me how to take them apart and put them back together. I like to figure out how things work. Mostly I do tech support and help people with their software program challenges. My clientele is typically over the age of fifty. I enjoy helping my clients. Many of them have my name and number taped to their monitor.

I always try to be patient. Maybe my capacity to be patient comes from having a rare genetic disorder. You have to be patient with that. It just kind of bleeds over into other things in life. I do admit, it's not always easy to be patient when I am tired or hungry. But I try my best.

-John W



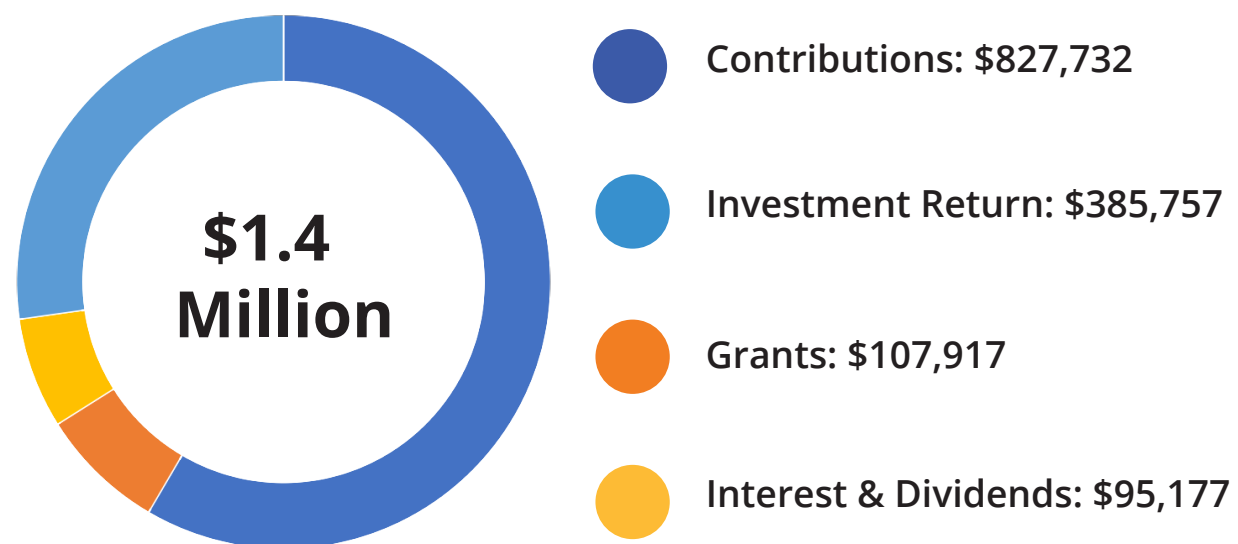
"The information from the Barth Syndrome Foundation's website has given me, as a mother, a sense of empowerment. I am able to provide quality information to my son's doctors and teachers, and this allows Isaiah to have a customized plan of care that fits his unique needs." -Leah T

FINANCIALS

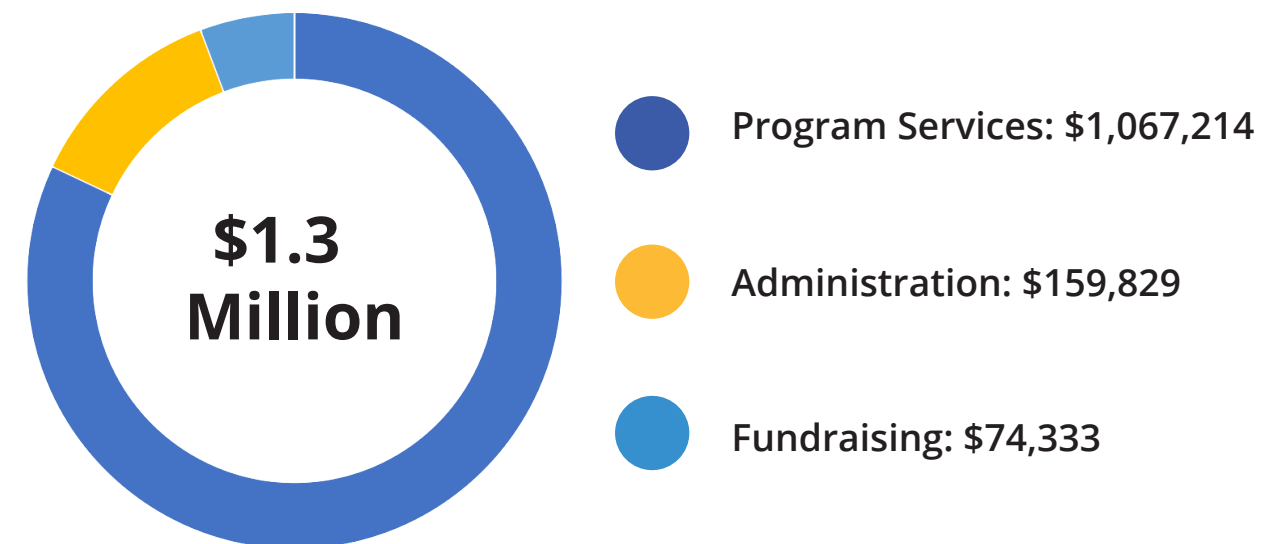
BSF remains a financially healthy organization that continues to encourage and initiate research and development of potential new treatments. Thank you to our donors for your continued support in helping us work toward our vision-- a world in which Barth syndrome no longer causes suffering or loss of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

2019 was an exciting year for BSF with our first ever portfolio review, expanded family services, and increased opportunities to advocate for our community. We are looking forward to continued program growth and research focused on potential treatments for Barth syndrome.

Revenue



Expenses



Net Assets

BSF ended 2019 with **\$4.7 Million** in net assets.
That equates to a **23% increase** over the past 5 years

Program Services Breakdown

Research Grants	\$360,000
Science & Medicine	\$300,933
Communications & Awareness	\$142,871
Advocacy & Awareness	\$136,369
Family Services	\$106,552
Barth Registry and Repository	\$20,489

IN MEMORY OF...

Timothy Monahan



The thirty years we shared with my brother Tim were a blessing, but they were far too few to spend with such an amazing person.

Tim was a source of infinite love and endless humor. He was the kind of guy who would hug you until you felt like your bones might break, and then, even though he shouldn't lift heavy objects, would try to pick you up mid-hug just to see what kind of reaction it would elicit. He loved a good reaction.

Barth syndrome ultimately took Tim's life, but he never let it define him. Instead, I believe Tim would have defined his life in terms of love and of friendship. He'd define it in holidays, crafts, and parties with family, and in cuddling his dog, and gaming with his friends, and a million perfect, little, Timmy moments, where he wasn't a guy with an illness, but one who was living his best life and embracing joy.

Even when Barth syndrome attacked his body, it never defeated Tim's courage, nor his spirit. He never stopped being Tim, and that vibrant personality lives on in our hearts and memories, never to be dulled. Despite the battle ending, Tim's spirit is forever. And in living on in those who love him, Tim has, at the end of all things, found victory over his illness.

-Jennifer M, Tim's sister

Franco Gabriel Álvarez Cedrés

Franco vino a dar amor, así de simple pero así de profundo fue su paso por este mundo.

Su hermosa mirada reflejaba la pureza de su alma.

Su sonrisa y ternura hacían estremecer hasta el más duro, y su simpatía junto a su "Hola" contagiaban a todos los que encontraba aunque fuera casualmente.

Hay personas que viven muchos años pero sin embargo no dejan nada en los demás... Franco en 15 meses nos dejó su huella indeleble... nos enseñó a sobreponernos a todo... a pesar del dolor mirar la vida con una sonrisa...

a crear lazos que nunca imaginamos... nos regaló amigos que pasaron a ser familia ... nos hizo darnos cuenta de lo verdaderamente importante.

Si hubiera podido reflexionar en voz alta nos hubiera dado sabiduría y paz, nos hubiera explicado que la vida muchas veces golpea a niveles profundamente inesperados, pero que vale la pena vivirla para aprender y trascender.

Fue muy feliz con la mejor hermana que pudo tener, esa súper hermana que le hacía reír constantemente y que lo acompañaba a donde fuera, tanto a jugar como a fisioterapia. Feliz de ver a sus tíos y primos que lo adoraban. Feliz de estar con sus abuelos que lo disfrutaron. Feliz con sus padrinos que siempre estuvieron para él. Feliz de reunirnos a todos ... feliz de elegirnos como su Papa y su Mamá.

Nunca imaginé que mi pequeño podría convertirse en mi gran maestro, me mostró un camino completamente desconocido, antes de su primera internación a los 13 meses tuvimos el diagnóstico que tanto buscamos pero que nunca imaginamos encontrar... Síndrome de Barth.

Si bien las fronteras son ficticias (la gran Familia Barth nos lo enseñó) Franco es el primer caso en América Latina y justamente nació en un país pequeño con forma de corazón, de ese corazón valiente y amoroso, ese que físicamente no resistió pero que perdura con nosotros por siempre. Estás entre nosotros Franquito y te amamos sin principio ni final.

-Claudia C, Franco's mother



LOOKING AHEAD



OUR AREAS OF FOCUS OVER THE COMING YEAR

Building cross-sectoral research partnerships in the fields of mitochondrial medicine, gene therapy, cardiology and endocrinology.

Continue championing the voice of the patient with Barth syndrome at a national level through advocacy and engagement with organizations such as FDA, NIH and NHC.

Leveraging data & insight from a global community of patients & families for talented researchers & clinicians to advance potential therapies for Barth syndrome

Serving each person with Barth syndrome and their supports through innovative educational initiatives, including webinars, symposia and publications.

WHY GIVE TO BSF?

Dear Barth Individuals and Families, Donors, Partners, Volunteers and Other Community Members,

In retrospect, 2019 was the last of our previously normal times. It is now even a bit difficult to remember the “pre-Coronavirus” era, but that was only a few months ago. Much has changed, but our mission and our goals at BSF have not. We remain completely determined to help develop treatments for those with Barth syndrome and also make their lives better while we are working on these solutions.



Two milestones achieved in 2019 bear special mention. The first is the completion of our first two human clinical drug trials, and the second was the holding of our first Barth Research Portfolio Review. The FDA is now evaluating the extended results of the TAZPOWER trial for elamipretide in the US, while results of the CARDIOMAN trial using a different drug called bezafibrate in the UK are being analyzed as well. We all learned a great deal during these processes and maintain our cautious optimism about their results, but regardless of the outcome, we will not reduce our momentum. So much progress has been made toward various other treatment possibilities that a special day-long meeting in 2019 was required to hear directly from researchers and to discuss the best ways forward with our Board of Directors, our Scientific and Medical Advisory Board (SMAB) and several additional expert advisors. We had very helpful discussions about several specific drug development prospects and also about gene therapy. This highly regarded occasion will be the first of many as BSF continues to strategically advance clinical therapy development for our community. It was a crucial milestone and one which we had been working toward ever since BSF was created in 2000.

The dire need for treatments continues to be made painfully evident as individuals with Barth syndrome continue to succumb to this devastating disease. We must accelerate our progress, and, to do that, we need for all of us to work together doing our parts toward our common goals. I know that we will reach our goals.

Thank you one and all for being an integral part of the Barth team. Each donation, hour volunteered, introduction made, research project undertaken and patient cared for are essential to the whole. You have no idea how valued your varied contributions are and how much difference each one makes. We thank you sincerely. Please keep up the good work!

Kate McCurdy

Katherine McCurdy
BSF Board Chair

THANK YOU

We believe the most efficient way to finding 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.

\$25,000 +

Cynthia & the Hon. Richard Blumenthal
Abigail Johnson & Christopher McKown
Laura & Scott Malkin
Kate & Steve McCurdy
Tracy & Marc Sernel

\$10,000 +

Barth Italia Onlus
Susan & John Bellig
Branagh, Inc.
Gaylord & Bradley Lummis
Marilyn Lummis

\$5,000 +

Elizabeth Beynon
Matthew Blumenthal
Diane & Tom Branagh
Nancy & Bill Branagh
Meredith & Eugene Clapp
Ann Ingersoll
Patty McCormack
Allene & Dr. Robin Pierson
Dr. Paul S. Russell
Brenda Shapiro
Nancy & Tim Taussig
Jerry Wilkins

\$2,500 +

Katie & Matthew Branagh
Dillon Foundation
Karen & Paul Isaac
Jewish Communal Fund
Scott Jordan
Jill & Matt Korpita
Karen & Phillips Kuhl
Denise Lascurettes & Vincent Mangiapane
Susan McCormack & Ken Marra
Grace & Kent Mitchell
Helen & Peter Randolph
Susan Sherer & Peter Osnos
Dr. Hazel Szeto
T. Rowe Price Foundation, Inc.
Amy & Joseph Wald
Stacey & Kevin Woodward

\$1,000 +

Miriam Acuna
Adobe Corporation
Aerojet Rocketdyne
Amazon Smile
Association Syndrome de Barth France
Stephen Baffa
Tricia & Clarke Bailey
Jon Bauer
Monica & Bruce Bercovich
Carla & Rick Berry
Bill & Melinda Gates Foundation
BNY Mellon Corporation's Community Partnership
Shelley & Michael Bowen
Megan & John Branagh
Nicole & Jenn Branagh-Houston
Nancy & Les Buckley
Marita & Chuck Burmeister
Debbie Campbell
Kate & Frank Canova
Mary Cleveland & Thomas Haines
Natalie & Paul Cohn
Colgate
Brandi & Nick Dague
Lynn Deithorn
Sarah & Nicole Derusha-Mackey
Sherry & Mark Dugan
Tanya & Chris Eklund
Renee Engberg
Matt Engott
Leah & Paul Epstein
Michelle & Angelo Florez
Susan & David Fowler
Linda & Rudy Garcia
Lisa & William Glenn
Jeffrey Griffith
Bryan Hales
Dana Hart
Elizabeth & Jordan Haviland
Peg & Bruce Henricks
Keli & Greg Holly
Kim Hurtz
Mariana & Paul Ingersoll
Deloris & Malcolm Johnson
Suzanne Kavetas
Vivian & Fred Kiechel
Karyn & Don Kintzer
Rachel & Ben Krauskopf
Suzanne & Matt Kroger
Sarah & Dr. Seth Lederman
Marvin Lummis

\$1,000 +

Jenny & Tony McAuliffe
Ginny & Bill McCurdy
Kris & Chris McCurdy
Kathryn & Sandt Michener
Microsoft Matching Gifts Program
Martha & Kirt Miller
Martha Miller
Emily Milligan & Daniel Castro
Eleanor & Walter Minor
John Montgomery
Henry Mueller
Reshmi Paul & Francois Odouard
David Olson
Diane Pattee
George Peyton
Play 4 Cade
Lisa Rey
Beth Roberts & Natan Vaisman
Sharon & Francis Robinson
Dr. Nina Russell
Sara & Paul Russell
Sharon & Gordon Sernel
Kristin & Peter Somers
Linnet Tse
Kelly & Quinn Vidrine
Suzanne & David Wahrhaftig
Gill & Richard Watt
Carolyn & Michael Welcome
Delores & Robert Weltlich
Sue & Mike Wilkins
Carolyn & Larry Woodcox
Deborah Wunderlich
Carlyn & Jon Zehner

\$500 +

James Baffa
Matthew Baffa
Stacey Ballard & Frank Baffa
Amy & Seth Barad
Tom Bellig
Ana Maria & Rick Berry
Taylor Black
Carroll Bogert
Nancy & Ron Bowman
Anne & Andrew Branagh
Kimberly Branagh
Lynette Branagh
Jeanne & David Brody
Tracy & D.W. Brody
Amanda & Jacob Brooks
Rita & James Cain
Linda Cheatham
Andrew Colon
Maura Concannon
Amy & Burt Coombe
Dr. Gerald Cox

\$500 +

Renee & Aaron Croteau
David Himelberg Foundation
Molly & Patrick Devinger
Agnes & Marc Engberg
Julie & William Esrey
The Ford Foundation
Judy & Michael Gewitz
Dr. Iris & Raul Gonzalez
Greg Gordon
Anne & John Grandin
Kathleen & Alex Griffith
Nora & Madison Grose
Dacey Hall
Greg Harrison
Tim Heine
Erin Holliday
Jane & David Joyce
Eileen Juico & Bill Knauer
Shelly Kaiser
Elizabeth Kelly
Karen & Charles King
Ann & Collier Kirkham
Robbin & Fred Kroger
Karen & Peter Kugelmann
Juliette & John Kvernland
Nancy Lascurettes
Jennifer Lee
Susan & David Lehner
Dr. Edward Lesnefsky
Jonathan Linen
Sue & George Loranger
Jun Makihara
Abbey & Stan Massie
Nannette & Mark McNally
Yoko & Steven Michaud
Anne & Steve Milligan
Louise & Marc Morgenstern
Laili Moshirzadeh Moayedi & Dr. Michael Schlame
Kathy & Erik Nemanic
Tina & Ken Olson
Jackie & Bob Purcell
Elizabeth Rutledge
Judy & Dan Schwarz
Heather & Mark Segal
Kathleen "Shi" Shiring
Zdenek Sita
Skyline Chili
Mary Ann & Ted Sonderegger
William Stuhlereyer
Whitney & Zac Thompson
Lindsay & Ryan Wagner
Rachel Wald
Kristen & Lee Wilkins
Ann & Gordon Woodward
Randy Wunderlich
Susan Yadgar
Anne Zeller

THANK YOU

\$250 +

Susan & Andy Alisberg
Barbara Apple Sullivan
Dr. David Axelrod
Michelle & David Baffa
Cristy Balcells
Stacey & Steve Bauer
BD Associate, Charitable giving program
Wesley Bell
Christie Kurys Belscher & William Belscher
Donna & Norbert Bertling
Sarah & Peter Beshar
Susan & Thomas Bognanno
Michael Bornscheuer
Patrice & Michael Botto
Debra Brown
Ellen Bruno
Leslie Buddemeyer
Jocelyn Buly
Cleo Burtis & James Comerford
Elaine Calvert
Paola Cazzaniga & Paolo Muller
Debbie & Stephen Chapin
Brandy Christensen
Lisa & Eric Christensen
Lending Club
Jane Condon
Shirley Courtney
Jean & Robb Craig
Gale Crocco
Kayleigh & James Cumpton
Leslie Dahring
Alain Demers
Philip & Mary-Alice Dennehy
Laura Derusha
Janet & John Dollard
Bryan Drake
Teresa & Frank Drake
Whitney & Adam Dugan
Ann & Tyler Elliston
Thomas Engberg
Julie & Dewayne Fairchild
State Farm Companies Foundation
Brian Feingold
Juliana & Will Fiske
Allie & Nick Garcia
Kristen & Ryan Gehrig
Liz & Ned Hazen
Debbie & Norm Hedgecock
Mark Helfferich
Lucille & Denton Hester
Liz & John Higgins
Amy & Alex Hood
Pamela Hubby

\$250 +

Emily & John Irving
Brie Chandler-Kalapasev & Ned Kalapasev
Helen Kauder and Barry Nalebuff
Charlotte Kelly
Jenifer & Bryan Kester
Joseph Kimmey
Allis & John King
Kristin & Neil King
Valérie Lallemand
Elizabeth Liscio
Christie & Scott Logan
Meredith & Scott Long
Jamie Luttrell-Houlihan
Molly & Sean Maduck
Rosemary & Angelo Mancino
Tricia Mangiapane
Winnie Mann & Carter Hill
Brian Martinez
Gary Mathies
Rebecca McClellan
Patricia & David Melrose
Carolyn & Michael Milligan
Zona Morgan
Tim Murphy
Luke Neidigh
Teri & Scott Nelson
Ruth Nemzoff & Dr. Harris Berman
Pamela & Rick Newacheck
Kim Overaa
Dr. Christina Pacak
Dr. Carl Pergam
Andrea & Carl Peterson
Milene & Jason Petralia
Dr. William Purdy
Jennifer & Dave Roth
Katherine & Adam Rothschild
Mary & Andrew Rotondi
Margo & Harold Russell
Todd Sabin
Dr. Claudio Sandoval
Kathy & David Scadden
Cathleen Schulze
Sage Scott
Anne Segal & Mark Silvershotz
Greg Shepherd
Joanne & Frank Sims
Kirsten & Alex Slawson
Sharon & Robert Stevenson
Denise & Garrett Stone
Linda Strohl
Wanda Threadgill
Marilyn & Dr. Matthew Toth
Birgit & Dan Townley

\$250 +

Emily & Chris Trigger
United Health Group
Marcia VanBuren-Brown
Jerre Vogt
Theda Watson
Giselle & Alan Weissman
Mark Weltlich
Kim Wheeler
William White
Jessica & Mark Wiederspan
Joanne Wilkins Burnham
John Wilkins
Anne & Mike Willcoxon
Jenni Winters
Susan Wintersheimer
Ellen & Michael Wyman
Jeffrey Young
Holly Zierk-Marfaglia

\$100 +

Nancy & Mike Abel
Judith Adler
Karen & John Allen
Maureen & Peter Allman
American Express Employee Giving
Jenelle & Andy Andersen
Jeanne Aronson
Lisa Arter
Charles Arthur
Colette & Ernie Asaff
Michael Aviles
Kevin Bailey
Mary & Jeff Barbano
Alex Barbour
Amy & Cam Bardwell
Mary Gail & John Barry
Peter Barth
Debbie & Rod Basler
Barbara Bass
Jennifer Bater
Judith Bellig
Martha & Bob Berardino
Harriet & Wayne Berens
Kate & Kenneth Bialo
Big Red Publications Company
Robert Binckley
Dave Bingham
Laurie Biscoe
Sally & Pete Blair
Claire Blakemore
Ellen & Dr. David Blumenthal
Rick Bogdan
Amy Bogert & Robert Baldwin

\$100 +

Sally & Nick Bogert
Kenyon Bolton
Jenny Bonawitz
Alanna & Kevin Boozer
Anita & John Bosak
Jill & Tom Boyce
Brandon Boyer
Greg Braselton
Tawnya & Cameron Brooks
Jared Brown
Susan Brown
Dee Browne
Adrienne Buhmann
Char & Larry Burgess
Agnes Burke
Margaret Burke
Jackie & Jon Burmeister
Ashley & William Cade
Sally & Jack Campbell
Frances Cappello
Suzanne Carbery
Betty Carner
Ellen & Terrence Carroll
Alice & Matthew Cavanaugh
Beverly & Norb Cent
Liz Champagne
Darla & Robert Chandler
Annah & Nick Chapman
Joyce & Keith Cheatham
Sharon & John Cheatham
Henry Choo
Patricia & Mike Cimino
Cindy Clark Little
Francie Clark
Rhiannon & Matt Clark
Jeanette Clinkenbeard
Vicky & Paul Cohune
Mark Condon



THANK YOU

\$100 +

Jennifer Connally
Susan Coogan
Austin Corcoran
Diane Crowley
Kelly & Jim Cunniffe
Custom Ink Fundraising
Matthew Cutler
Libby Dafferner
Allison Dale
Dianne & Daniel Danzig
Jennifer Davis
Jules Degive Lallemand
Anna Deignan
Brianna Demers & Andrew Buddemeyer
Katheryn Derusha
BJ Develle
Rico Diaz
Teresa Dillow
Meredith Dolley
Jennifer Donnalley
Marcia Drake Hall
Elizabeth Duffey
Brooke Dugan & Madison Dillow
Sue Dynarski
Madeleine Egueur
Marian Eivins
Carol Enterline
Deborah Ettington & Thomas Burean
Diane Fennimore
Jane Floyd
Sarah Foster
John Franklin
Danielle Freda
Erin & Ronald Galli
Pamela & Doug Ganz
Lin Garcia
Patricia & William Garrett
Dena Garrison
Caroline & Jonathan Garrity
Genentech, Inc
Trevor Gentry
Barbara Gessler & Paul Schwendener
Betsy & Merrill Glasgow
Howard Gleason
Carmen Goitia
Ginny & Bill Goodwin
Amy & Howard Gorman
Melany & Dusty Gray
Laura Grimmett
Lindsay Groff
Sandra Guasco
Missy Halberstadt
Leigh Hall

Melissa Harmon
Yvonne Haynes
Lisa Heck
MaryAlice & Mike Hennessey
Katherine Herr
Randy Hester
Kelsey Higgins
William Hill
Tina & Jim Hille
Christiane & Michael Hope
Shelly Horkey
Nancy Horton
Jennie Iannariello
Ann Ison
Fala Israel
Teresa & Jay Jackson
Liz Jankowski
Jeff Jansen
Cate Jarrett
Clint Jarrett
Rob Jensen
Jose Juico
Bridgette & Matt Kaiser
Jemma Kalberg
Alyssa Katherine
Sue & Ken Kay
Susan & Timothy Kelly
Jenni & Paul Kim
Sharon & Eurus Kim
Kayla & Brandon Kirkbride
Kirkland & Ellis, LLP.
Chloe Kleiss
Anne Knight
Dr. Emily Koelsch Rebori
Arthur Kuhlmann
Becky & Tony Lai
Michelle Lay Foster
Susan Leon & Andy Fredman
Pat & Max Linder
LLR, Inc.
Margaret Logan
David Lyman
Emily & Kevin Lyon
Janie Lyon
Margaret Magee
Mark Manley
John Mann
Shelia & David Mann
Lisa Mansour
Hal Marsh
Ruth & Dr. Martin Massengale
Fenn Matthew
Martha & Douglas Maxwell

Dr. Edwin Maynard
Laura McClendon
Mary McCormack
Elisabeth & Hugh McCutcheon
Molly McFarland
Jmel Wilson & Wendy McFee
Annie McGreevy
Peter McKillop
Gail McNair
Patricia Medina
Noel Melliza
Gail & Doug Melton
David Merchant
Andra Mercier
Trish Messman
Jason Midler
Merle Miller
Marilyn Milligan
Shannon & Daniel Milligan
Jason Moen
Brittany & Nate Morgan
Susan Muaddi Darraj
Annie & Doug Myers
Judy Myers & Ira Schwartz
Robert Myers
Justin Nelson
Laura & Joshua Nelson
Tina & John Nurkowski
Nancy & Steven Olsen
Daphne Pals
Tania Parissi
Amy Parker
Dr. Hitendra Patel
Tejal & Dr. Kepal Patel
Matt Perkins
Audrey Peyroles
Janet & Dr. Colin Phoon
Jacqueline & Jean Paul Plumez
Alison Poritzky
Sally & Paul Prater
Trisha Purpura
Brandon Queen
Maryline Rassin
Emily & Jason Reaves
Jenna & Stewart Rentz
Monique Robidoux
Jane Robinson McFarland
Cathy & Lawrence Rogers
Mary Rogers
Tom Roper
Lois & Dr. Ronald Roskens
Gregory Russell
Mary & Martin Russo

\$100 +

Robert Ryan
Anne & Hugh Sampson
Jennifer Sandling
Kristy Schwabauer
Tresenda Scott
Zdenka Sedenka
Michele & Robert Setterbo
Bernadine Seymour
Kerry Sheehan
Marilu & Peter Sherer
Tony Sherer
Anna Sherwood
Kari Shinsato
Jocelyn & Manny Silva
Craig Singer
Adrienne Skinner
Dr. Herbert Smith
Susan & Kenneth Smith
Fran & Robert Snedeker
Reeny Sondhi
Jennifer & Brett Spitnale
Dr. Jules Spotts
Summer Staino
Jeanne Stevens
Tim Stewart
Donna & Fraser Strain

Ellen & Mike Stuart
Kimberly Stuhlreyer
Timmy Sullivan
Summit Financial Strategies
Kelly Sundberg
Matthew Suzuki
Carolyn & Rodney Swabe
Ginny & Bob Swain
Dr. Carolyn Taylor
Jo Anna & Michael Telles
Christy & Nic Thach
Meghan Thompson Payne
Dr. Nina Tolhoff-Rubin
Daniela Toniolo
Faith Torbert
Deborah Tripp
Scott Truxton
Nora & David Tulchin
Jean Turner
Evelyn Unger
Philippa & Matt Vail
Marco Valentini
Judy Varner
Doreen & Bill Wagner
Brian Walker
Dennis Wallace

Nancy Walsh
Walt Disney Company Foundation
Darren Walton
Helen Waters
Vickie Waters
Linnea Weikleenget
Wendy Weinstein & Marion Karp
Blair Wellington
Philippa & Philip Wharton
Ann & James Wiederspan
Lisa & John Wiederspan
Nancy & John Wiederspan
Family of Marilyn Wilkins
Kristen & Lee Wilkins
Kathleen Williams
Kati Wind
Laura Winston
Steven Woodward
Sheryl Wunderlich
Christina & Cohlby Youtsey
Gail & Tom Zierk
Robin & Greg Zorthian



LEADERSHIP

BOARD OF DIRECTORS

Susan McCormack
Chair (Outgoing)

Katherine R. McCurdy
Chair (Incoming)

David Axelrod, MD
Board Member

James (Jamie) Baffa
Board Member (incoming)

Matthew Blumenthal
Board Member

Megan Branagh
Board Member

Brandi Dague
Board Member

Nicole Derusha-Mackey
Secretary (Incoming)

B.J. Develle
Board Member

Michelle Florez
Board Member

Florence Mannes
Board Member

Emily Milligan
Board Member, ex-officio
Executive Director

Nina Russell, M.D.
Board Member (incoming)

Peter van Loo
Board Member

John Wilkins
Secretary (Outgoing)

Kevin G. Woodward
Treasurer

SCIENTIFIC & MEDICAL ADVISORY BOARD

Michael Schlame, MD
Chair

Prof Peter G. Barth, MD, PhD
Emeritus

W. Todd Cade, PT, PhD

Brian Feingold, MD, MS, FAHAH

Miriam L. Greenberg, PhD

Grant M. Hatch, PhD

Michio Hirano, MD

John Lynn Jefferies, MD, MPH, FAAP, FACC

Erik T. Lontok, PhD
Director of Research

William T. Pu, MD

Mindong Ren, PhD

Colin G. Steward, PhD, FRCP, FRCPC

Arnold W. Strauss, MD

Hilary Vernon, MD, PhD

Ronald J. Wanders, PhD

Katherine R. McCurdy
Emerita

EXECUTIVE STAFF

Emily Milligan
Executive Director

Valerie (Shelley) Bowen
Director, Family Services &
Advocacy

Cristy Balcells
Program & Communications
Consultant

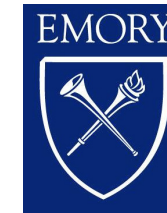
Natalie Cohn
Controller

Erik T. Lontok
Director of Research

Brett Smith
Operations &
Communications

Lynda M. Sedefian
Executive Assistant

PARTNERS



University of Pittsburgh



Depuis 80 ans, nos connaissances
bâtissent de nouveaux mondes