Barth Syndrome Foundation®

BSF BEAT

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

News and updates from the Barth Syndrome Foundation Spring 2021

BSF Listening Session With the FDA:

Barth syndrome patient and caregiver perspectives on tolerance for less certainty of treatment benefit

In making any health care decision, each individual and his/her family must consider risks and benefits associated with various courses of action. But generally, we spend most of our time and effort thinking about the risk side of that equation: What is the safety profile of the product? How likely am I to experience adverse side effects? What if there is some unknown risk? In serious and life-threatening conditions, the choice to forego treatment itself comes with its own risks – the risks of continued or progressive morbidity and disability in addition to potential severe clinical events and even death. It is well-established that patients living with such chronic serious and life-threatening conditions have a higher tolerance for safety risks, both known and unknown.

On March 3, 2021, the Barth Syndrome Foundation (BSF) held a Listening Session with 28 participants from various areas within the U.S. Food and Drug Administration (FDA) to discuss just this topic. In attendance were representatives from both the Center for Drug Evaluation and Research (CDER) Division of Cardiology and Nephrology and the Division of Rare Diseases and Medical Genetics, as well as representatives from the Center for Biologics Evaluation and Research (CBER). BSF and the global Barth syndrome (BTHS) community requested a meeting, which the FDA welcomed, and then a carefully considered list of specific and highly thought-provoking questions for BTHS patients and caregivers to address was created. This provided first-hand accounts of whether and to what degree the BTHS-patient community would be willing to tolerate benefit uncertainty relative to that which is typically demonstrated in more common conditions for which multiple, large studies are able to be conducted.

But there is another side to all this – the uncertainty of a treatment's benefit. What

In This Issue

if it is not clear exactly what the benefit might be or whether, even if there is positive outcome for some people, it might not occur in all individuals? **This is a common situation that is faced in the rare disease community.** In most rare disease clinical trials, the number of participants involved simply cannot be large enough to produce the high levels of statistical confidence we all strive to achieve. However, patients have not systematically had the opportunity to provide their preferences around their tolerance for less certainty of treatment benefit.

The Barth syndrome community fully understands and completely concurs with a comment made by Dr. Norman Stockbridge (Director, Division of Cardiology and Nephrology, CDER, FDA) during the meeting that not all approved drugs work for everyone. Yet we know that drugs are approved even with this uncertainty. Kate McCurdy pointed out that, by her records, her son tried 92 different mediations (79 of which were prescriptions drugs) during his 28-year life, and they surely did not all give him benefit. If they didn't work, he would move on, but he was very accustomed to that kind of uncertainty and would keep trying new approaches to see what might help him.

She emphasized a point made by one of the discussants in slightly different words: that our community is very concerned about the possible commission of a Type 2 error, also known as a false negative conclusion, in which a drug really works but it is concluded that it doesn't. It would be a tragedy to let a relatively safe, potentially very helpful medication for an ultra-rare disease with no treatments pass by just because the data could not provide confidence levels on a par with those for common diseases. The patients should be given a choice as to whether or not they want to try such a treatment to see if it offers them any benefit.

Representatives of the Barth syndrome community conveyed the following messages:

- There is a dire unmet need for a treatment for our serious and life-threatening ultra-rare disease.
- We are an informed, thoughtful and engaged patient community who are willing to participate in clinical studies and trials.
- There is a high tolerance for uncertainty, including uncertainty of treatment benefit.
- Life itself is a basic human goal, but quality of life also is crucial. Reflecting just how challenging and life-constraining Barth syndrome can be, a number of patients and caregivers even stated that they would choose a better, somewhat shorter life to a much more difficult, longer one.
- We understand that not everything will work for everyone but there is an eagerness to have treatments to try, given the very bleak alternative.

The BTHS community thanks the FDA very much for this opportunity, particularly Chris Melton (Professional Affairs and Stakeholder Engagement Staff, CDER, FDA), Dr. Norman Stockbridge, and Dr. Patroula Smpokou for their partnership in planning this meeting. We are especially grateful for the interesting and truly thought-provoking questions that the FDA posed to us, as well as the time to discuss our patient and caregiver perspectives.

To read the full summary, Please visit:
www.barthsyndrome.org

SCAN ME

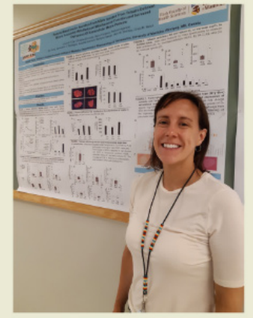
Advancements In Barth Amino Acid And Insulin Research

Impaired and limited metabolism is a central feature of Barth syndrome, with implications in how our affected individuals eat, sleep, and function. A key challenge in our field is understanding how a dysfunctional enzyme (tafazzin) and its abnormal lipid product (cardiolipin, CL) results in altered metabolism. Although much has been impacted by COVID, our research community continues to forge forward in addressing this critical question.

Recently published work by BSF's Scientific Medical Advisory Board (SMAB) member Dr. Todd Cade and colleagues – and involving the participation of five of our affected individuals – has shown 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 both Dr. Richard Kelley's early work and also on Dr. Cade's 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.

Published in January 2021, Dr. Laura

Dr. Todd Cade



Dr. Laura Cole

Cole, SMAB member Dr. Grant Hatch, and colleagues demonstrate a critical role for *TAFAZZIN* in regulating insulin release from the pancreatic beta-cells. Utilizing *tafazzin* knockdown mice, Dr. Cole found that while the total number of beta-cells was unaffected, the amount of insulin they released as well as overall mitochondrial function were reduced when compared to controls. The exact mechanism of how loss of *tafazzin* function results in impaired insulin secretion (and therefore glucose metabolism) merits further research, as Dr. Cade's previous work showed the unusually high dependence of our affected individuals on glucose for energy.

OUR COMMITMENT

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 engage the partners essential to achieve our vision of a world in which Barth syndrome no longer causes suffering or loss of life

2021 Research Grant Awards

Vetted by BSF's Scientific and Medical Advisory Board (SMAB), 2021 grantees' projects span novel areas of Barth basic science, drug repurposing and testing in the heart-specific tafazzin knockout mouse, and remotely conducted clinical research.

Activating Pyruvate Dehydrogenase Complex to Improve Barth Syndrome Cardiac Function

Development Award, $100,000 over two years

Charles E. McCall, PhD
Wake Forest University
Health Sciences

Awarded to the multi-disciplinary team of Professors Charles McCall, Miriam Greenberg, Peter Stacpoole, and Boone Prentice, this Development Award will investigate the drug dichloroacetate's (DCA) impact on the heart-specific *tafazzin* knockout mouse. Involving animal research at Wake Forest University (McCall), cellular and cardiolipin expertise from Wayne State University (Greenberg), and clinical experience and research tools via the University of Florida (Stacpoole & Prentice), this mouse project asks whether DCA can be repurposed as an investigational drug and potential therapy for Barth syndrome.

This project's funding was made possible by the generous support of the Will McCurdy Fund for Advancement in Therapies for Barth Syndrome.

Cardiolipin synthesis and remodeling regulate mitochondrial metabolic plasticity and signaling function

Idea Award, $50,000 over one year

Mauro Corrado, PhD
University of Cologne

Awarded to Dr. Mauro Corrado, this Idea Award builds upon novel findings that cardiolipin plays a key role in the development and function of T cells - immune cells that play a role in adaptive immunity. Utilizing mouse models, Dr. Corrado is focusing on the implications of impaired immune function and its impact on the health and function of mouse muscle cells. Given the immune issues faced by humans affected by Barth syndrome, this effort has the potential to provide a more holistic view of immune dysfunction in Barth syndrome.

This project's funding was made possible by the generous support of the Paula and Woody Varner Fund.

2021 Research Grant Awards

Surveying TAZ genetic interactions and mutational landscape in human cells

Idea Award, $50,000 over one year

Jason Moffat, PhD
University of Toronto

Awarded to Professors Jason Moffat and Charles Boone of the University of Toronto, this Idea Award enables us to better understand the TAFAZZIN gene, in and out. Inwardly, Dr. Moffat proposes to connect changes in gene sequences to their functional consequences on protein function. Known as deep mutational scanning, this effort has the potential to expand our understanding about gene variants in our community. Outwardly, via a CRISPR-mediated genome-wide screen, Dr. Moffat proposes to identify genes that interact with TAFAZZIN and recorded gene variants. By increasing our understanding of TAFAZZIN interactions as well as gene variants and mutations' impact on tafazzin protein function, the research team seeks to identify insights into the variable manifestations, or phenotype, of 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.

"What is Barth Tired?": A mixed methods approach to qualifying and quantifying fatigue in males with Barth syndrome

Idea Award, $50,000 over one year

Stacey E. Reynolds, PhD
Virginia Commonwealth
University

Awarded to Associate Professor Stacey Reynolds and in collaboration with Assistant Professor Virginia W. Chu of Virginia Commonwealth University, this Idea Award aims to capture the fatigue experienced and voiced by our affected individuals. Dr. Reynolds's team will first conduct interviews with affected individuals, siblings, and parents to capture the impact of fatigue on daily living. Dr. Chu will then capture individuals' self-assessment of fatigue in real-time using a novel phone application and map those ratings onto activity data collected by wrist-worn watches worn by affected individuals. This mixed-methods approach aims to qualify and quantify fatigue in our community and is in direct response to the narratives shared during BSF's 2018 Patient Focused Drug Development meeting with the U.S. FDA.

This project's funding was made possible by the generous support of the Will McCurdy Fund for Advancement in Therapies for Barth Syndrome.

BSF Strategic Plan 2021 -2023 "Never, Ever Give Up!"

"What can we do at BSF to have a real impact?" This question has been the driver of BSF's strategic planning process which spanned more than half a year.

While 2020 was an unprecedented and difficult year in many ways, BSF remained strong and continues to make significant progress. BSF works as an agile team. The staff and Board are very committed, and our families, research community, and international affiliates continue to help make our programs and progress possible. Below is the result of our collaborative efforts to determine BSF's focus areas for the coming three years. The strategic plan was presented in detail during our virtual townhall meeting earlier this year, and a recording of that meeting is available on the BSF website: www.barthsyndrome.org/aboutbsf.

SCAN ME

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 Strategy We will invest in research and development (R&D), support Barth families, advocate for our community, and most importantly, never, ever give up!

Invest in R&D

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

- Continue to fund discovery research and tools to improve our understanding of Barth syndrome.
- Invest in a natural history study to make it easier for potential partners to work with us.
- Focus resources on specific research areas, including drug repurposing & disease management, that can improve treatment options for Barth syndrome.
- Pursue collaborations that allow us to advance gene therapy and enzyme replacement therapies.

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.

- Make it easier for families to access critical information about Barth syndrome.
- Bring in outside experts that can help affected individuals and families.
- Be there for affected individuals & families when they are scared, unsure, or in the middle of a crisis.
- Connect families across the world through our conference, outreach events, and online communities.
- Include affected individuals in opportunities to help steer our future.

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.

- Give members of our community the tools they need to champion our cause.
- Promote the interests of our community with state and federal legislators.
- Generate external interest by sharing community stories and the science of Barth syndrome.
- Involve ourselves in product development and regulatory processes when we believe it will help.
- Fund a health impact study and educate payors to improve access to care.

Never, Ever Give Up!

No matter the obstacles, we remain committed to our vision and the people we serve.

- Seize opportunities to progress our mission.
- Unite our community to advance our cause around the world.
- Bring together volunteers, families, and donors to grow our community and make it stronger!

Board of Directors Update

Welcome

Maryanne Chrisant, MD

Andrew Buddemeyer, JD

We want to thank all of the people who volunteer for BSF, including the diverse group of leaders who serve a maximum of three 3-year terms on BSF's Board of Directors!

David Axelrod, MD and Matthew Blumenthal, JD stepped down from our Board in April, after each serving for an incredibly active and important period in BSF history – David for nine years and Matt for six years. David and Matt also are unique in that they were the first people to join BSF's Board who were not family members of someone with Barth syndrome. They also were our first physician and lawyer on the Board, respectively. David's experience, expertise, and perspective as a pediatric cardiologist in California, and Matt's as an attorney and state legislator in CT were invaluable, and both of these individuals were just the right people to help us evolve in very important ways. David and Matt, on behalf of the lives you touched and helped by providing BSF your sage counsel and guidance, thank you! We look forward to your continued association with BSF as lifelong members of our BSF community.

Two incredible individuals Joined the BSF Board also in April, and we are delighted to have them on the team. They bring fresh talent and unique perspectives to our community. Maryanne Chrisant, MD is the head of Pediatric Cardiac Transplant, Heart Failure and Cardiomyopathy at Joe DiMaggio's Children's Hospital Heart Institute in Florida. Her expertise and interests led her to BSF over a decade ago. Maryanne is a thoughtful and passionate clinician who brings academic and scientific rigor as well as direct Barth patient experience to the Board. Also joining is Andrew Buddemeyer, JD. Not only are we very excited to invite Andrew to share his perspective as an adult with Barth syndrome, but Andrew also is a practicing attorney who finds time to serve on his local hospital's advisory board for heart transplant programming as well. We look forward to the many contributions each of these individuals will make to BSF in the coming years.

BSF is truly a volunteer-led and donor-driven organization. The talents and dedication of all four of these special people showcase BSF's unique position to be both a leader in rare disease while simultaneously being very community and family focused.

Please join us in thanking David and Matt and in welcoming Maryanne and Andrew!

Thank You

David Axelrod, MD

Matt Blumenthal, JD

Meet The Community

Jordan and Mikki are the proud parents of two boys, Thomas and Matthew who are 8 and 4 years old respectively. During our interview, you could see them both playing in the background, and easily tell that they are very much fans of each other.

Tommy's and Matthew's journey to a Barth syndrome (BTHS) diagnosis began not with Tommy, but with his younger brother. Matthew was born in heart failure that progressed over time. It was a "series of events over three years that led us to and away then finally back to a BTHS diagnosis."

Jordan's pregnancy was uneventful, but the moment she held Matthew after he was born, she knew something wasn't right. "He was so alert but looked at me like he was scared," Jordan said about those first few moments. The doctors identified his cardiac issues 6 weeks later, but Alaska (where they live) did not have the means to care for his condition. They had to wait for two days for Seattle Children's Hospital to open up a spot and then Matthew was life-flighted there with his mother and his father was able to join two days later.

Matthew spent four weeks in the Cardiac Intensive Care Unit (CICU), where he responded phenomenally to the treatment of his cardiomyopathy (Dilated Cardiomyopathy and Left Ventricle Non-Compaction Cardiomyopathy). It was when he was moved to the observation unit that Barth syndrome came up for first time. Matthew had a spell of neutropenia which got the doctor's attention. "I just thought my baby was born with a bad heart," Jordan said.

While they were waiting for test results, Jordan immediately went to google and found the BSF website. She started reading all the stories and information and thought to herself, "This is my 4-year-old (Tommy), not my newborn."

The TAFAZZIN gene in Matthew initially came back fine along with his neutrophil countss, so BTHS was ruled out. Jordan continued studying all the patient stories and comparing them to both the gene report and Matthew's continued development. She contacted the doctors with her thoughts on Matthew's condition and noticeable delays, but they said it was still probably not BTHS. "I always knew we were missing something," Jordan recalled.

Jordan continued studying the BSF website over the years and eventually reached out to a BTHS mom Kristi Pena who began supporting Jordan and Matthew in their quest. Kristi and Jordan exchanged videos, pics and information over the course of many conversations over the year.

Matthew started having supraventricular tachycardia (SVT) episodes around his third birthday in 2020. Their local cardiologist was referred them to an outside electrophysiologist, Dr. Susan Etheridge. When Dr. Etheridge first

Meet the Community (cont.)

walked into the exam room, she took one look at Matthew and asked, “does he look like anyone else in your family?” After Jordan responded, Dr. Etheridge said, “he has Barth syndrome.”

In March of 2020, Dr. Etheridge sent the family to the Intermountain Primary Children’s Hospital Cardiomyopathy Clinic and Metabolic Genetic Department with the University of Utah Health where Matthew would have a loop recorder placed. It was at this point that Jordan reached out to Shelley Bowen, Director of Family Services and Advocacy at BSF. “We flew out to Utah at the beginnings of the COVID shutdowns (March 13, 2020) for the surgery and genetic testing.” As if the growing pandemic wasn’t enough, while Matthew was in surgery, Utah experienced significant earthquakes. Due to COVID-19 and the earthquakes, the family’s stay in Utah was cut short and they were sent home immediately after the LR placement. BTHS testing continued through the family’s local Alaskan pediatrician’s office and Telehealth.

The doctors sent Matthew’s blood samples to a lab in Amsterdam, where they determined his cardiolipin ratios were consistent with BTHS. Finally having the BTHS diagnosis brought both a sense of relief as well as continued concern tempered by the knowledge Jordan gained from BSF and the community. “As every parent and community member and patient that I’ve met in the wonderful community has stated, it’s not something we wanted to be a part of,” Jordan explained. “But the answers and quality of life that has improved as a result—I can’t even describe it! Knowing the demon my boys and [we] are fighting is so empowering.”

Jordan and Mikki still sit down some nights and continue to study BTHS, because if they need to take Matthew or Tommy to the hospital, they may run into a many doctor’s who have never heard of the disease. Jordan’s advocacy for her boys isn’t limited to medical settings. She consistently fights against labels like “lazy” in their everyday life.

“The hardest part about advocacy is how young and healthy they look from the outside,” Jordan said. “[People] don’t see how much of an everyday effort and interventions it takes to get them to that point.” Jordan said she is well known around the community as a strong advocate for her boys. Their diagnosis “emboldened a part of me that wasn’t there before”

Jordan made a point of saying, “I like to advocate for the dads in the community too” While it’s Jordan’s face we see most of the time, Matthew’s and Tommy’s dad, Mikki, is right alongside the family in all of the appointments, studying material, advocating, doctor visits, therapy appointments and activities. “When it comes to the care and the needs of the boys, it’s not 50/50, it’s 100% from both of us, 24/7” Jordan said with a smile.

As a family, they enjoy outdoor activities like camping, fishing, skiing, sledding, and gardening. As an active Alaskan family, they are learning to adjust and adapt activities for the boys, especially the more physically demanding activities. Jordan describes Matthew and Tommy as “incredibly intelligent, charismatic, caring boys” and “there is nothing about my boys or their lives that doesn’t make me extremely proud.”

At the end of the interview, we asked Jordan two questions, the first being: is there anything you would like to say to another family going through the diagnosis journey? “Don’t give up. It sounds so simple, but it’s one of the hardest things to do sometimes when facing all of the unknowns.” She also added, “I’ve always wanted to say thank you to Kristi Pena for believing in me.”

The second question we asked was: what is one thing you would like to tell your past self about your family’s journey? “I tell my past self all the time, how proud I am of myself. Particularly for not suppressing the questions and persevering through the journey to diagnosis.”

STAT Act: Speeding Therapy Access Today

BSF is proud to partner with EveryLife Foundation in support of H.R. 1730/S. 670, a bipartisan, bicameral, community-led bill aimed at improving the development of and access to therapies for the rare disease community.

The STAT Act will enact targeted, impactful, and attainable policy reforms at the Food and Drug Administration (FDA) to accelerate development of therapies across the spectrum of rare diseases and disorders and facilitate patient access to such therapies.

The STAT Act will:

- Improve rare disease coordination, stakeholder engagement, and policy development within FDA by expanding existing authority to create a Rare Disease Center of Excellence
- Inform rare disease policies and actions by creating a Rare Disease and Condition Drug Advisory Committee
- Fund regulatory science and related activities to support development of therapies to treat very small rare disease populations
- Strengthen rare disease patient access to FDA-approved therapies in both public and commercial plans through enhanced FDA and Centers for Medicare and Medicaid Services coordination, proactive engagement of payers, and specific actions intended to strengthen Medicare and Medicaid beneficiary access to novel therapies

The FDA already has authority under the 21st Century Cures Act to establish Centers of Excellence (COE). The COE would not supplant any authorities held by the FDA review divisions. The Rare Disease Center of Excellence would be cross-cutting, capacity-building, and consultative to support review of rare disease applications but would not supplant any authority of the existing Centers. Three years ago, the FDA established the first FDA Center of Excellence, focused on oncology, which has been extremely successful in bringing new cancer therapies to patients.v

“Between 93% and 95% of the more than 7,000 known rare diseases have no U.S. Food and Drug Administration approved therapy” –EveryLife Foundation

Fore more information, including how to take action, visit: www.statact.org

Volunteer Spotlight

Christiane Hope

We extend a special thank you to our longtime volunteer, Christiane Hope!

"I cherish the opportunity to spend time with other families…having a heart-to-heart with a few other moms, watching the affected individuals and their siblings grow up, evolve and take charge… I think BSF gives you a connection not found elsewhere," she says.

Chris has served in many volunteer roles at BSF and Barth Syndrome Foundation of Canada since her first conference in 2002. From supporting families to planning the conference agenda to fundraising, Chris is always an able and willing contributor and a core volunteer in our organization.

Thank you, Chris, for your dedication to Barth syndrome families everywhere!

Thank you, Dr. Carolyn Taylor, long-time volunteer and advisor to BSF!

Carolyn Taylor, MD

Dr. Taylor attended her first BSF conference in 2002 as part of a collaborative effort to begin a systematic evaluation of cardiomyopathy in people with Barth syndrome.

Nearly 20 years later, Dr. Taylor is still extremely involved. "The greatest satisfaction and biggest reason I like to stay involved is the wonderful group of families and affected individuals, as well as the scientists whowork together to improve the health and wellbeing of this patient population with a rare disease," she says. "BSF is a terrific organization that advocates tirelessly for all people with Barth syndrome, and the progress over the years in the scientific front is astounding. The basic scientists, translational scientists, clinical scientists and industry working together is a model for other rare diseases."

Thank you for your dedication to this community, Dr. Taylor! We are so grateful to you!

Advocacy in Action

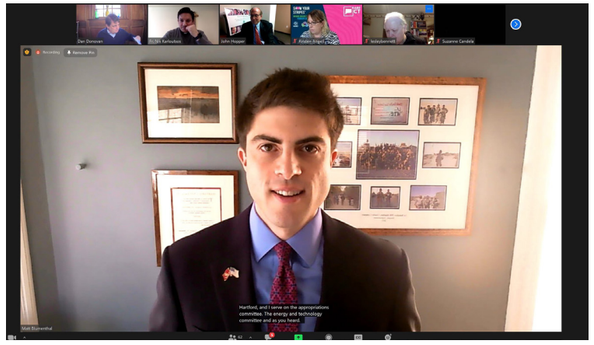
4th Annual
Gene Therapy for Rare Disorders

BSF's Executive Director, Emily Milligan, was an invited panelist at The 4th Annual Gene Therapy for Rare Disorders Digital Event in February.

She joined an elite and diverse panel of industry and government speakers on the manufacturing and commercial potential of gene therapies for the rare disease community.

Speaking alongside Jill Dolgin (Head of Patient Advocacy AGTC) and Jayne Gershkowitz (Chief Patient Advocate, Amicus Therapeutics), Emily spoke about the importance of incorporating the patient voice at every stage of the program.

Matt Blumenthal Advocates for Rare Diseases



Connecticut State Representative Matt Blumenthal spoke on behalf of BSF as part of nationwide initiatives in the month of February to advance awareness and support focused on rare diseases.

"It was an honor to represent BSF during the CT Rare Disease Day event. 'Rare' diseases are not, in fact, rare — roughly 10% of Americans live with one. In honor of the individuals and families who are affected by Barth syndrome, we will work together to ensure the rare disease community receives the recognition and resources it deserves. Thanks to everyone in it — affected people, families, allies, healthcare professionals, scientists — for your work to get us to this point."

Thank you, Matt, for championing our community!

BSF Core Values

Credibility • Integrity • Inclusion • Compassion • Professionalism

Research Being Conducted: Fatigue in Barth Syndrome

Dr. Stacey Reynolds from Virginia Commonwealth University is conducting a research study examining fatigue in Barth Syndrome. She will be looking at how fatigue in individuals with Barth Syndrome impacts daily routines and roles. She is asking for individuals with Barth, and their family and close friends to participate in interviews and focus groups via Zoom. Participation requires about **30-90 minutes** of your time.

You are eligible to participate if you:

- Are an individual with Barth syndrome age 5 years or older OR
- Are age 12 years or older and are a parent, sibling, close family, or close friend of an individual living with Barth Syndrome
- Are able to understand and answer questions in English
- Have access to the Zoom platform (or a telephone if needed)

Interested in participating or want more information?

Email the research team at: dawe2@vcu.edu OR

reynoldsse3@vcu.edu

Corona Yoga: Still Going #BarthStrong

In March of 2020, life changed. No matter where in the world you lived, businesses and schools shut down. Given the stress of living with or caring for someone with Barth syndrome on top of the pandemic, BSF began to offer free online yoga classes for the Barth community last year.

Now, nearly one year later, “Corona Yoga” is still going strong, much to the surprise of those who participate. Instructor Cristy Balcells has served in a communications and advocacy support role for BSF since 2018. “As a mom to a handicapped child with mitochondrial disease who was home from school, I was stressed. And as a nurse and yoga instructor, I knew the value of movement and mindfulness. I wanted to share that with the Barth community.” Cristy began offering classes 4 times a week via Zoom, and most of the attendees had never done yoga before.

“I think about the times I’ve watched a yoga class from the other side of the door at the gym, and thought *I wish I could join*, but I was afraid. Now I absolutely and wholeheartedly have fallen in love with yoga, it is now part of me, and I am unashamedly proud of myself for trying”, says Allanna in Scotland. Even her husband Tommy joins the sessions. “To my amazement, not only did I enjoy the workout on my body, but the mindfulness aspect was really helpful”, he says.

Yoga is considered a “lifelong practice” because it can be adapted to people with many different abilities. And despite the stereotype, it’s not all about being flexible. “I love that my shoulder and back pain have decreased so much, and that I can now do side planks and planks,” says Michaela. But she notes, like most of the participants, that the mindfulness practice has been very powerful. “It’s helped me change my mindset from focusing on what I don’t have to being grateful for who I am, what I can do, and for all that I have.”

Chris started doing yoga when his hockey team cancelled practices and games. “I love the interaction with the others in the group (even though I’ve been told I make a lot of noise in class). So far my biggest accomplishment has been being able to touch my toes.” Classes are offered online at no cost to interested members of the Barth syndrome community on Mondays, Wednesdays, Thursdays and Saturdays at 9:30 am Eastern time. For more information, contact Cristy at: communications@barthsyndrome.org

Barth Syndrome® Foundation

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