

## Announcing the Iris L. Gonzalez Prize

BSF is thrilled to announce the creation of the Iris L. Gonzalez Prize ("Prize") to advance our collective understanding of genetic variants of Barth syndrome.



Generously supported by The Paula and Woody Science and Medicine Fund, BSF will award a \$10,000 prize to an individual or team proposing innovative use of BSF's acclaimed Tafazzin Human Variants Database. Through this open competition, BSF seeks to identify ideas bearing the most potential to accelerate the mission of BSF with the Prize recipient presenting the results at BSF's 2024 International Scientific, Medical and Family Conference.

The Human TFAZZIN Variants Database ("database") is a critically useful,

noncompetitive resource for the entire Barth syndrome (BTHS) community. As a valuable hypothesis-generating tool, the database has informed research efforts, diagnostic testing panels, as well as families who have sought to understand more about their diagnosis. Initiated 23 years ago and meticulously curated by Dr. Iris L. Gonzalez, the database now encompasses:

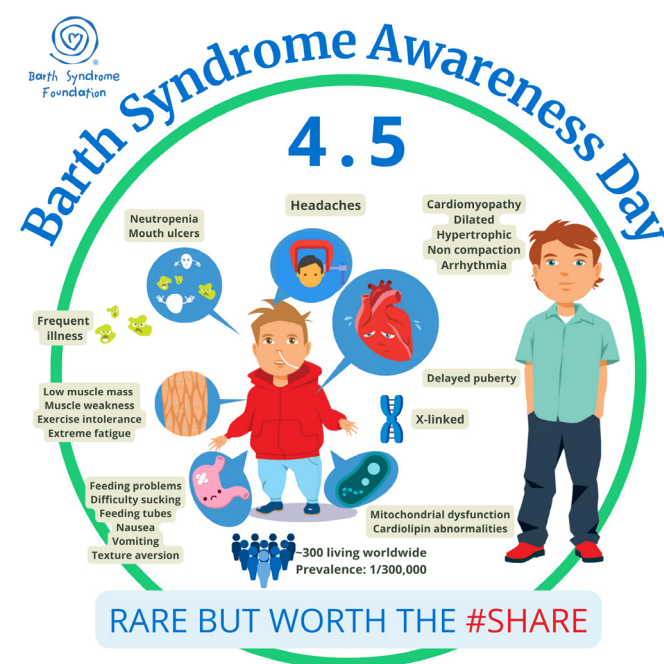
- >350 disease-causing or pathogenic variants
- >140 variants of unknown significance (due to insufficient data or as defined by American College of Medical Genetics pathogenicity guidelines)
- >200 non-specific polymorphisms or benign variants

Alongside the Prize Organizing and Review Committee (ORC), our first order of business is to develop the selection criteria, with a plan to open submissions in July of this year. To keep up to date on everything related to Barth syndrome research, visit [barthsyndrome.org/research](http://barthsyndrome.org/research).

### In This Issue

Gonzalez Prize	P. 1	Strategic Initiatives	P. 8	Happy Heart Week	P. 14
Barth Day	P. 2	AAAS Fellow	P. 11	Hatch Retires	P. 15
Mayo Clinic	P. 4	Looking Forward	P. 12	Board Updates	P. 16
Research GUID	P. 6	SMAB Update	P. 13	Legislation Watch	P. 18

# Barth Syndrome Awareness Day



On April 5, 2023, we celebrated the first-ever Barth Syndrome Awareness Day, bringing together all members of our ultra-rare disease community for a single cause: To increase awareness of Barth syndrome so all affected individuals can access safe and effective treatments. Barth syndrome is caused by a mutation in the tafazzin gene, also called G4.5. For this reason, 4/5 (or April 5th in the United States) is the designated day around which individuals and their families highlight the need for accurate and early diagnosis, advancements in research and development, and access to therapies.

US Congressman Paul Tonko (D-NY) introduced a resolution in the US House of Representatives (H.Res.276) with US Congressmen Gus Bilirakis (R-FL) and Ralph Norman (R-SC) as original co-sponsors to recognize April 5 as Barth Syndrome Awareness Day and highlight the need for increased awareness, improved diagnosis, new therapies for this disease and regulatory

pathways for ultra-rare drug development.

Lynda Sedefian, parent of two sons with Barth syndrome, Eric (who is deceased) and Derek, met with Rep. Paul Tonko [D-NY-20] to discuss his support of the Barth Syndrome Awareness Day Resolution in the US House of Representatives (H.Res.276). Lynda shared her experience as a mother of two individuals with Barth syndrome, the impacts of the genetic disease, and the importance of raising awareness.

The first-hand account of an individual's and family's journey with Barth syndrome proved impactful. Rep. Tonko heard from the caregiver perspective how access to care is limited for ultra-rare, debilitating diseases like Barth syndrome. Raising awareness is an integral step to reducing barriers to care by catalyzing education of health care providers, raising funds for research, and spurring drug development.

"For the millions of Americans living with a rare or ultra-rare disease, precious little information and even fewer treatments are available, leaving patients and their families without options and without hope," Rep. Tonko said. "To that end, I'm immensely proud of my work alongside advocates to pass my HEART Act. This law amplifies the voices of those living with a rare disease and the doctors who treat them, all while accelerating the development and approval of new treatments. But my work to uplift the needs of those living with these rare conditions is far from over, which is why today I am introducing a resolution to raise awareness for Barth syndrome. I'm thankful to the individuals who have shared their experiences with this life-threatening condition, and I pledge to continue working to ensure



Congress meets the needs of those living with a rare disease."

The legislation currently has six co-sponsors who have signed support. "[Rep Tonko's] leadership in Congress to designate April 5th as "Barth Syndrome Awareness Day" is of significant value to the Barth Syndrome Foundation community," Sedefian said. "Thank you so much for raising awareness and elevating our community's voice."

Lynda, a resident in Rep Tonko's district, was pivotal in securing his support of H.Res.276 and catalyzing cosponsors to sign on in support of the resolution. She personalized the Barth syndrome community's plight for treatments and care that will lead to better, longer lives for people affected by this disease.

"No legislation is feasible without the community's advocacy," said Emily Milligan,

Barth Syndrome Foundation's Executive Director. "It literally takes a village, and we need people like Lynda who are willing to give voice to the Barth syndrome community's need for treatments and care options."

"We are tremendously grateful to Representative Tonko, Representative Norman and Representative Bilirakis for introducing bipartisan legislation to recognize the first annual Barth Syndrome Awareness Day," said Emily Milligan, Executive Director of BSF. "We celebrate being heard and recognized, since people living with Barth syndrome and other ultra-rare diseases face significant under-recognized health inequities that can only be addressed through drug development pathways and access policies that have been designed with ultra-rare diseases in mind. Every person with Barth syndrome or any other ultra-rare disease deserves a chance for a better, fuller, longer life."

Thanks to the continued efforts of our community, US representatives Neal P. Dunn [R-FL-2], Nancy Mace [R-SC-1], Doris O. Matsui [D-CA-7], and William R. Timmons IV [R-SC-4] have signed on as co-sponsors of the resolution. If you would like to encourage your representatives to support the Barth Syndrome Awareness Day resolution, visit [barthsyndrome.org/advocacy](https://barthsyndrome.org/advocacy).

## Our Vision:

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



STUDY PARTICIPATION OPPORTUNITY

# Volunteers Needed for Barth Syndrome Research Study



Mayo Clinic is currently seeking research participants that have been diagnosed with Barth syndrome to provide blood samples for a research study. This research study is being conducted by Mayo Clinic in collaboration with the Barth Syndrome Foundation.

**How you can help:**

This study is open to English-speaking children and adults that have been diagnosed with Barth Syndrome (BTHS) by a doctor or through genetic testing.

Participation involves a one-time blood sample collection by having a blood draw at Mayo Clinic OR through a dried blood spot collection (DBS) at home, which can be mailed back to Mayo Clinic.

**Contact information**

For more information, please contact Jenna Capelle at [capelle.jenna@mayo.edu](mailto:capelle.jenna@mayo.edu) or Karen Friedt at [friedt.karen@mayo.edu](mailto:friedt.karen@mayo.edu) or at 507-284-9080.

## STUDY PARTICIPATION INFO

Mayo Clinic in Rochester, MN is currently seeking US-based individuals diagnosed with Barth syndrome to take part in a research study to investigate testing methods to assist in diagnosis.

**Background:**

Barth syndrome is a rare and serious X-linked genetic disorder, caused by a mutation in the TAFFAZIN gene. As a result, individuals with Barth syndrome have low amounts of the lipid cardiolipin (CL) and high amounts of an intermediate lipid, monolysocardiolipin (MLCL) in their blood. Previous studies have shown how this result is unique to Barth syndrome affected individuals, and this research project aims to assess the MLCL to CL ratio in both affected and unaffected individuals to better understand its potential utility in diagnosing Barth syndrome.

**Who may qualify for participation:**

Those diagnosed with Barth syndrome by a doctor or through genetic testing

Are English-speaking children or adults

**Participation involves:**

A one-time blood sample collection by having a blood draw at Mayo Clinic

OR

A dried blood spot collection (DBS) at home, which can be mailed back to Mayo Clinic

**Contact information:**

If you are interested in learning more about this research study, please contact study coordinators Jenna Capelle at [Capelle.Jenna@mayo.edu](mailto:Capelle.Jenna@mayo.edu) or Karen Friedt at [Friedt.Karen@mayo.edu](mailto:Friedt.Karen@mayo.edu) or at 507-284-9080.

For more information, please visit the Mayo Clinic Clinical Trials website at <https://www.mayo.edu/research/clinical-trials/cls-20535806>



Barth syndrome is an ultra-rare disease, so each piece of data generated by participating in research is of outsized importance, and its careful curation is key.

- Our Research GUID (Global Unique Identifier) Program aims to:
- Efficiently connect datasets across many other Barth syndrome research efforts
  - Promote data sharing and thus maximize the utility of each dataset
  - Ensure the fidelity and confidentiality of research participant’s personal identifiable information (PII)

**For Affected Individuals and Families:**

When you participate in a research study, researchers typically assign you an ID number that links to your data within that study. Each study uses its own ID number system, making it really difficult to connect a participant’s data across various studies.

The Research GUID program aims to tackle this obstacle by employing an ID numbering system that can be used to link and share your data across many studies without revealing who you are. It is really important that affected individuals and their family members can obtain a Research GUID number and card by enrolling in the Barth Syndrome Registry and Repository, as this will increase the usefulness of the research data that you have been willing to generate. To get your own Research GUID card, please contact us.

**For Researchers:**

A major challenge for Barth syndrome research is its rarity and the availability of data has a huge impact on scientific progress and treatment development. Our Research GUID is a secure mechanism that can efficiently connect datasets with various other Barth syndrome clinical research efforts by employing a common participant identifier within and across research studies and repositories. By using Research GUIDs, you will be able to link your dataset to the larger Barth syndrome dataset and identify matches, thus potentially augmenting both the data you have access to and maximizing the utility of your data. If you are interested in employing the Research GUID in your research, please contact us.

**How the Research GUID Works:**

A participant shows their Research GUID card so that researchers collect the necessary PII and store that data in a local database that is not made available outside their institution.

- The 9 fields required to generate a Research GUID are all listed on the card:
- Complete legal given (first) name of the participant at birth
  - If the participant has a middle name, the complete middle name of subject at birth
  - Complete legal family (last) name of participant at birth
  - Day of birth
  - Month of birth
  - Year of birth
  - Name of city/municipality in which participant was born
  - Country of birth
  - Sex at birth

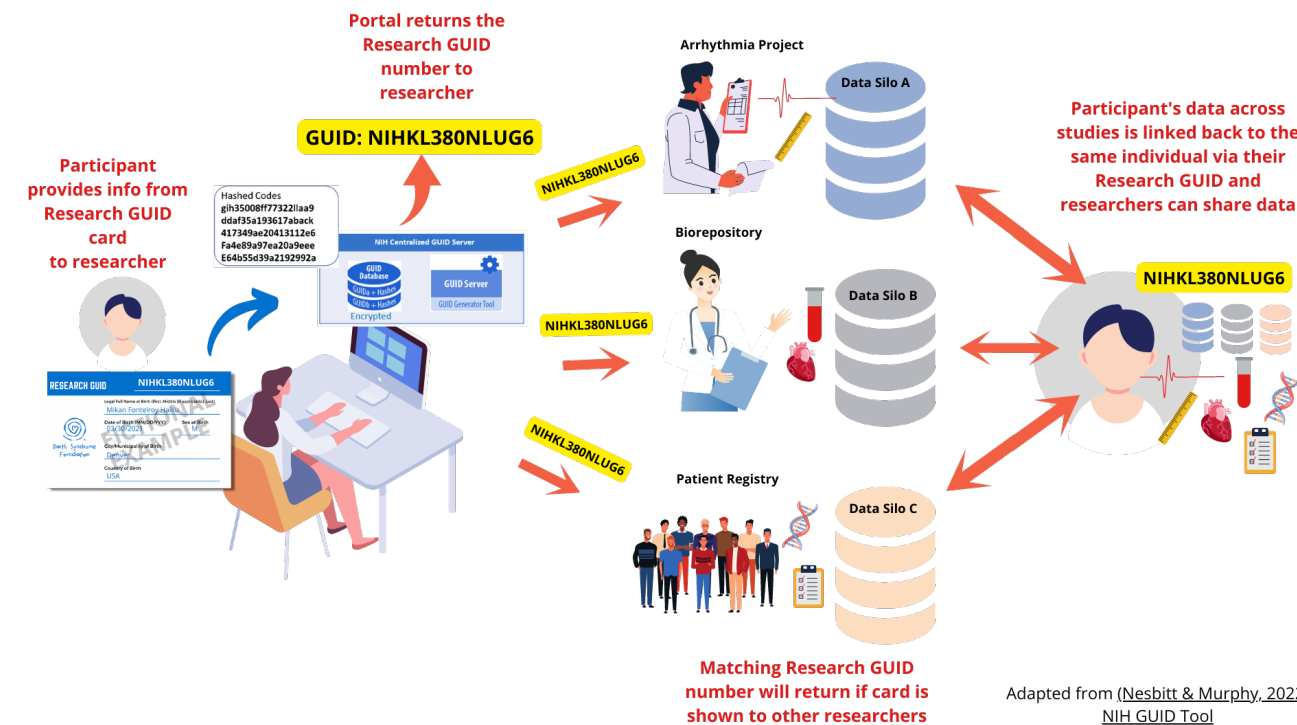
**Advantages of the Research GUID program**

As you can imagine, it is absolutely critical that each piece of this information is given and entered exactly the same way each time so the system does not think you are a different person. BSF equips members of our community with Research GUIDs to ensure the PII given to researchers remains consistent across studies. PII never leaves the organization that is inputting the data. Potential research collaborators will only need to communicate once through this universal, yet unique de-identified participant coding system for data.

Once a researcher enters the necessary PII accurately into a secure NIH-hosted server, it will generate either a new GUID or match to the participant if they already exist in the system. This GUID will stay with that research participant forever, so the process only needs to be done once. Researchers can match their list of Research GUIDs to BSF’s master list and determine if there are matches across other studies and initiate the data sharing process with the other researchers/ institutions.

**Contact Information:**

If you have questions about our Research GUID program, want to get your own Research GUID card, or are a researcher who wishes to implement the Research GUID for your study, please reach out to Melissa Huang, Clinical Research Coordinator at [melissa.huang@barthsyndrome.org](mailto:melissa.huang@barthsyndrome.org)





# BSF Strategic Initiatives Program

BSF's Strategic Initiatives Program is a flexible funding vehicle designed to advance our Barth syndrome research and development (R&D) pipeline. Complementary to our competitive seed grant program, the Strategic Initiatives Program (SIP) is geared to bolstering and accelerating the development of potential therapies and interventions for Barth syndrome as identified in BSF's strategic plan.

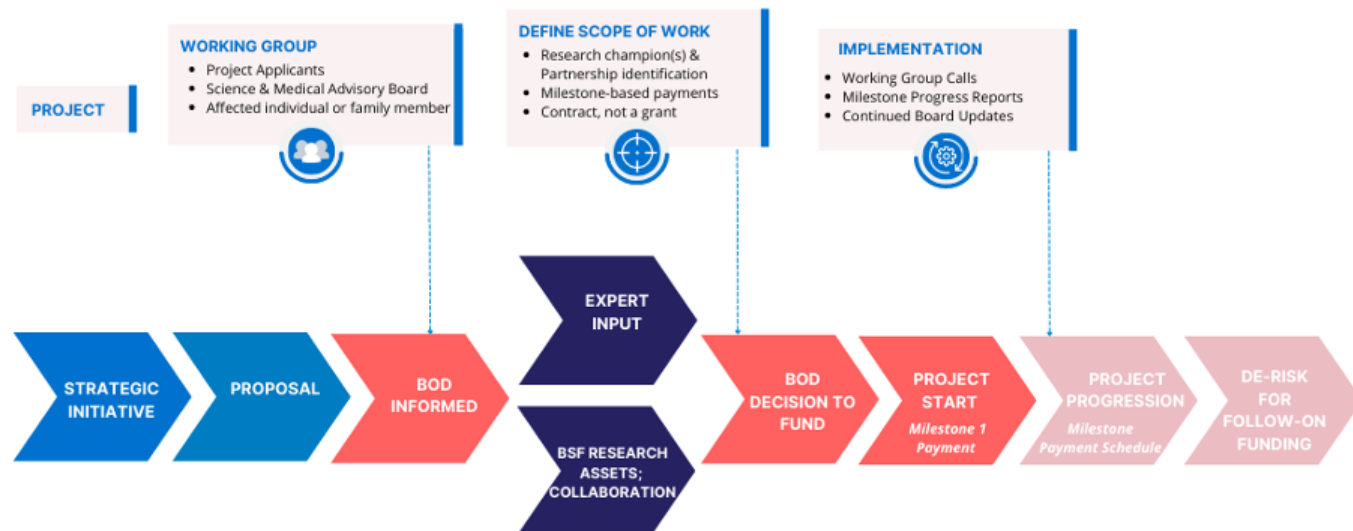
## SEED GRANT PROGRAM

Expanding Barth syndrome biology



## STRATEGIC INITIATIVES PROGRAM

Translating Barth science into medicine



## What is the BSF Strategic Initiatives Program (SIP)?

As part of BSF's strategic plan, specific programs intended to accelerate therapy development are identified as requiring targeted approaches to solving complex technical, manufacturing, clinical knowledge deficits, regulatory, and/or financial obstacles. SIP is a funding vehicle intended to address the challenges of advancing translational and/or clinical efforts to new inflection points and accelerating potential therapies and interventions along the R&D continuum.

## Why did BSF establish SIP?

Since the launch of our peer-reviewed seed grant program in 2002, we have supported research projects that span a range of projects from discovery bench science, to physiological and psychosocial characterization of our population, to innovative cellular and animal models of disease. As the understanding of underlying Barth syndrome science has evolved, so too has our approach. The newly created SIP requires the intersection of several important inputs:

## STRATEGIC INITIATIVES PROGRAM

**Goal:** Accelerate targeted approaches to advance potential therapies & interventions through the R&D continuum.




## How is SIP different from BSF's seed grant program?

BSF's seed grant program calls for novel ideas across the spectrum of Barth syndrome biological pathways and clinical symptomology. Seed grant applications can potentially inform a product development program, or they can generate critical knowledge to inform the field on Barth syndrome biology. Commonly, successful seed grants produce pilot data required for follow-on funding from federal or other outside agencies. BSF issues an open call for applications annually, with awardees announced in the Spring of each year. In short, candidates for funding through this mechanism are usually in the discovery or preclinical stage and do not necessarily require extensive preliminary data to be successful, nor do they need to be tied to a potential development program.

SIP provides a funding vehicle for hypothesis-driven scientific or clinical questions that


may inform or lead to future products or treatments for Barth syndrome. Unlike the seed grant vehicle, these programs have been identified as focus areas in BSF's strategic plan. It is expected that initial data have been published suggesting the potential application of a technology or treatment in the context of Barth syndrome. For product-focused proposals, academic researchers sometimes encounter gaps in funding and/or expertise on their team required to translate an important discovery or early-stage findings to later-stage development opportunities. This funding mechanism seeks to bridge resource deficit(s) and ensure momentum is maintained with the focus on specific areas of research and/or product development for Barth syndrome. This program funds on a rolling basis and incorporates independent expert perspectives as part of the development of a funding proposal, rather than as a traditional peer review process for competitive grant open calls.

BSF assesses potential projects for SIP based on several criteria, which include some of the following considerations:



**Strategic Alignment**

Are the research project's objectives and potential outcomes key to BSF's strategy?



**Funding Vehicle Availability**

Is there another funding vehicle likely to advance the research? If BSF doesn't fund the work, how likely is it to stagnate/delay development?



**Transformative Potential**

How potentially transformative is the target product profile or intervention to attenuate the impact of Barth syndrome?



**Feasibility & Viability**

What body of evidence exists to suggest the feasibility and viability of the possible therapy?



**Milestones**

What milestones must be achieved to heighten the interest of downstream funders to potentially license or support development?



**Resources**

What additional capital is required to mature the technology/intervention for favorable consideration by regulatory affairs and/or investors?



**Research Team Commitment**

Is the research team committed to investing their time and talents specifically to develop a therapy/intervention for Barth syndrome?

What projects have been awarded?

To date, one project has been funded under the Strategic Initiatives Program:

The Optimization of Barth Syndrome Gene Therapy Project, also known as “Novel Capsids Gene Therapy”

Led by Dr. William Pu, this project is focused on the next generation of viral capsids, or packaging, and aims to improve the overall gene therapy package for Barth syndrome.

Researchers interested in learning more about this program should approach Dr. Erik Lontok at [erik.lontok@barthsyndrome.org](mailto:erik.lontok@barthsyndrome.org) or Dr. Melissa Huang at [melissa.huang@barthsyndrome.org](mailto:melissa.huang@barthsyndrome.org).

Dr. Miriam Greenberg named AAAS Fellow!



Congratulations to Miriam Greenberg, Ph.D., professor of biological sciences in the College of Liberal Arts and Sciences at Wayne State University, who has been named an American Association for the Advancement of Science (AAAS) Fellow. Dr. Greenberg, who currently sits on both the BSF Board of Directors and Scientific and Medical Advisory Board (SMAB), received this prestigious honor “for her distinguished contributions to the field of lipid function, particularly for elucidating the role of cardiolipin in Barth syndrome (BTHS) and for identifying molecular mechanisms of control of inositol homeostasis.”

We are truly grateful for Dr. Greenberg’s dedication to and work within the Barth syndrome community.



# Looking Forward in 2023

Welcome 2023!

At Barth Syndrome Foundation, we had a great 2022 and we couldn't have done it without support of donors such as you.

In 2022, we...

- **Invested in Research and Development** allowing us to be able to fund 3 new projects.
- **Supported Barth families** by hosting 4 separate, highly successful Stronger Together World Tour events.
- **Advocated for our community** by working with the FDA and sponsor, BSF resolved access issue through expanded access program. Focus is on regulatory approval so all patients can access the therapy if prescribed by treating physician.
- **Never, ever gave up!** We increased volunteer leadership by more than 20 new volunteers.

We're looking forward to another successful year in 2023. We hope to begin building another center of excellence as a clinical and research hub in Europe as well as identify and implement three or more novel educational and research initiatives. Our families are at the forefront of our mission. We will continue to strengthen and support the Barth community through extending the Stronger Together World Tour into summer 2023 by hosting a gathering in Gurnee, IL.

All that we've achieved and all that we strive to achieve would not be possible without the support of our Barth community of donors, fundraisers, and volunteers. We can't thank you enough for your continued engagement and generosity!

Sincerely,



Emily Milligan, Executive Director  
Barth Syndrome Foundation



July of 2024 will mark the sixth year since we last hosted the BSF International Scientific, Medical, and Family Conference. The good news is that the clock will reset on July 29th because we will once again host our conference at the Hyatt Regency Coconut Point Resort and Spa in Bonita Springs, Florida.

## Here are some good reasons why you won't want to miss this event:



You'll learn about current Barth syndrome research, with opportunities for researchers and affected individuals, along with their families, to meet and get to know each other beyond the science.



You'll have the opportunity to share your experience with other affected individuals and family members to find new practical skills that will help you navigate your daily Barth syndrome journey.



You'll be with people who just get it! It's often lonely navigating life with Barth syndrome and being around others who are on a similar journey is empowering.



You'll make new friends and deepen existing friendships with people you can reach out to even after the conference ends.

**All of this at a luxury resort featuring restaurants, a spa, private beach and more. So mark your calendars for an unforgettable week and we'll see you there!**



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



## Happy Heart Week Raises over \$119K



Every year in May, we celebrate Happy Heart Week (HHW). HHW is an awareness and fund-raising campaign headed up by BSF board member Megan Branagh in honor of her son Henry who has Barth syndrome. Through email and social media, HHW is dedicated to raising funds to support BSF and get the word out about the Barth syndrome. This year, HHW raised over **\$119,000!!!** We are truly grateful to the Branagh family and everyone who donated and helped spread the word.



### Help Support BSF

Since 2000, BSF has been a lifeline for those who suffer from Barth syndrome, offering 24/7 support, pioneering standards of care and diagnosis, creating collaborations between clinicians, researchers, and patients, and most importantly, making sure no person with Barth syndrome is ever alone.

Your support is vital to the success of our mission, so please consider making a gift today.

Donate at [www.barthsyndrome.org/donate](http://www.barthsyndrome.org/donate)



## Grant Hatch Retires from SMAB

Grant Hatch, PhD retired from BSF's Scientific and Medical Advisory Board (SMAB) in April, after being a stalwart and dedicated member ever since BSF's group of research and clinical advisors was created in 2001. Dr. Hatch is a world-renowned lipid biochemist who holds a Canada Research Chair in Molecular Cardiolipin Metabolism. He has had a productive laboratory at the Children's Hospital of Manitoba (Canada) and has been a Professor of Pharmacology & Therapeutics, Biochemistry & Medical Genetics at the Max Rady College of Medicine, University of Manitoba.

After having trained in the Vance lab, one of the leading centers of lipid research, Grant started his career as an independent scientist with pioneering work on cardiolipin. This was at a time when very little was known about this lipid and only a handful of scientists studied it. Grant was then one of the first who recognized the significance of Barth syndrome and redirected his research to study our disease.

Grant has been the recipient of five BSF seed research grants to study biochemical mechanisms involved in Barth syndrome, ranging from the underlying molecular mechanism of the disease to mechanistic aspects of cardiolipin remodeling. Among his many achievements is the discovery of monolysocardiolipin acyltransferase activity in the trifunctional enzyme.

But to those of us at BSF, Grant is also a wonderful, devoted team player who has been a steady and trusted contributor and a real friend. His quiet, calm voice belies the strength of his perspective and wisdom. All of us at BSF have benefitted from his



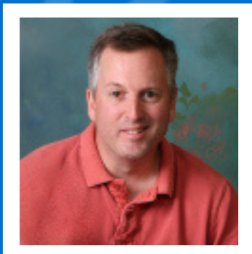
expertise in the regulation, biosynthesis and remodeling of cardiolipin and his interest in our ultra-rare disease. He also has been highly involved in the planning of several of our highly-touted International Scientific and Medical Conferences and in elevating the profiles of new investigators at these meetings. His contributions as a top-notch scientist, even from our early days, definitely helped place the Barth Syndrome Foundation "on the map."

We are all indebted to Grant Hatch, an excellent scientist and a lovely man. Please join us in thanking him for his service to our global Barth syndrome community and in wishing him the very best in his well-deserved retirement.

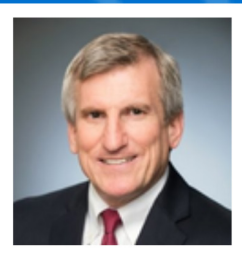


# BSF Board Updates

## BSF Board Update



Kevin Woodward



Mark Greene

After nine years of service, Kevin Woodward retired from the BSF Board in April, having served his Board limit of three consecutive terms. Kevin has been a loyal and involved Board member, not only taking on Board duties but also volunteering to be BSF's Treasurer. Using his corporate "day job" experience at T. Rowe Price, he has helped guide our organization through many pivotal moments, and we are very grateful for all he has done for BSF and for our Barth community. Importantly, he always has offered the Board an important perspective as the father of a Barth son. We have counted on him for his insights and dedication to our cause, and he certainly will be missed! In addition to everything else, Kevin has served as the Master of Ceremonies at several of our pre-pandemic in-person International Conferences and continues to advise BSF on other key initiatives. He has been integral to many of BSF's activities, and we hope Kevin will continue in critical roles within the

foundation despite his exit from the Board.

Stepping into the role of BSF's Treasurer (and therefore also an Executive Committee member) is Mark Greene, a very capable and experienced financial expert who is committed to helping nonprofit organizations serve the communities they represent. Mark earned a BS in Business Administration from the University of California, Berkeley and an MBA from the University of Chicago Graduate School of Business. More recently, he served as the Chief Financial Officer (CFO) of JDRF International, directing a staff of 50 to manage the finance, technology, facilities, investment and compliance functions of the organization that raises funds to support research and advocacy to cure Type 1 Diabetes. He also has held CFO and other senior financial positions for several companies in the fashion industry, including Coach Inc., and in various financial leadership positions at American Express. Mark has been a dedicated and valued member of the BSF Finance and Investments Committee for two years and of the BSF Board for a year. We look forward to benefiting from his expertise as BSF continues to evolve toward being more actively involved in the strategic and financial complexities of developing treatments for our ultra-rare disease, while absolutely keeping affected individuals and their families at the core of our mission.

Please join the Board in thanking Kevin for this service and in welcoming Mark to his new position.

# BSF Board Updates

## Welcome



Jonathan Stokes

Please help us welcome Jonathan Stokes to the BSF Board of Directors. Jon is a Senior Director in Patient-Centered Outcomes Research overseeing neurology, ophthalmology, and specialty therapeutic

areas at AbbVie. With 18 years of experience conducting patient-centered research and generating patient experience data, Jon has a devoted interest in understanding and integrating the patient voice in drug development. He has extensive experience in designing, evaluating, and implementing clinical outcome assessments (COA) to evaluate treatment benefit and has made significant contributions to major product approvals and launches, as well as to the development of multiple novel patient-reported outcome (PRO) instruments for use in clinical trials and real-world practice. Through his work at Adelphi Values, Jon was a lead on the development of a patient-centered outcome tool for Barth syndrome and has continued to work extensively with our affected adult population, earning their trust and confidence as someone committed to the people and the BSF mission. Jon holds a MBA from Northeastern University.

## Barth Syndrome Community Voices

"My son was diagnosed with Barth syndrome in December 2021 and the Barth Syndrome Foundation has been phenomenal as a resource as we continue our medical journey. We use the website constantly to look up recent information on the condition and how we can better assist our son with the issues he may face. It was the first website I came to when I was needing to know everything about Barth syndrome and will continue to be what we look to when we need our questions answered. We are so thankful for the Barth Syndrome Foundation!" --**Chandler L.**

# Advocacy: Legislation to Watch

## Access to Genetic Counselor Services Act

Access to Genetic Counselor Services Act H.R. 2144/ S. 1450 is proposed to address the significant barriers Medicare beneficiaries encounter in accessing genetic counselor services, and these barriers even worsened during the COVID-19 pandemic. Recent evidence also finds disparities to accessing genetic counseling services based on social determinants of health and geographic location. This bill provides for coverage under Medicare of genetic counseling services that are furnished by genetic counselors. Covered services also include incidental services and supplies that would otherwise be covered under Medicare if provided by a physician.

## 21st Century Cures 2.0 Act

The proposed 21st Century Cures 2.0 Act H.R. 6000 builds on the framework and successes of the 21st Century Cures Act, passed in 2016, to advance biomedical research, regulatory science, public health, and payment policy innovation so critical for rare disease patients and families. The draft aims to further modernize the nation’s healthcare pipeline in the hopes of avoiding some of the burdens that the system has faced during the COVID-19 pandemic. Some of the proposed areas for policy include public health and pandemic preparedness, healthcare delivery systems, patient engagement in healthcare decision-making, caregiver integration into the care team, modernizing CMS, and increasing diversity in clinical trials.

## Ending the Diagnostic Odyssey Act

The Ending the Diagnostic Odyssey Act S. 2022 allows state Medicaid programs to cover whole genome sequencing services for certain individuals.

Specifically, states may cover such services for individuals under the age of 21 (or a lower age, if the state chooses) and for former foster youth under the age of 26 who (1) have been referred or admitted to an intensive care unit or seen by a medical specialist for a suspected genetic or undiagnosed disease, or (2) are suspected by a medical specialist to have a neonatal- or pediatric-onset genetic disease.

To learn more about the legislation that we are watching and to get involved, please visit [www.barthsyndrome.org/advocacy](http://www.barthsyndrome.org/advocacy).

## BOARD OF DIRECTORS

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<b>Andrew Buddemeyer, JD</b> Board Member	<b>Miriam L Greenberg, PhD</b> Board Member	<b>Jonathan Stokes</b> Board Member
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## AFFILIATES

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