



Barth Syndrome
Foundation

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Barth Syndrome Journal

Volume 18, Issue 2 ~ Fall 2018

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

BSF Makes History with PFDD Meeting in Florida

By Emily Milligan, Executive Director, Barth Syndrome Foundation (BSF)



July 18, 2018, went down in the record books for the Barth syndrome (BTHS) community. BSF became the 14th organization of more than 7,000 rare diseases to host an externally-led Patient-Focused Drug Development (PFDD) Meeting with the U.S. Food and Drug Administration (FDA).

The PFDD meeting was a pivotal event in BSF's history to increase awareness and educate the FDA about the challenges of living with BTHS and influence regulatory decision making. The half-day event followed the successful model that the FDA developed to host similar meetings and focused primarily on a range of viewpoints of BTHS. Panelists and speakers covered symptoms and impacts on daily life that are most important to affected individuals and their perspectives on existing and future treatments. Shanon Woodward from the FDA's Center for Drug Evaluation and Research, commented, *"We are incredibly grateful for the opportunity they provided us in sharing their stories."*

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BSF leads the global research and advocacy efforts to create a world without BTHS, while providing ongoing education and community for affected individuals and their families. As a result, BSF decided to approach the meeting differently. *"We took a calculated risk to hold the PFDD meeting in Clearwater, Florida, and not in Washington, DC,"* said Shelley Bowen, Director of Family Services. In keeping with the underlying value of the PFDD meeting, BSF capitalized on the largest turnout ever of its biennial international conference and appealed to the FDA to come to the community's meeting.

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SciMed Sessions of 2018 BSF Conference Impress

By Matthew J. Toth, PhD, Science Director, Barth Syndrome Foundation (BSF)

Every two years, the BSF conference gathers together physicians and researchers to focus on Barth syndrome (BTHS) and what can be done to find treatments for individuals who suffer from this unique mitochondrial disease. Every two years, the progress made towards realizing this goal is impressive, and the 9th conference in 2018 continued this long and remarkable tradition. The first pharmaceutical clinical trial specifically for BTHS, TAZPOWER, began after the last BSF conference in 2016. The BSF community is now experiencing what it has long wanted—an era where its boys/men are involved with pharmaceutical clinical trials. Underscoring this new era of participation and community involvement was the unique externally-led Patient-Focused Drug Development Meeting with the FDA that took place on Wednesday, July 18th during the BSF conference (see article on pages 1/4-7). This 5-plus hour meeting was a riveting event for all and was recorded for those who could not attend in person. The next two days of the Science and Medicine sessions demonstrated the advances made since 2016 and highlighted the need for continuous volunteer involvement to fully realize the common goals of all.

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Dear BSF Community

By Emily Milligan, Executive Director, Barth Syndrome Foundation (BSF)



Emily Milligan

It is an exciting moment in my tenure as your new Executive Director to be able to write this letter and share some perspectives on the activities of Barth Syndrome Foundation (BSF) over the past six months as well as our aspirations and goals for our future. We are deeply grateful for your partnership and your generosity as we tackle together our shared goal of treating and curing Barth syndrome (BTHS). If you are reading this letter, you have likely been touched by the devastating effects of BTHS, either personally or through a loved one or friend. This shared experience provides a tightknit community critical to our historical success and the new paths we will chart together.

I want to take this opportunity to share with you my vision for the organization and some of the important work we are already doing to bring these goals to fruition.

- **Bringing Great Science to Individuals and Families.** Beginning in 2002, BSF launched our seed grant program to fund novel ideas with scientific potential for BTHS. Due to the successes of this program, much of the early stage research funded by BSF is now progressing into clinical studies. We are capital limited, though, and serve a small number of individuals available to participate in trials. This means we will be forced to make some calculated strategic decisions to support programs that have near-term promise of clinical impact. To guide our thinking, BSF will hold a portfolio review in 2019 with world-renowned experts to define BSF's support of the various potential pathways in the clinical space. These include possible treatments focused on genetic replacement and repair, mitochondrial-restorative agents, and other technologies with scientific potential.
- **Fundraising.** In a 6-week timespan, BSF pulled off a herculean feat and raised just under \$100,000 to support the externally-led Patient-Focused Drug Development (PFDD) Meeting with the FDA held in July 2018. As mentioned in the Susan McCormack's Chair letter, BSF received a \$1 million anonymous gift following the PFDD meeting. Such a large vote of confidence in our work is extremely gratifying, and we look to continue the momentum to stimulate additional gifts to support the important work BSF is doing. Giving Tuesday is on November 27th, and we already have secured one match of \$10,000. We raised more than \$60,000 on Giving Tuesday 2017 and are looking to surpass this figure in 2018. We will only achieve this goal if everyone joins in and helps out. Nicole Derusha-Mackey and Megan Branagh have volunteered to lead this effort, and we are enormously grateful for this. Please make a contribution on November 27th, become an ambassador within your social networks, and help us to identify other potential sources of philanthropists and corporate sponsors.
- **Continued Team and Capacity Building.** Great people are ultimately what drive great results. BSF is fortunate to have a small but experienced core of dedicated professionals who bring tremendous value to our mission, and effective communication is critical to their and our success. This is why we brought in a communications consultant to improve the quality and frequency of information you receive about the important work we are doing. In 2019, we will be developing and distributing communications specifically tailored to your interests and in ways aligned with your preferences. We are encouraged that these new measures will deepen your appreciation of, and commitment to, our cause and expand visibility across new constituents we hope to convert to our mission.
- **Partnering to Grow BSF's Sphere of Influence.** Our priorities and opportunities for impact extend beyond our research program. We see a growing interest in, and potential for therapies, but we are aware of challenges ahead. Partnering across the various groups in research and development, including industry, academia, legislative, and regulatory, will be critical to spearheading treatments and cures for BTHS. We have already begun many

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Dear BSF Community

(Cont'd from page 2)

efforts in this regard. At the PFDD meeting, 310 individuals attended either in person or through the webcast, including seven FDA officials from the Center of Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER) of the U.S. Food and Drug Administration (FDA). In addition, staff have been on stage and contributing to the dialogue at the Rare Diseases and Orphan Products Breakthrough Summit organized by the National Organization for Rare Disorders (NORD) and the National Health Council's (NHC) Chief Medical/Scientific Officers Conference. Most recently, I had the opportunity to attend the Future of Health Summit hosted by the Milken Institute where I was able to engage a panel on regulatory concerns facing small foundations such as BSF.

I know I speak for everyone on the BSF staff when I say that passion, dedication, and will for a better tomorrow are the beacons that guide our daily thoughts and actions. We are so grateful for your partnership and generosity, and your commitment to make a difference in the lives of people affected by BTHS. Please do not hesitate to contact me at (646) 937-4123 or Emily.Milligan@barthsyndrome.org should you like to discuss our plans or wish to offer other comments.

A Time for Reflection and Thanks

By Susan McCormack, Chair, Barth Syndrome Foundation (BSF)



Susan McCormack

As I sit down to write this letter, the beautifully-colored leaves outside my home office window are falling from the large maple tree in our back yard. As this year is ending, I find myself reflecting on 2018 and appreciating all that BSF has accomplished through the hard work and generosity of our international community. Together, we are making significant strides toward therapies – and eventually a cure – for Barth syndrome (BTHS). In this newsletter, you'll read about many of BSF's recent accomplishments. I am profoundly grateful for every one of them – from the smallest donation to the largest international meeting. Each step we take together as an organization brings us that much closer to our dream of a world in which BTHS no longer causes suffering or loss of life!

I want to inform you all about three important developments at the Foundation for which I am particularly appreciative.

First, the BSF Board of Directors welcomed two new members at its October meeting: Brandi Dague and Michelle Florez.



Brandi Dague

Brandi lives with her husband, Nick, and her two children in Hollywood, FL. Her daughter, Adeline, is five and her son, Deacon (BTHS), just celebrated his third birthday. Brandi helped organize the Carrier Sessions at the recent conference and is active in the volunteer group dedicated to this issue. Brandi also organized the highly praised grandparent gathering during the 2018 conference.



Michelle Florez

Michelle's elder son, Michael Telles, passed away from Barth syndrome in 2009 at the age of 7. She currently lives in Kyle, TX, with her husband, Angelo, their daughter, Angelina (4), and her

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A Time for Reflection and Thanks

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son, Matthew (13). Matthew has volunteered as a healthy control in BTHS research since 2014. Michelle has served in various volunteer roles for BSF for many years, including attending volunteer workshops and serving on family service and conference committees.

I speak for the entire BSF Board when I say that we are deeply grateful for Brandi and Michelle's willingness to take on the increased responsibilities that Board service entails.

Second, the BSF Board has set up three new working Committees which have attracted a number of energetic volunteers. These Committees, described below, will assist the Board with the internal workings of the organization, increasing the Board's capacity to focus on the strategic future of the BSF.

Finance & Investments Committee: Periodically reviews the actual financial results of the Foundation versus budget, evaluates BSF's investment strategies and assesses BSF's overall financial health. Chaired by Kevin Woodward, Committee members include myself, Rosemary Baffa and Kelsey Bastian. This Committee will be working on BSF's 2019 budget over the coming two months.

Audit Committee: Working with BSF's professional auditor, produces an annual audit which correctly reflects the organization's financial position and makes appropriate filings with the United States IRS. The Committee also reviews BSF's risk policies and insurance coverage annually. Chaired by Steve McCurdy, this Committee includes John Wilkins and Bill Belscher as members.

Governance Committee: Nominates members to the Board, educates new Board members, evaluates BSF's By-Laws as well as its various policies and procedures, and annually assesses the performance of members of the Board. Chaired by Megan Branagh, Committee members are Nicole Derusha-Mackey, Florence Mannes, Michaela Damin, Chris Hope and Tracy Torbert.

BSF is fortunate that so many volunteers have stepped forward to work on these new Committees, and we thank them for their commitment to our mission.

Lastly, just after the 2018 conference, BSF received a substantial donation of \$1 million from a donor who wishes to remain anonymous. This money, dedicated to the Will McCurdy Fund, is to be used for the advancement of therapies to treat Barth syndrome. While we are, of course, overwhelmed and humbled by this donor's generosity, it confirms to us that our donors believe that we are on the right path to reach our goals. We thank this donor – and ALL of our donors – for their commitment to our mission and their trust in BSF.

As I look forward to 2019, I see so much hope for us all. We have accomplished much in 2018 and are poised for significant advancements in the years ahead. We are focused on new, life-changing treatments for our BTHS individuals and their families and have several potential candidates in our development pipeline. For volunteers, for donors, for scientific and therapeutic advancements, for our wonderful, supportive and loving community – for all of this, I could not be more thankful.

BSF Makes History With PFDD Meeting in Florida

(Cont'd from page 1)

"Hope! That is why we have come together today. Together we forge new collaborations. Together we chart new pathways for meaningful therapies. Together we dream and mobilize around a world without Barth syndrome." ~ Emily Milligan, Executive Director, BSF

More than 25% of the Barth syndrome community representing more than 12 countries, converged to voice experiences and perspectives of living with and caring for someone with BTHS. Marc Boutin, Chief Executive Officer of the National Health Council, who has been a long-standing advocate of collecting input and information from patients to inform drug research, remarked, *"The participation by the Barth community demonstrates the power of the patient voice to inform the development of new treatments."*

BSF's goal is to deliver effective therapies into the hands of affected individuals and their caregivers. There is only one way to achieve this goal: Collaboration across the research and development (R&D) continuum. In addition to 7 members from the FDA, 28 members from industry and 40 academic researchers joined 204 caregivers and affected individuals to participate in the event. *"This was a community experience, with people and professionals from around the globe working for one cause,"* said Matthew Toth, BSF Science Director.

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BSF Makes History With PFDD Meeting in Florida

(Cont'd from page 4)

In looking forward, the conclusion of the PFDD meeting represented a beginning, not an end. The Voice of the Patient report is scheduled for an end of 2018/early 2019 release and publication to the FDA's website. The report will further BSF's contribution to inform researchers and regulators alike, to prioritize clinical outcomes and to accelerate approvals of therapies in order to strive for a world in which there is no longer loss of life or suffering from BTHS.

Furthermore, and if not more important, the PFDD meeting prompted an honest discourse of courage and hope to further unify the Barth community. *"The PFDD meeting was great. It was like hearing everything I had been keeping in my head about my son. It wasn't easy to hear, but it was necessary. Now let's do something about it!"* said Joe Wald, parent of a young son with BTHS. BSF is more united than ever, in part because even more people now have joined the quest for a better future for affected children and adults, their families, and generations to come.



Emily Milligan
(Photo courtesy of BSF 2016)

Profiles in Courage: The Externally-Led Patient-Focused Drug Development Meeting

By Arnold Strauss, MD, Professor of Pediatrics, University of Cincinnati and Cincinnati Children's Hospital, Cincinnati, OH; Scientific and Medical Advisory Board, Barth Syndrome Foundation (BSF)

I hope you were present on July 18, 2018, with me and many members of the BSF Scientific and Medical Advisory Board (SMAB) at the afternoon long externally-led Patient-Focused Drug Development (PFDD) Meeting. If you were not able to attend, please find five hours to view the event at <https://www.barthsyndrome.org/newsevents/pfddmeeting/livestreaming.html>. It was an extraordinary event for several reasons.

First, it was, we believe, the very first time that Federal Drug Administration (FDA) officials travelled away from Washington to attend a PFDD meeting. Second, it was an opportunity for all of us involved in BSF to focus on the long awaited possibility that drug therapy to improve Barth syndrome (BTHS) is on the horizon, a welcome development, long overdue. Third, the presentations, family and patient videos, and discussion provided poignant, impactful, and emotional descriptions of the clinical manifestations, family burden, and difficult outcomes for all to hear and feel. For the FDA representatives, this was a time to learn about BTHS and the need for intervention and specific therapies. This was, of course, the goal, and the goal was admirably achieved for all of us.



Arnold Strauss, MD
(Photo courtesy of Amanda Clark 2016)

Fourth, and to me the most incredible, the afternoon was a **"Profile in Courage"**. This is the title of a book by then Senator Jack Kennedy, read during my youth, but it is a highly apt description of those who presented, commented, and participated. **Courage** because of the open, frank, and difficult subjects mentioned, including the deaths of beloved family members, sons and brothers; of struggles with addiction and depression; of fighting to live through chronic fatigue every day; of taking many medications and shots to fend off infection and heart failure; and of nutritional challenges. **Courage** because of knowing future outcomes, while dealing with those symptoms. **Courage** to participate in clinical studies despite fatigue, uncertainty, pain, and the stress of doing so because BTHS patients and parents know that this is the only path to new knowledge and finding new therapies. And to participate in such studies over many years of attendance at BTHS meetings without the certainty of benefit to oneself. I know from discussions with several SMAB members who attended and with many family members that the **courage** of the participants was obvious and greatly admired. And, that **courage** to be open and frank provided even greater incentive to the scientists and clinicians to give our best, to push forward as quickly as possible, and to make a difference for BTHS patients NOW. I have been at several BTHS meetings, worked in the laboratory with BTHS animal models, and cared for BTHS patients for over 20 years. The PFDD meeting was the most concentrated and forceful lesson to go faster that I have experienced in all of those years.

During the PFDD meeting, I was seated with one of the FDA representatives, a biochemical geneticist. Both by watching her response and talking with her during the breaks, it was apparent that the message got through. We shared some tears during the presentations, we watched every second of the videos, and we never touched our smart phones. I am certain that this event will carry over to the time that the FDA reviews potential treatments and will help to expedite that review so that interventions can move forward. I also believe that the impact will go beyond BTHS because so many other rare genetic disorders require similar review. I think the BTHS story will facilitate approval of interventions, including gene therapies, for other rare and orphan diseases through the FDA.

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BSF Makes History With PFDD Meeting in Florida

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For myself and the members of the SMAB, I thank the presenters at the PFDD meeting, those who commented, and all of the BTHS families and patients for your amazing **courage**, your honesty, your humility, your hard work, and your friendship. On July 18, you were a **"Profile in Courage"** for all to see and hear. You made a difference for the future.

The Personal Growth of a Panelist

By Peter (age 31, BTHS), The Netherlands

The PFDD meeting was a huge success in many ways. Not only did we succeed in painting a compelling picture to the FDA about how much we are willing to risk for a possible cure or treatment of the entire syndrome rather than individual symptoms, but participating has led to great personal development as well. Before I was invited to join the panel, I could not have imagined speaking to such a crowd.

Writing my story was rather challenging. Normally I deal with my limitations and everything that comes with it one issue at a time, and that is also how I wrote my story: topic by topic. When I was satisfied with each part and read through the entire thing for the first time, I was shocked by my own experiences. For me this was also the first time I had faced all consequences of BTHS at once. It is a common defense mechanism to downplay issues, but eventually you have to face reality to improve matters for yourself. In contributing to the PFDD meeting it was necessary for me to do that and it made a world of difference.

In the past, I always compared myself to healthy people and set goals based on what portion of their activities I should be able to do too. My body often disagreed and this led to frequent disappointment for me and others when I had to cancel plans. My speech made me realize that my condition is worse than I had been willing to admit. Though this may sound negative and was a harsh truth to me, it caused a change in my approach to life.

I now make much fewer plans in advance, consider my energy every morning and adapt my goals to what is really possible. Instead of disappointment when looking back on my day in the evening, I now sometimes surprise myself by having done more than planned. It is still frustrating to look back on the past weeks and on goals I still haven't accomplished though, so a cure or treatment is still equally welcome.

The support of the people involved in the preparation has been wonderful, the feedback from the community to my delivery was very empowering, and I felt truly inspired by the audience participation. A big thank you is definitely in place for everyone who has helped me through this experience and I'm happy to have helped in this way. Now, I am looking forward to the various ongoing research projects to reach a stage where the FDA gets involved so we can really see the impact of this meeting on others.



Peter

Groundbreaking Meeting Shatters Barth Stereotypes

By Lynn, Mother of Affected Individual, Canada

Every BSF conference is an important event in our lives, and my son, Adam, and I have managed to attend each one since the beginning, many times accompanied by some of the family. We always learn a tremendous amount from the conferences, renew friendships and make new ones, and Adam participates in scientific research. The 2018 conference had some new elements to it, and one of those was the PFDD meeting.

The PFDD meeting marked a very important opportunity to hear from our population of affected individuals and families around the world, understand the direct impacts of Barth syndrome (BTHS), and gather opinions on priorities for the organization, research and possible treatments. In order to gather information from everyone who wanted to participate (including those watching online), polling software was acquired, and questions were posed real-time. People from all countries, all ages and levels of technical comfort were able to answer critical



(L-R) Lynn and Adam

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BSF Makes History With PFDD Meeting in Florida

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questions and reflect their main issues and priorities. Thankfully, two of our BTHS affected individuals who are technology wizards, John and Peter, were available and helped people so everyone was ready to go. During the event, our AV crew assisted, and I monitored the responses coming in, so we were sure everyone was able to respond and see results in real-time.

The meeting itself was quite moving. After some introductory remarks, there were panels of affected individuals and parents who spoke. They covered different ages, countries and a variety of circumstances. One by one, they spoke about their own experiences and some of the stages they or their children have experienced. Each person who spoke shared extremely personal experiences and gave us a deeper level of insight into what people face as they live with and around BTHS. We heard from one of the founders of the organization who took us through the stages in her son's experience through to his tragic passing. We heard from several of the older affected individuals about their fatigue and struggles with daily living, and we heard from parents of younger children on how BTHS affects their family and how challenging seemingly simple things like eating and attending school can be for their sons.

During the talks, the packed room was quiet, as families, researchers and clinicians all listened intently, along with the online audience. Between each panel, polling questions were asked of the audience in the room and online, and we watched as the responses were tracked and displayed for everyone. It was fascinating to watch the responses build so that we gained a comprehensive view of how key elements of the condition are affecting the population. After each segment, there was an opportunity for questions and open discussion, so others had the chance to speak.

There were many revelations during these few hours, and, as a parent, I found it emotional. We talk regularly to a subset of the affected young men and families, but there were still several surprises during this meeting. When the question of what areas are the most important to address, I was sure it would be the heart and the most obviously life-threatening areas would be top of the list. They were important, of course, but it was clear that daily living challenges, such as fatigue and muscle weakness, are critical to address. It was surprising just how much that affects the quality of life and that was reinforced during the most emotional portion of the meeting – the video of “Uncle Bob,” one of the oldest Barth affected individuals.

The “Uncle Bob” video helped us to see how the condition has affected Bob's life and how he has gone downhill over the years to the point where this man in his 50's is in a care facility and unable to function without assistance. Once able to be active and operating farm machinery, drive a vehicle and live a relatively “normal” life, Bob is now dependent on others for basic living and has only fond hopes of driving his truck again. This video showed us one possible future for affected individuals, even those with less severe heart function and neutropenia issues, if we are not able to find them suitable treatments or a cure to BTHS. I sincerely hope and believe that this, along with the open, honest information shared by the people at the meeting and on-line, has galvanized the science and medicine community so we can accelerate critical research and treatments for BTHS.

Industry Perspective of Barth Syndrome Foundation Externally-Led Patient-Focused Drug Development (PFDD) Meeting

Stealth Biotherapeutics, Inc. would like to thank the BSF for including us in the externally-led PFDD meeting. The information that the panelists presented was very impactful and informative. Learning what symptoms matter most to this patient population will continue to be helpful in designing clinical trials and determining the symptoms that matter most to patients. The information presented by not only those affected by Barth syndrome (BTHS) but also family members and caretakers will help to educate researchers, industry and the FDA on the burden of the disease and the outcome measures that are most meaningful in this community. The panelists' experiences of living with BTHS help incorporate the patient voice in clinical development programs and will be helpful when incorporated into the FDA's risk-benefit analysis for potential new treatments for BTHS. *(Photos courtesy of BSF 2018)*



Kate McCurdy shares her son's journey with BTHS



Moderator, James Valentine, polls questions from the audience at the PFDD meeting

SciMed Sessions of 2018 BSF Conference Impress

(Cont'd from page 1)

Pathomechanism(s) of Barth Syndrome

William Pu, MD (Boston Children's Hospital) led off the Scientific and Medical sessions discussing the mouse models of Barth syndrome and how their differences may impact the traditional understanding of the pathomechanism of this mitochondrial disease. Christoph Maack, MD (University of Wurzburg) spoke about how calcium uptake reduction in the mitochondria leads to oxidative stress, decreased NADH+ levels and energy depletion. Douglass Strathdee, PhD (Beatson Institute) discussed new observations with the knockout (KO) mouse model. Christian Reynolds, PhD (Wayne State University) showed how nicotinamide replacement improved mitochondrial function in preclinical and animal models of Barth syndrome. Xujie Liu, PhD (Boston Children's Hospital) revealed how increased reactive oxygen species (ROS) in BTHS mitochondria activates CaMKII (Ca²⁺/calmodulin-dependent protein kinase II) leading to RYR2 phosphorylation and increased calcium leak in BTHS stem cells (iPS) differentiated into cardiomyocytes. Laura Cole, PhD (University of Manitoba) spoke about how altered islet function may promote a lean phenotype in knockdown (KD) mice. Jan Dudek, PhD (University Medical Clinic Würzburg) showed how defective mitochondrial cardiolipin remodeling causes alterations in cellular signaling pathways leading to OXPHOS super-complex disruption and a reduced HIF-1 alpha mediated response to hypoxia conditions.



Dr. Matt Toth leads off session on "Potential Therapies for Barth syndrome"

Potential Therapies for Barth Syndrome

Christina Pacak, PhD (University of Florida) provided a convincing case for gene therapy in Barth syndrome with adeno-associated virus (AAV) vectors reversing the cardiac and fatigue phenotype of KD mice. Hilary Vernon, MD, PhD (Johns Hopkins University) described the ongoing TAZPOWER phase 2 trial with the Elamipretide compound. Colin Phoon, MPhil, MD (New York University School of Medicine) evaluated antioxidant therapies in the KD mouse model of Barth syndrome but found no significant changes. Michael Chin, MD, PhD (Tufts Medical Center) described novel mitochondrial targeting peptides for tafazzin protein designed for enzyme replacement therapy. Riekelt Houtkooper, PhD (Academic Medical Center) spoke about cross-species-omics integration to identify new potential treatment targets for Barth syndrome. Hazel Szeto, MD, PhD (Weill Cornell Medical College) described how Elamipretide, the first cardiolipin-protective compound, may benefit Barth syndrome individuals.

Clinical Characteristics of Barth Syndrome

The second day started with Carolyn Taylor, MD (Medical University of South Carolina) describing the natural history of cardiomyopathy and cardiac conduction in Barth syndrome with data she has collected over several years. Brittany Hornby, PT, DPT, PCS (Kennedy

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(L-R) Drs. Christoph Maack and Jan Dudek presented during "Pathomechanism(s) of Barth Syndrome" session



(L-R) Drs. Christina Pacak and Michael Chin presented during "Potential Therapies for Barth Syndrome" session

SciMed Sessions of 2018 BSF Conference Impress

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Krieger Institute) described the functional exercise capacity, strength, and balance and motion reaction time in Barth syndrome individuals measured at previous conferences. Todd Cade, PT, PhD (Washington University School of Medicine) updated the metabolic phenotype in Individuals with Barth syndrome and showed little difference with or without cardiac transplantation in skeletal muscle. Anthony Aiudi, PharmD (Stealth Biotherapeutics) summarized data from the Barth Syndrome Registry which has been useful in their clinical trial. Colin Steward, PhD, FRCP, FRCPC (University of Bristol) gave a most inspirational talk about twin sisters, one of whom suffered many of the symptoms associated with Barth syndrome due to extreme pathological skewing of her X-chromosome.

Cardiolipin and Barth Syndrome

Ulrich Brandt, PhD (Radboud University Medical Center) spoke about the composition and dynamics of the mitochondrial complexome. Miriam Greenberg, PhD (Wayne State University) described the mechanisms underlying TCA cycle (also known as the Krebs cycle) defects in *tafazzin* deficient cells. Yuguang (Roger) Shi, PhD (University of Texas Health Sciences Center) spoke about inhibiting the ALCAT1 gene in Barth syndrome as a way to make BTHS mitochondria function better. Nathan Alder, PhD (University of Connecticut) described biophysical approaches toward understanding the molecular mechanism of action Elamipretide. Markus Keller, PhD (Medical University Innsbruck) described the structural molecular diversity of cardiolipins.

Poster Session

Thursday evening's Poster Session showcased 25 posters from which four were selected to be presented at the Friday sessions. George G. Schweitzer, PhD (Washington University) described how increased anaerobic metabolism during exercise in BTHS may result from augmented liver glycogenolysis. Renata Goncalves, PhD (Harvard University) showed that superoxide/H₂O₂ production rates in isolated heart and skeletal muscle mitochondria from the KD mouse model were unexpectedly not changed from wild type levels. Grant Hatch, PhD (University of Manitoba) spoke about how aberrant cardiolipin metabolism is associated with cognitive deficiency and hippocampal alterations in KD mice. Arianna Anzmann, MD (Johns Hopkins School of Medicine) described metabolic, and molecular factors in BTHS.

Conclusion of an Impressive Conference

The Science and Medicine sessions, which were recorded for viewing later (<https://www.barthsyndrome.org/newsevents/conference/>), continue to impress the attendees with the openness of the discussions, the extent of the collaborations, and the implications of the work presented. Thirty percent of the attendees were from outside the US and almost 60% have attended two or more past conferences. The Q & A periods after each talk were particularly invigorating and certainly led to more detailed discussions off-line. The wrap-up session where the session chairs summarized their sections, brought the SciMed talks to an end but left all who attended with

(Cont'd on page 10)



Dr. Carolyn Taylor presented during "Clinical Characteristics for Barth Syndrome" session



(L-R) Dr. Brittany Hornby and Anthony Aiudi presented during "Clinical Characteristics of Barth Syndrome" session



(L-R) Drs. Ulrich Brandt and Nathan Alder presented during "Cardiolipin and Barth Syndrome" session

SciMed Sessions of 2018 BSF Conference Impress

(Cont'd from page 9)



a renewed sense of optimism and new ideas for experiments to perform back in the lab. Friday night brought the Science and Medicine attendees together with Family attendees in a social event filled with food, refreshments, fun, dancing, and games. Everyone is looking forward to 2020 with anticipation and hope for clinical improvements with the current clinical trials and with high expectations for new ones that are being planned. (Photos courtesy of BSF 2018)



(L-R) Poster award winners Drs. George G. Schweitzer and Renata Goncalves had the opportunity of presenting during the SciMed sessions on Thursday and Friday afternoon

Family Sessions at BSF Conference Offer Hope

By Shelley Bowen, Director, Family Services & Awareness, Barth Syndrome Foundation (BSF) and Christiane Hope, Board Member, Barth Syndrome Foundation of Canada (BSFCa)

We have become familiar with stretching ourselves to the limits and, when necessary, adjusting a bit to push the bar even higher. We squander no resource and seize every opportunity in our crusade to propel our mission forward. We have no choice. Lives are at stake. When we learned it might be possible for us to host the externally-led Patient-Focused Drug Development (PFDD) Meeting, it seemed appropriate to do it during the conference since so many of our affected individuals would be present. So, we dug in and moved forward to make it happen. Once again, the people of this organization rose up to the challenge.

Our community of families, researchers, healthcare providers, volunteers and vendors are steadfast in their determination to make each conference “*the best one yet*”. A conference isn’t just an event, it’s a life-changing experience. Every detail is painstakingly scrutinized to make each conference memorable. Michaela Damin (Board Member, Barth Syndrome Trust) and Chris Hope (Board Member, BSFCa) worked tirelessly to make this year’s happening special.



(L-R) Michaela Damin, Christiane Hope and Shelley Bowen

People are often astonished to learn the biennial conference is a seven-day event. Many of the two hundred and twenty-three family members who traveled from twelve nations around the world began cycling through research studies, individual appointments and small focus groups on Sunday, July 15th.

Ten veteran conference goers were carefully matched to serve as mentors with the ten first-time family attendees. Volunteers Donna Strain and Shelia Mann spearheaded the mentor matching. One such pairing was Anna Dunn with the families from Italy. She was decidedly helpful in providing Italian translation for the families during individual appointments and throughout the sessions as well. Kevin Woodward (Board Member, BSF) was an outstanding Master of Ceremonies from beginning to the end of the conference.

Everyone loves someone diagnosed with Barth syndrome (BTSH), including grandparents. Many of the grandparents in our group also know the heartache of losing a child with BTSH. Brandi Dague (volunteer) organized a lovely beachside wine and cheese gathering for the grandparents on Monday evening. It was the perfect icebreaker. And, the Dague family also served as babysitters for the night so that the grandparents who came with their grandchildren wouldn’t miss out on a thing.

(Cont'd on page 11)

Family Sessions at BSF Conference Offer Hope

(Cont'd from page 10)

Small Group Meetings, Clinics & Consultations

We realize families and affected individuals have various levels of understanding about BTHS. It is our goal for them to learn as much as possible about the disorder during the conference week. Therefore, we began the week with primer/refresher sessions filled with Barth syndrome essential information presented by a team of healthcare providers and led by seasoned families.



Emily Love led small group session for parents of pre-school aged affected individuals

In the three days preceding the PFDD meeting, over 500 research and consultation appointments were scheduled and carried out. When families were not in research and consultation appointments, they participated in small group sessions and focus group sessions. Susan McCormack (Chair, BSF), Michael Bowen (volunteer) and Michelle Florez (volunteer) kept this intricate schedule on time throughout the “clinic days.”

Treatment often comes in pill form, and pills are not always easy to swallow for someone who has sensory sensitivities such as many people with BTHS do. Stacey Reynolds, PhD, OTR/L (Virginia Commonwealth University) and Consuelo Kreider, PhD, MHS, OTR/L (University of Florida) led the pill swallowing workshop, teaching techniques to help ease individuals’ daily therapies. Stacey also kicked off a research study to investigate sleep patterns in people with BTHS during the conference.



Dr. Stacey Reynolds led pill swallowing workshop

Rebecca McClellan, MGC, CGC (Johns Hopkins University and Kennedy Krieger Institute) and Iris Gonzalez, PhD (A. I. duPont Hospital for Children) met with families and individuals on a one-on-one basis to discuss genetic concerns. Rebecca was also actively involved with Susan McCormack, Jessica Wright (volunteer) and Brandi Dague in sculpting the carrier sessions. Nicole Derusha-Mackey (Board Member, BSF) led the small group sessions and served as the family representative in sessions where the voices of heart transplant recipients needed to be heard.

Hilary Vernon, MD, PhD (Johns Hopkins University and Kennedy Krieger Institute) had back-to-back consultation appointments to address general questions, while Brittany DeCroes Hornby, PT, DPT, PCS (Kennedy Krieger Institute), Arianna Anzmann, MD (Johns Hopkins School of Medicine) and Ryan Manuel (Kennedy Krieger Institute) moved participants through their scheduled appointments to participate in research. Carolyn Taylor, MD (Medical University of South Carolina) collected the cardiac data that will conclude her longitudinal study which was initiated in 2002. A study to better understand the problems those with Barth syndrome have with eating was initiated by Florence Mannes (President, Association Syndrome de Barth France) with support from speech therapists Catherine Thibault and Sandrine Aliotti-Condamy (Sorbonne University).

(Cont'd on page 12)



Jon Stokes led small group session for high school aged affected individuals



(L-R) Jessica Wright, Rebecca McClellan, Christiane Hope and Susan McCormack during sessions for carriers

Family Sessions at BSF Conference Offer Hope

(Cont'd from page 11)

Translating Science into Medicine

On Tuesday evening, Todd Cade, PT, PhD (Washington University School of Medicine) and Matt Toth, PhD (BSF Science Director) provided a research update tailored to the 16 years and older individuals who have been diagnosed with BTHS. Following that session, BJ Develle (Board Member, BSF), Iyar Mazar, Doctoral Candidate (Boston College), Jon Stokes, MBA, Amer Randell (volunteer), Ashley Espensen, MPH (Adelphi Values) and Emily Love, BA (Adelphi Values) spearheaded age-specific small group discussions which extended throughout the week.

Audrey Anna Bolyard, RN, BS (Severe Chronic Neutropenia International Registry) enrolled numerous families into the Severe Chronic Neutropenia International Registry. Thirty individuals enrolled or updated their data in the Barth Syndrome Registry. Volunteers Sue and Mike Wilkins, Donna Strain and Shelia Mann worked with the Sunstar Medic volunteers to get vitals on every individual participating in a research study.



Drs. Christina Pacak & Todd Cade during the "Potential Therapies for Barth Syndrome" session

During the PFDD meeting, the youth went out on a nature boat ride sponsored by volunteer, Kevin Dollard. The 90-minute Sea Life Safari Boat Tour took the crew throughout the Tampa Bay intracoastal waters. One of the highlights of the trip was dolphin spotting, including a mama dolphin with her baby. The excursion ended with an ice cream social. After the PFDD meeting, everyone needed a little breather. All attendees gathered on the beach for a sunset dinner.

The youth and their volunteers kicked off Thursday with a Build the Boat team building experience. This high-energy, fun way to spend the day was led by Shawn Whiting and donated by Boucher Brothers. Three teams built boats and carried them to the pool to compete in a race. Team Franco-Italia (aka BJ's team) took the gold. Other team leaders were volunteers Alanna Boozer, Hillary Burlew-Miniello and Jessica Gosselin.

Cardiac Aspects of Barth Syndrome

Thursday's educational sessions featured presentations by Drs. Carolyn Taylor, Brian Feingold, MD, MS, FAHA (University of Pittsburgh) and John Lynn Jefferies, MD, MPH, FAAP, FACC, FAHA (Cincinnati Children's Hospital Medical Center) about the cardiac aspects of BTHS. Family members Nicole Derusha-Mackey, Andrew Buddemeyer and Tracy Torbert led a panel about the transplant experience with BTHS.

Clinical Research Updates and Their Impact on Treatment

Kate McCurdy (*Emerita*, BSF Scientific and Medical Advisory Board) provided families with a primer on the hows and whys of clinical trials, while Drs. Cade, DeCroes Hornby, and Yoonjeong Lim, PhD, OTR/L (Georgia State University) discussed details and outcomes

(Cont'd on page 13)



(L-R) Dr. Brian Feingold, Andrew (age 29, BTHS) and Nicole during the "Cardiac Aspects of Barth Syndrome" session



(L-R) Kate McCurdy, Drs. Yoonjeong Lim, Todd Cade, Brittany Hornby, and Michaela Damin participated in "Clinical Research Updates" session

Family Sessions at BSF Conference Offer Hope

(Cont'd from page 12)

of current and concluded clinical studies. Families were then able to hear about potential therapies and treatments. Following this long day of sessions, Barry Byrne, MD, PhD (University of Florida) and Christina Pacak, PhD (University of Florida) graciously responded to families' requests and gave an ad hoc "simplified" talk about gene therapy.

Luminaries on the Beach

Thursday evening, the Hone and Wright families once again generously sponsored the beach luminaries. These lights celebrate and honor all individuals with Barth syndrome. This year's moving event, with a piped march to the center of our logo, was caught on film using an overhead drone helpfully sponsored by the Grzesiak family. *(View drone video)*



Luminaries lit the beach in honor of all individuals with Barth syndrome

Neutropenia and Barth Syndrome

Friday morning sessions started with Colin Steward, PhD, FRCP, FRCPC (University of Bristol), Jean Donadieu, MD, PhD (Trousseau University Hospital), and Audrey Anna Bolyard, RN, BS (Severe Chronic Neutropenia International Registry), who joined from Washington via the internet. The three shared their experiences with neutropenia and BTHS and then answered questions from families. Dr. Steward then started the genetics and carriers' session detailing one family's story. Rebecca McClellan and Cynthia James, ScM, PhD (Johns Hopkins University) helped families understand genetic testing, and, using humorous play acting, were able to provide the audience with tips on talking with children about BTHS.

Living with Barth Syndrome

The final session on Friday dealt with how to live (and thrive) with BTHS. BSF Science Director, Dr. Matthew Toth, Dr. Stacey Reynolds, Anthony Aiudi, PharmD (Stealth BioTherapeutics) and Iyar Mazar all gave wonderful updates, summaries, suggestions and key points to move forward not just for an individual's gain, but also how to provide help to everyone with BTHS. We were also joined by Nicol Clayton, Specialist Paediatric Eating Disorders Dietician, (Bristol Royal Hospital for Children) who gave families important information on nutrition and feeding, always a challenge with individuals with BTHS.



(L-R) Gene Kelley and Anthony Aiudi participated in "Living with Barth Syndrome" session

Friday Night Social

On Friday evening, all conference attendees joined together to celebrate the week-long conference. Led by Lynda Sedefian, volunteers Michelle (and her son, Matthew), Jessica, and Brie planned for an interactive evening of "Family Feud" wherein Steve Harvey hosts,

(Cont'd on page 14)



(L-R) Drs. Jean Donadieu and Colin Steward participated in "Neutropenia and Barth Syndrome" session



Audrey Anna Bolyard participated in "Neutropenia and Barth Syndrome" session



Iyar Mazar participated in "Living with Barth Syndrome" session

Family Sessions at BSF Conference Offer Hope

(Cont'd from page 13)

David and Fraser entertained us all! Teams competed throughout the evening answering questions related to BTHS. And the winners were ... a very large family from Texas who knew every answer and were determined to make their mark. Go #TeamBarth.

Closing Ceremony

We closed the conference with a wonderful video of the lovely family images that were taken throughout the week by professional photographer, Amanda Clark. All were moved by testimonials given about the impact of the conference delivered by family members Matej (age 19, BTHS), Pietro (age 16, BTHS), Kelsey (parent) and Brandi (parent).



Family Feud hosts Fraser (L) and David (R) with Ben (age 21, BTHS) at the Friday Night Social

Families have often said that for one week every two years, Clearwater Beach is the safest place on the planet for a child with Barth syndrome. In large part, that is due to the continued support offered by Pinellas County and SunStar Paramedics. Medics and emergency support are conspicuously present from the beginning until the end.

A big thanks to the following individuals who also volunteered throughout the conference planning and onsite:

- Develle and Buddemeyer families for providing local support and allowing their homes to be transformed into BSF warehouses in the weeks leading up to the conference
- Debbie Develle (a/k/a BJ's mom) for working with Amanda Clark to keep families on schedule for their portrait appointments
- John Wilkins for creating a beautiful slideshow presentation of our families that was shown throughout the conference
- Kendal Lucas who volunteered his time as our DJ during the Friday Night Social
- Rough Riders for their generosity in providing the children with stuffed bears

Barth Italia, represented by the Muller, Benedetucci and Ghirvu families, created a makeshift storefront featuring beautiful logo-embossed accessories and apparel as a fundraiser to offset conference expenses. No one went home without Barth bling (*see page 35*).

Volunteers Lois Galbraith and Sharon Olson were there to greet conference goers and provide direction when needed. They have been the first faces you see at a BSF conference since 2004. This was their last year to volunteer with us at the registration desk, and we will be lost without them. "Lois" was the name presenters were most familiar with making travel and lodging arrangements. They also learned pretty fast that Lois is persistent, when she needed information, she got it. We will miss these invaluable volunteers. (*Photos courtesy of BSF 2018*)



(L-R) Sharon Olson and Lois Galbraith will surely be missed at BSF's conferences!

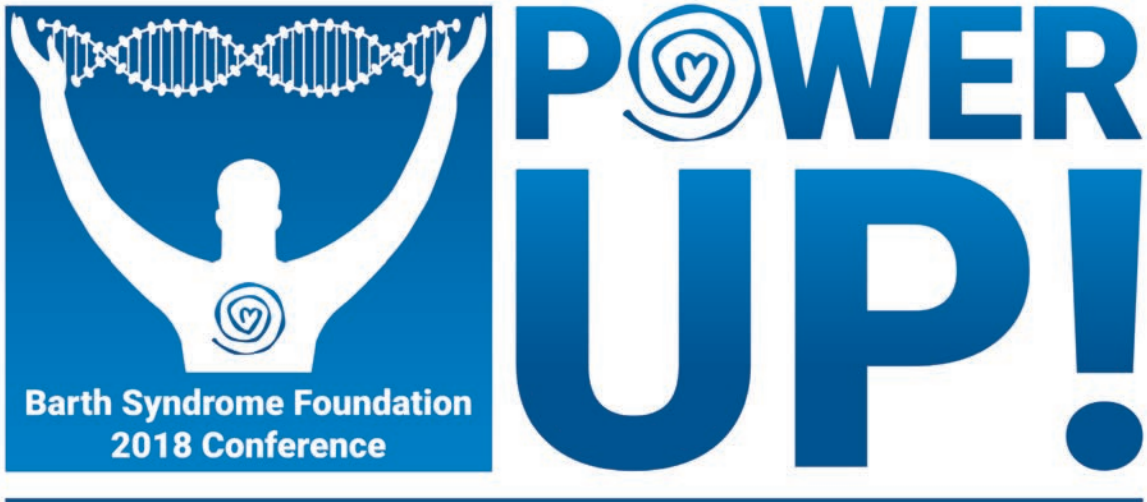


Pinellas County and Sunstar Paramedics



Matej and Pietro provided powerful closing remarks

Memories of BSF 2018 Conference



Barth affected individuals unite at BSF's 2018 conference



Science and Medicine community gather at BSF's 2018 conference

What is a Registry and Why Does BSF Have One?

By Matthew J. Toth, PhD, Principal Investigator, Barth Syndrome Registry

"A central repository for clinical data provides a valuable resource for researchers. ... Only with a critical number of patients is it possible to know what is common and what is not, what is expected and what is not, and what works and what does not." ~ Gerald Cox, MD, PhD, Chief Medical Officer, Editas Medicine, Boston Children's Hospital, Boston, MA

A patient registry, at its most basic level, is a list of names and contact information, sometimes referred to as a contact registry. For BSF this starts as a list of people with Barth syndrome which is kept confidential. It is BSF's most important resource for many reasons. BSF also has a registry which contains much more information than just a list of names — it is called the Barth Syndrome Registry or BRR2.0 for short. BRR2.0 contains a bank of over 100 multiple-choice questions that registrants of BRR2.0 answer (or their caretaker answers for them) about their personal experiences with Barth syndrome that covers areas such as what drugs are taken, what symptoms are most difficult, what was the heart ejection fraction at your last echocardiogram, etc. Answering these multiple-choice questions and regularly updating the answers are very important for researchers who are trying to find new treatments for Barth syndrome.

We started work on developing a registry in 2005, before I even came to BSF. BSF recognized early on that it needs to inform its community as well as researchers about what Barth syndrome really is in a way that everyone can trust and which belongs to the patients. BRR2.0 does this in a systematic way by collecting information directly from individuals and gives them real-time insights into ways their responses anonymously compare to others. BSF from the beginning has been patient-centered, and we will continue to leverage the knowledge gained through BRR2.0. The anonymized data is freely available to anyone who has a real interest in knowing more about this disease and seeks to help BSF fulfill its mission.

How is BRR2.0 used to find treatments? Barth syndrome researchers will often have an idea for a therapy or a hypothesis to test, but they do not know how relevant this idea or hypothesis is. One researcher asked me how many Barth syndrome individuals use GCSF, and I was able to answer him almost immediately by using the BRR2.0. Currently, Drs. Brian Feingold and Carolyn Taylor are working on a study dealing with heart-transplanted Barth syndrome individuals. They are using the data in the BRR2.0 along with a large international pediatric registry to define the major issues with this heart transplant group. The Patient-Focused Drug Development Meeting at the 2018 BSF conference clearly demonstrated the difficulties with fatigue for many of our Barth syndrome individuals. Looking at the BRR2.0, I can tell you that almost 90% of the registrants indicate tiredness as being a significant problem, but only ~ 30% say that pain interferes with their enjoyment of life. Data like this, which the BRR2.0 can provide, gives researchers and drug developers the tools to design effective therapies.

Another important function of BRR2.0 is as a means to advertise clinical studies/trials to its registrants. Because BRR2.0 registrants record where they live and their age, clinical studies/trials can use that information to judge whether they have sufficient numbers to do their clinical study. Clinical studies are the hallmark of clinical progress and they often lead to clinical trials and hopefully to effective treatments. And how will we know if these treatments are effective? We will know because the BRR2.0 will tell us what Barth syndrome individuals experienced before the treatment which will be compared in the clinical trial to what the individual experiences after the treatment. For example, if a treatment lowers the 90% tiredness value mentioned above by half, then that would probably indicate the efficacy and value of that treatment.

Currently, approximately 100 registrants are listed in the BRR2.0. **We need more.** In addition, the BRR2.0 is changing over time to make it more useful to researchers. For example, earlier this year BRR2.0 added questions about fatigue and about heart transplantation for Drs. Feingold and Taylor so they could perform their study. Both of these new sets of questions are adding to our knowledge of what Barth syndrome is, how Barth syndrome individuals deal with their condition, and what they would consider to be an effective therapy. The data contained in the BRR2.0 helps researchers formulate and test hypotheses that can lead to clinical studies/therapies which the BRR2.0 can also help to make happen. BRR2.0 will continue to evolve and will become more valuable as our community participates in the current clinical studies/trials and researchers design new clinical studies/trials until we finally reach our ultimate goal: a world in which Barth syndrome no longer causes suffering or loss of life.

Opportunities to Participate in Research



By participating in a research project, you can play a role in helping physicians and other researchers develop and test better ways to diagnose and treat individuals affected by Barth syndrome. You can make a difference! Please consider becoming a research participant today. Please visit BSF's website to learn more about these opportunities (<https://www.barthsyndrome.org/waystohelp/participateinresearch.html>).

- Characterization of the 'metabolic phenotype' in Barth syndrome with cardiac transplantation
- A study seeking to describe how individuals with Barth syndrome do after being formally listed and/or receiving a heart transplant
- Effects of resistance exercise training on cardiac, metabolic and muscle function and quality of life in Barth syndrome
- Barth Syndrome Registry & Repository



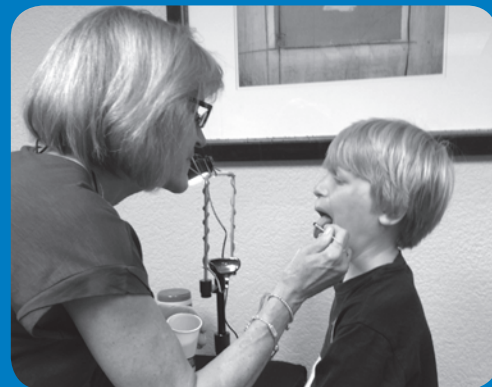
(L) Nick and (R) Andrew participated in Dr. Todd Cade's research study



Dr. Stacey Reynolds performs smell test



(L) Raphael and (R) Henry participated in Dr. Carolyn Taylor's research study



Dr. Shelley Lane performs taste receptor test

BSF Recognized Nationally as a Great Nonprofit for the Fifth Year in a Row!

“

BSF has been part of our life since our grandson was diagnosed about nine years ago and today we can't imagine how we and our grandson's parents would have coped with the syndrome without the support of this wonderful Foundation.

”

“

I have watched a dear friend lose two sons from this disease. Since then she has worked tirelessly to research it hoping to find a cure. This Foundation has great people that are so involved in trying to find a cure.

”

“

So many lives saved by this great organization! What we know about this syndrome has been learned because of this Foundation.

”

“

Awesome group of people who are dedicated to new treatments and support for each and every patient and family. Small but mighty group! If I had to live this life without them it would be devastating.

”

“

Wonderful organization, fabulous source of information, great support for my son and our family. Always striving for the good of everyone through research, family services and support for our rare condition.

”

“

The Foundation is a true example of how groups for rare diseases should be run. The way they bring patients, families, doctors and scientists together is unique and the amount of knowledge gathered in its mere 18 years of existence is mind blowing.

”

“

Opened my eyes to a syndrome I knew nothing about! This organization is very passionate and well organized! Thanks for all you do!

”



“

The Foundation has literally helped our family in so many ways, there is not enough thank yous in this world to thank them enough for what they've done for my son and our family. They are always there for us in any way we need them.

”

Power of Kindness

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Editors Note: We all know intuitively that thinking takes energy; so it stands to reason that for people who have difficulty producing energy (as is true for those with Barth syndrome [BTSH]), thinking and remembering things might become especially taxing. Several individuals alluded to this in the PFDD meeting this summer.

But except for Michele Mazzocco's work beginning in 2001 which described some mild learning disabilities in BTSH (especially in math), we have not spent much time looking at BTSH brains because our guys tend to be bright, verbal and wise beyond their years. Their physical issues (including cardiac and neutropenia) have seemed to be their most prominent problems.

At the 2018 conference, however, Dr. Grant Hatch presented a poster showing that changes in cardiolipin metabolism in Barth knockdown (KD) mice can lead to some cognitive deficiency and alterations in the mouse's hippocampus (a region of the brain which is involved with memory). This is a very interesting new area of inquiry for BTSH.

How is Memory in Mice Altered by Barth Syndrome

By Laura Cole, PhD, Postdoctoral Fellow, Department of Pharmacology and Therapeutics, Faculty of Medicine, University of Manitoba, Winnipeg, Manitoba, Canada



Laura Cole
(Photo courtesy of Laura Cole 2018)

That is the question that Canadian scientists from the University of Manitoba set out to investigate using a mouse model of Barth syndrome (BTSH). We were interested in investigating this question since there has been concern about some poor academic performance associated with Barth syndrome. For example, it was previously determined by Michele M.M. Mazzocco and colleagues at the Kennedy Krieger Institute in Baltimore, MD that BTSH was associated with math difficulties in school age children. One of the possible reasons for this discrepancy could be a weakness in memory. Therefore, we were interested in determining whether memory was altered in the mouse model of BTSH and what changes in the brain may be contributing. This may point to useful future detailed investigations to further our understanding of the far more complex and subtle human brain.

Our study consisted of analyzing the memory of mice lacking *tafazzin* protein (BTSH mouse) and comparing them to mice with normal amounts of *tafazzin* (control mice). We measured the ability of mice to remember using the novel object recognition memory test.

In this test, two identical objects were placed in a small caged area and each mouse was allowed to explore the objects freely during a 10 min training session. On the following day, mice were placed back in the small caged area and presented with one of the objects used during the training session (now considered a familiar object) and a novel, unfamiliar object of different shape and texture. The time spent exploring each object was recorded during the 10-minute session with a video tracking system.

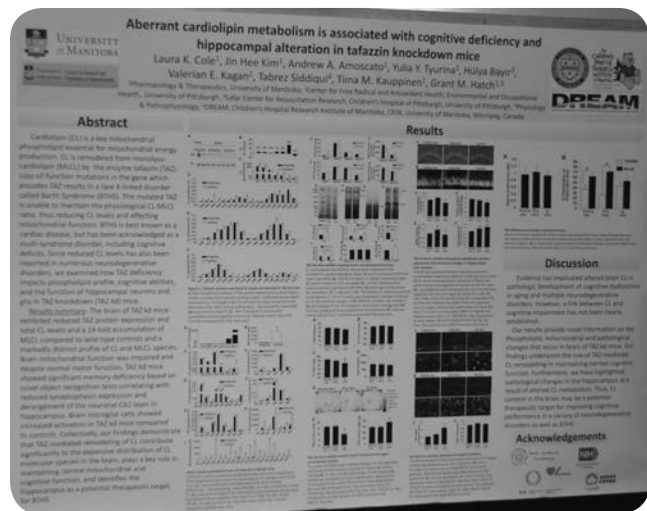
As expected, we determined that the control mice spent a greater proportion of time (60-70%) exploring the new (novel) object and a shorter amount of time (30-40%) exploring the familiar object. This was significantly different from the BTSH mice. Instead, BTSH mice spent equal lengths of time exploring the two objects (~50%) indicating an inability to remember being previously introduced to the familiar object the day before.

We also analyzed the brains of BTSH mice and compared them to the control mice. We identified structural changes in the part of the brain responsible for memory called the hippocampus. In the control mice, the cells (neurons) in the hippocampus appeared normal, as they were arranged close together in an organized well-defined layer. Similar to visualizing a bunch of helium balloons with similar length strings. In comparison, the neurons in the hippocampus of the BTSH mice appeared further apart and disorganized. This can be visualized by imagining that the helium balloons with different length strings so some are floating up further away than others. These results indicated that the cells in the brain may not be communicating with each other efficiently, and thus it may be more difficult to generate memories.

(Cont'd on page 20)

How is Memory in Mice Altered by Barth Syndrome

(Cont'd from page 19)



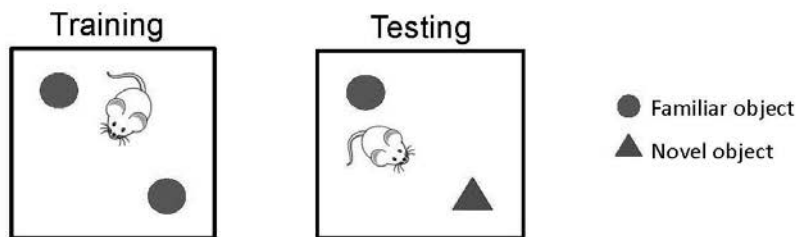
Dr. Laura Cole presents poster at BSF's 2018 conference: "Aberrant cardiolipin metabolism is associated with cognitive deficiency and hippocampal alteration in tafazzin knockdown mice" (Photo courtesy of John Wilkins 2018)

Finally, we looked at how well the cells of the brain were able to generate energy. Since, brain cells need energy to communicate, this would give us a better idea of how well the neurons might be "talking" to each other. We measured how fast oxygen was being used by the brain cells because energy production (motor running) is closely linked to the rate of oxygen use (gasoline). Our results indicated that under normal conditions, the brain cells from the BTHS mice were working harder (using more oxygen *i.e.*, gasoline) to generate energy or keep their "motor" going. This was a problem because the maximum oxygen consumption rate (amount of gasoline) for the BTHS and controls remained the same. Therefore, the cells from the BTHS mice were going to "run out of gasoline quicker" when experiencing greater energy demands/stresses compared to the controls. This could help explain why memory is impaired in the BTHS mice because normal brain functions including memory require large amounts of energy use.

We are delighted to have our data published in BBA Molecular Basis of Disease (Biochim Biophys Acta Mol Basis Dis. 2018 Oct;1864(10):3353-3367). We are continuing to study the precise role of various cell types in the brain and the differences in energy production between BTHS and control mice. We anticipate that our current and future results will foster additional studies, understanding and directed educational support to promote a better future for all patients with BTHS.

Finally, I would like to express our gratitude to all our collaborators, as well as the funding provided by the Barth Syndrome Foundation of Canada to conduct this work.

Novel object recognition test



During training (10-15min) mouse is supposed to explore objects, so that it will become familiar. A day after training mouse are placed to open field for 10min with two objects. One of the objects is the same what was introduced during the training day (familiar object) and one is new distinctively different object (novel object). If mouse recognizes the familiar object from training day, it will be interested to explore the novel object more than the familiar object.

Growing Friends and Funds

Growing Friends and Funds had its first gathering at the Conference this past summer. Offered as one of the optional family sessions that week, attendees came together to hear about some of the ways our members are raising awareness of Barth syndrome and raising funds as well. Not only was it a great time for learning tips and tricks and sharing ideas, it was an invaluable chance to hear the reasons why it is so important that we rally together as an organization to help grow our supporters and donors in our effort to eliminate the suffering from Barth syndrome. Best practices and success stories were shared of all shapes and sizes, new ideas emerged, and hopefully all walked away inspired and challenged to do something to help.

As members of the community and BSF Board, Nicole Derusha-Mackey and Megan Branagh have formed a mentoring program to help anyone interested in doing their own grass-roots awareness and fundraiser. Please reach out for a Fundraising Toolkit and any support needed to assist in your efforts!

Connor's Crew

Kevin and Stacey from Phoenix, MD, USA, held a fundraiser in support of the Barth Syndrome Foundation conference. They asked friends and family via social media to join Connor's Crew wherein they could support the cause through the purchase of Connor's Crew t-shirts and sweatshirts and/or by making a direct donation to the BSF. Everyone who joined received a custom Connor baseball card as a thank you. (Baseball is Connor's favorite sport.) The campaign was well received and raised more than \$5,000. The family continues to offer Connor updates through a website they set up for the fundraiser. They hope to make Connor's Crew an annual event.



Connor (age 8, BTHS) with his brother, Ryan
(Photo courtesy of Stacey 2018)

2018 International Scientific, Medical & Family Conference



BSF's international conference is one of the most important activities for the Barth syndrome community. As in prior years, BSF provided meals, educational sessions, and events. You are probably aware that putting on such an incredible event is costly to us. In order to help defray the costs of food and beverage, we sought special gifts. Thanks to you, BSF raised \$60,565 in support of this important event.

Externally-Led Patient-Focused Drug Development (PFDD) Meeting for Barth Syndrome

BSF held the PFDD meeting on Barth syndrome between our community and the U.S. Food and Drug Administration (FDA). The PFDD meeting for Barth syndrome helped to advance BSF's mission: *Saving lives through education, advances in treatment, and finding a cure for Barth syndrome.* Thanks to you, BSF raised \$96,550 in support of this important event.

	 <p>Friends & Family of John Wilkins</p>		
			

(Cont'd from page 22)

Growing Friends and Funds

(Cont'd from page 21)

Levi's Genes

Joe and Amy from Hartford, WI, are doing a fundraiser to support and spread awareness of the Barth Syndrome Foundation. They have shared the news about their son, Levi, having Barth syndrome and wanted to spread awareness and raise money for the foundation through the purchase of Levi's Genes T-shirts and sweatshirts and/or making a direct donation to BSF. Everyone who purchased or donated received a custom Levi's Genes thank you. The campaign is a big deal to the family as this was how they shared Levi's story with friends and family. The overall event went very well, showing lots of love, support and generosity from everyone. They raised over \$3,400. The family will continue to host Levi's Genes campaign or a Levi's Genes event to raise money every year for BSF.

One Family's Approach to Celebration

Our family's fundraising efforts have been influenced by whatever is happening in our lives. The past year or so has brought us heartache at the loss of two mothers/grandmothers, great joy with the wedding of Kevin's uncle/Godfather, Frank, and his beloved Stacey, and celebrations galore with Ted and Kevin's 60/30 birthday and our 35th wedding anniversary. Whew! All of these events have a dual purpose, giving hope to BSF through donations made in memory of, in lieu of, and in celebration of.

This is such a personal and easy way to ask those who know and love you to share in your quest for a treatment and ultimately a cure for Barth syndrome. The notice in the obituary is notice enough. In the wedding and party invitation, we included the BSF website for online donations as well as postage paid envelopes addressed to BSF. We also had (and will have) Barth brochures and more envelopes at the events, making it very easy to donate whatever way is most convenient. We also worked with Lynda Sedefian and Emily Milligan to personalize the thank you letter for each situation. All good!

Team Will Participates in Westchester Triathlon

Team Will members Heather Segal, Gary Rodbell, Jaime Jofre, and Francois Odouard participated in the Westchester Triathlon on September 23, 2018, in memory of Will McCurdy. Team Will member helpers Paul Epstein, Stefan Tunguz, and Mark Segal were also in force! Collectively, they raised over \$12,000 for BSF in memory of Will McCurdy. Team Will continues to push hard, and the momentum is strong. And Will McCurdy is in our hearts every step of the way. Once again, thank you so much for your generous and continued support of Team Will and BSF. Without you, none of this would be possible.



Levi
(Photo courtesy of Amy²⁰¹⁸)



Kevin (age 29, BTHS)
(Photo courtesy of Amanda Clark²⁰¹⁸)



(L-R) Members of Team Will
(Photo courtesy of Heather²⁰¹⁸)



Heather Segal
(Photo courtesy of Heather²⁰¹⁸)

#Giving Tuesday — A Global Day of Giving

By Nicole Derusha-Mackey, Board Member, Barth Syndrome Foundation

"I am a scientist who has worked on Barth syndrome research since 2000 when BSF was started, and it is amazing what they have accomplished since then. The biennial conference brings together families, scientists and clinicians creating an opportunity for studies of the syndrome, opportunities for the families to meet and exchange experiences, opportunities for scientific collaborations. In between, one needs only to look at the BSF website to see all the services they offer to families and clinicians. All this effort is culminating in some currently ongoing clinical trials of therapies." ~ Anonymous

#GivingTuesday is a global day of giving fueled by the power of social media and collaboration.

Celebrated on the Tuesday following Thanksgiving (in the U.S.) and the widely recognized shopping events Black Friday and Cyber Monday, #GivingTuesday kicks off the charitable season, when many focus on their holiday and end-of-year giving. On Tuesday, November 27, 2018, people from around the world will come together for one common purpose: to celebrate generosity and to give.

Thanks to you, BSF is not only funding research for a cure but is also tirelessly informing and advocating for individuals and families who suffer from Barth syndrome.

Every year, BSF embraces new families from around the globe, providing support, educational programs, and awareness, while serving as the collective patient voice. We are relentless in our efforts to support every affected individual from diagnosis throughout their lifetime, and to honor the memory of those we have loved and lost by strategically working toward life-saving therapies.

Your support on Giving Tuesday and throughout the year makes this possible!
(<https://www.barthsyndrome.org/waystohelp/donate/events/givingtuesday.html>)

We hope you'll join this global movement to raise money and awareness for Barth syndrome. We couldn't do this without you!
#POWERUPBSF

SAVE THE DATE
11.27.2018



#GIVINGTUESDAY
#POWERUPBSF

You Can Make A Difference

Donate by check: Make check payable to **Barth Syndrome Foundation, PO Box 419264, Boston, MA 02241**

Donate online: You can donate to BSF by going to our website, www.barthsyndrome.org, and clicking on the "DONATE" link on our home page.

Employer Matching Gift Programs: Many donors are now taking advantage of a "Matching Gift Program" offered by their employer. The employer matches the funds donated by the employee to a charity and provides a convenient method for the employee to donate to a charity of his/her choice.

Securities: Securities can be gifted to the Barth Syndrome Foundation (BSF) in two ways: either via electronic transfer (in which your broker transfers shares using the BSF DTC number) or by physically mailing the paper certificates to BSF. Either method is acceptable, but it is necessary for you to contact your broker to initiate the transfer.

Planned Giving: One of the best ways to support our continued efforts is to remember BSF (or its affiliates) in your estate planning. Talk to your lawyer or estate planning professional about including BSF (or its affiliates) in your will.



Awareness of Barth Syndrome Continues to Grow

Many Barth syndrome (BTHS) related peer-reviewed journal articles are now being published. To date, a total of **161** articles have been published on BTHS research conducted with the support of BSF and/or BSF affiliate funding (denoted below with *) and/or acknowledge biological samples and/or information from Barth families, the Barth Syndrome Registry and Repository, and/or BSF affiliates (denoted below with Δ). Listed below are articles relevant to BTHS that have been added to BSF's library since the last issue of the Barth Syndrome Journal. To view the complete bibliography on BTHS, please visit www.barthsyndrome.org.

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You are receiving this newsletter because of your interest in Barth syndrome. We would like to keep updating you with our latest news. If you wish to unsubscribe or change the format in which you read it — you may prefer to receive it by email for example — then please contact us and we will do so right away.

Barth Syndrome Trust

Fighting To Make a Difference

By Michaela Damin, Board Member, Barth Syndrome Trust (BST)

Without you, we would not exist. We are a tiny community fighting to make a big difference, and we could not do it without you. Here is a round-up of some of your amazing efforts this year..

Manchester Marathon in Memory of Rosa Fields – an Incredible £1472 Raised

By Louise & Stefan



Paul Osler
with well-deserved medal

Paul Osler ran the Manchester Marathon on 8th April 2018 in loving memory of his cousin Stefan and his wife Louise's little girl, Rosa. This beautiful and brave little girl tragically passed away on 12th August 2017 at the age of three due to Barth syndrome.

Before the race Paul wrote: "Running this marathon presents massive challenges for me personally, especially after developing HHT (a genetic disorder which affects my blood vessels), but it's a battle I am determined to win for Rosa and her family and any other boys or girls suffering with this condition."

For all our caring, amazing and generous family and friends who tracked Paul Osler in his gruelling training regime and donated to his chosen charity Barth Syndrome Trust, we wanted to let you know he smashed it!! So very proud of

you, Paul. We reckon Angel Rosa was surely with you in spirit. We feel very heartened and touched by all your efforts and determination. A massively emotional day as our beautiful baby is missed incredibly, every moment. Thank you from the bottom of our hearts.



Rosa
26.06.14—12.08.17

Hampshire Walk in Memory of Sebastian Raises £1030

By Jacquie Webber



Dan Hammond, Emma Hanson,
Hilary Palmer & Jacquie Webber

Whilst our company supports a variety of charities, we on the test team for the past two years set ourselves an additional challenge and have chosen to do a sponsored walk to support a charity that has a personal meaning to us. This year it was Barth Syndrome Trust.

In 2012, a new member of staff joined KFA Connect and over time we got to learn that his little boy, Sebastian, was suffering from a rare genetic condition that we now know was Barth syndrome. Over the following year we followed Sebastian's progress and hoped with all our hearts that he would start to improve or be eligible for a new heart or that a miracle would happen. But it wasn't meant to be, and sadly little Sebastian lost his brave fight for life at the tender age of two and a half.



Sebastian
24.06.11—11.12.13

This loss had a profound effect on all of us at KFA Connect.

All of our hearts broke on that day and, for those of us who are parents or have nephews and nieces, we went home and gave them all an extra big hug.

We know more about Barth syndrome because of Sebastian and we know that this small charity needs our support more than ever. Most people will only ever hear about it when they, or someone they know, has been directly affected.

(Cont'd on page 27)

Barth Syndrome Trust

Fighting To Make a Difference

(Cont'd from page 26)

And so, this year we decided to walk part of the Solent Walk in memory of Sebastian. On Sunday, 9th September we set off at 9.30 from Milford-on-Sea to Lymington and back again. A total of eighteen miles! Our poor feet. A lot of plasters were used that day. For the next week we suffered with blisters, but it was worth it, as our very generous work colleagues, friends and families have helped us to raise £1030 (including Gift Aid) for this great cause. It was a great day and we are happy to support Barth Syndrome Trust.

Two Virtual Runs

The first, Powered by Fairy Dust Fun Run raised £600

217 people took part in this event. They had to run or walk any distance between 1st and 31st July 2018 and, on proving their completion, they were sent a Fairy themed medal! A minimum of 20% donation (from the full price entry) was donated to the Barth Syndrome Trust.

The fairy dust obviously worked its magic! Thank you, to founder Susan Wheatcroft, all the Virtual Runners and to Kate Riseborough Evans for nominating us. (<https://www.virtualrunneruk.com/product/powered-fairy-dust-fun-run/>)



Fairy Dust Virtual Run medal

The second, Alasdair Gray's Barth Syndrome Virtual Run — £1200

By Laura Sutherland

I thought I would try and combine my passion for running and getting a nice medal with Alasdair's 5th birthday and raising some funds for the Barth Syndrome Trust.

I had some medals made up and asked friends and family to set themselves a challenge for October. Run, walk, cycle, swim, whatever they wanted; it was a personal challenge. Getting out and remembering Alasdair was the important thing. I have been overwhelmed by the response. The medals were all quickly sold, some going as far as Canada. I have loved seeing everyone's challenge for Alasdair going on throughout the month.

Alasdair's Virtual Challenge has made £700. As always, we are grateful to everyone who has made this possible and mostly for helping us remember Alasdair. With £500 from Matched Giving, a scheme run through my work to encourage employees to fundraise for charities, the total raised for Alasdair's Virtual Challenge is £1200.



Laura Sutherland

Terri and Friends

Terri Allison is one of our first volunteers and has been a regular fundraiser for many years. Her now traditional Loddon Vale Quiz raised £405. Thank you to quiz master, George White. The Barth Syndrome Trust Tennis Tournament at the Oakley Tennis Club raised £351. Colin Phillimore and friends donated £150 — half the total proceeds of a quiz. Thank you!

During the summer, Terri Allison and Michaela Damin hosted a lunch for some of the long-standing supporters of BST. It was a wonderful chance for everyone to meet up and enjoy a meal together and a chance for BST to say thank you to these dedicated fundraisers whose support remains essential to our work.



Lunch for volunteers & supporters

(Cont'd on page 28)

Barth Syndrome Trust

Fighting To Make a Difference

(Cont'd from page 27)

Good Hair Dye

When sibling, Nick, wanted to raise some money for Barth Syndrome Trust, he did a sponsored hair dye. Everyone who donated got a chance to vote for a colour and at the end he had to dye his hair the colour that got the most votes. He had never dyed his hair any wacky colours before (unlike his older brother, Alex), so he went from very regular brown to a lovely hot pink! And raised a whopping £517 in the process!

William's Birthday

By Sharon

Our annual fundraiser for William's 7th birthday once again proved to be a huge success with friends and family emptying their pockets for our worthy Barth Syndrome Trust. Thank you to everyone who donated raffle prizes, helped organise the day and shelled out for raffle tickets. A grand total of £300 was raised. We had to say goodbye to our giant Elf who William became quite attached to, but it was won by our lovely friend's boys, Jacob and Lucas, who will give him a good home! Already planning ahead for next year's bash so watch this space ... bigger and better to come! Without the support of all our family and friends this would not be possible so a huge thank you from all of us.

Thank you to Sharon's great aunt, Jean Marshall, for another donation of £1000!

Also see Steve's JustGiving page <https://www.justgiving.com/fundraising/runmore-cotch-runmore>. 81 marathons in the bag and 19 to go to reach his goal of 100 before his 51st birthday.

Other Donations and Sale of Goods

Total donations until end September £4458 which includes one off donations, monthly direct donations, charitable payroll giving, sale of Barth merchandise. Gift Aid income this year was £1161 so please remember to ask your supporters to add that extra 25% by simply ticking Gift Aid and supplying their name and home address. We do all the rest! Thank you all.

- Donations in memory of Mr. Edward John Spencer Baxter
- £300 from Chris and Robert Hope (Canada)
- Jill Bath £100 in memory of Sebastian Vavasour
- Legal and General – we were nominated by Wendy Riseborough-Evans — £150
- Roy and Patricia Craigie — £250
- £190 from sale of running shirts at conference



Nick's pink hair day



(L-R) Jacob, Elf & Lucas

Please sign up to Amazon Smile (smile.amazon.co.uk/). Amazon will donate 0.5% of the net purchase price (excluding VAT, returns and shipping fees) of eligible purchases to Barth Syndrome Trust.



NHS National Barth Syndrome Service

For enquiries please call the Barth Syndrome Administrator on +44 117 342 8102 or the Clinical Nurse Specialist on +44 7795 507 294, who can direct your call to the appropriate members of the team.

To contact a member of the team directly, please use the contact details below.

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Barth Syndrome Foundation of Canada

President's Report

By Susan Hone, President, Barth Syndrome Foundation of Canada

It is that time again when we take a moment to sit back and reflect on events and proceedings throughout the past year -- what worked, what could be improved upon, and where to go from here. The biggest event this year, of course, was the 2018 Scientific, Medical and Family Conference, for individuals, families, and the Foundation. As you will read in other articles in this journal, it was truly an inspiring and an eye-opening week for families and doctors. While members of Barth Syndrome Foundation of Canada (BSFCa) support the conference individually as volunteers, we also contribute as a proud affiliate. It is one of the most important programs that we invest in financially.

On the home front, we have started to go through some changes in our board. In June, at the annual general meeting, one of our founding members, Christiane Hope, elected to step down from the Board of Directors. She remains heavily involved in the Foundation and is completely committed and dedicated to our mission and goal.

While board turnover and changes are daunting, the BSFCa is a strong organization, and we know that these changes can be rewarding. In order to not just survive but to flourish, it is important for this affiliate to anticipate change and look to adapt and tackle all challenges head on. We hope to recruit a talented new board member (or two) and with their ideas and dedication we feel that we will be able to move forward and increase our efforts in all areas. *(Photo courtesy of BSFCa 2018)*



Canadians and some wanna-be Canadians

Caleb's First Conference

By Jasmine, Mother of Affected Individual, Vancouver Island, BC, Canada

"From the first moment we walked down the hotel hallway, we were literally welcomed with open arms. Everyone knew exactly what we were going through and was willing to share and relate with their own stories and experiences. The isolation that we've felt in regards to Caleb's diagnosis was somehow lifted, and we knew we were with our extended family." ~ Jasmine



Caleb

In his first six months of life, Caleb suffered cardiac arrest, had been put on Extracorporeal membrane oxygenation (ECMO) as his heart stopped beating for five days, he was on life support for two weeks, he was put in isolation due to a common cold virus, he received numerous doses of medications that you'd never imagine giving a child, he was poked and prodded on a daily basis for several months straight, he was intubated for 93 days because he couldn't breathe on his own, his chest and head got so swollen that the fear of a brain aneurism was very real. He wasn't expected to live.

Part way through those first six months, I chatted with an amazing lady who worked within the ICU. I told her how I had found the Barth Syndrome Foundation (BSF) and explained to her how we'd connected with

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Caleb's First Conference

(Cont'd from page 30)

some families and found out about a medical conference that is held every two years. The conference is an opportunity for doctors, specialists and families to come together to inform, bring awareness, and fight for a cure. This conversation was at a time when Caleb had started to make some progress but still had a long way to go. She said to me that she had a feeling we would be sitting at that conference in 2018. I'll be honest, at the time, I wasn't so sure. I didn't quite believe it.

Fast forward a year, and we had just returned home from BSF's 2018 International Scientific, Medical and Family Conference on Barth syndrome (BTHS)! Ten days prior, we prepared to fly from our home on Vancouver Island to Clearwater Beach, Florida. I was a nervous wreck. Completely anxious and nervous to meet the families that I'd been chatting with on Facebook.

We joined approximately 300 people (including 50 affected BTHS individuals) from around the world to connect, to share our stories, to participate in trials and studies, to fight for a cure, and to build HOPE. Over the six days of the conference, we had the chance to listen to so many specialists and researchers and learn more about this rare disease. We learned about drug trials and gene therapy, we had lunch with brilliant doctors, we drank wine on the beach with top doctors from other countries, and we danced to "We are family," with researchers from around the world. We heard from other families struggling with this disease and learned that there are others living with the same burdens of BTHS as we are.

In regards to the medical side, the conference was invaluable for us. We made some amazing connections, learned more about Caleb's syndrome, and were able to ask questions and get answers from people who focus specifically on this disease. Even more profound though, were the connections with our Barth family. From the first moment we walked down the hotel hallway, we were literally welcomed with open arms. Everyone knew exactly what we were going through and was willing to share and relate with their own stories and experiences. The isolation that we've felt in regards to Caleb's diagnosis was somehow lifted, and we knew we were with our extended family. From birthday parties on the beach, to late night swims in the pool, group selfies, endless tears and emotions, luncheons and photo booths; being a part of this group was an amazing feeling.

I'd like to thank Help Fill a Dream for sending us to this conference. Without them and their donors, we may not have had the chance to meet our new family. I'd like to thank all the people that worked so hard putting this conference together. I'd like to thank all the doctors and specialists who travelled to the conference not only to give presentations, but to mingle with the families and meet one on one to go over medical data. I'd like to thank those doctors and researchers who are working tirelessly to find a treatment or a cure for this dreadful syndrome. I'd like to thank all the families who shared their stories both joyful and painful. I'd like to thank those families for sharing their experiences of the everyday effects of having a child with BTHS. I'd like to thank the other moms for the hugs as we discussed the guilt that we carry knowing we're the carriers of this disease. I'd like to thank the older BTHS individuals for taking both of my boys in as your own little brothers and also for sharing their struggles with us despite how painful they were. These boys and men are the reason we left this conference with so much hope. They continue to fight for not only themselves but the young boys like Caleb and any future boys that find themselves living with BTHS.



Caleb (age 2, BTHS)



(L-R) Mark, Jasmine, Caleb and Jordan during research study at conference



(L-R) Jordan, Mark, Jasmine and Caleb enjoying the beach

To our Barth family, we are so grateful to have shared such an amazing week with you all. We were greeted with so much love, and we look forward to seeing everyone again in 2020! *(Photos courtesy of Jasmine 2018)*



Caleb

Association Syndrome de Barth France

My "Meeting" with Barth

By Sandrine Aliotti-Condamy, Speech Therapist, Pierre et Marie Curie University, Paris, France

Barth syndrome (BTBS). A disorder I had never heard of before starting speech therapy studies just three years ago. It was then that I met Florence and that I learned through spending time with her that her last born was diagnosed with the syndrome. Yes, indeed this child was small for his age, but nothing much was noticeable to me except that. I just had to be careful not to talk to him as if he were a younger child, because he understood everything as a kid his age!

In fact, I did not know much more about Barth syndrome. Then I learned. Symptoms, worries, specific needs at school, fatigue, sensitivity to infections, regular follow-ups at the hospital, and later, enteral nutrition, with gastrostomy. And I also learned of the tremendous commitment of this child's parents through the association. It was obvious to Florence, I think, that her master's thesis would be about Barth syndrome, and more specifically about "orality" disorders (especially feeding disorders) that some BTBS children suffer from. I was proud and happy when she asked me to accompany her on this project and to become her teammate.



(L-R) Catherine Thibault, Florence Mannes,
and Sandrine Aliotti-Condamy

A project

This is how our thesis topic was born: Etiology of Oral Disorders in Patients with Barth Syndrome. To put it more simply, the purpose is to identify, if possible, correlations or links between certain factors and eating disorders (dysfunctions or peculiarities of the oro-myofacial sphere -- meaning muscles of the mouth and the face), eating behaviors, medical history ...). With a goal, qualified as "secondary" in the terminology of the scientific approach but essential for patients and their families: being able to make recommendations for the management of these disorders.

What is orality? Briefly it is the set of functions of which mouth and face are the center: eating orality for chewing, perception of stimuli (analysis of tastes, textures ...), swallowing ... and verbal orality as regards the child's capacity to produce the words of one's own language. All of this has very close links with ventilation, which is the way air flows through our body.

We planned to meet the boys and men with Barth syndrome who would be attending the biennial conference of the Barth Syndrome Foundation in July 2018 and to conduct interviews and clinical tests. It was January 2018 and we had (only) seven months ahead of us to write the Thematic Research File for our faculty, to obtain the required authorizations, to finalize our protocol, to train to administer it and to prepare our departure.

Quite obviously, we asked Ms. Catherine Thibault, speech therapist and psychologist, lecturer at Sorbonne Universities and specialist in oral disorders, if she would agree to direct our master thesis. She did us the honor of accepting not only to direct the thesis but also to come with us to Florida, and we knew that her help would be very valuable.

But before leaving...

We first had to obtain the required authorizations. We had to respect the regulations concerning Research Involving the Human Person, governed by the French law # 2012-300 of March 5, 2012 (known as Jardé law), the regulation on the retention of data and data processing. We also needed to get the agreement of an IRB (Institutional Review Board), before BSF's Scientific and Medical Advisory Board could allow us to conduct this research at the conference. Our efforts led us to meet Mrs. Dosquet, President of the Inserm¹ Ethical Review Committee (CEEI). The mission of the CEEI is to render opinions on research projects directly or indirectly involving humans in the biological and medical field (health research), but also in the humanities and social sciences; registered with the United States Department of Health's Office for Human Research Protections (OHRP), the CEEI has the status of an IRB. After reviewing our file, it gave a favorable opinion on our research project in April 2018. We had permission granted by the French regulatory authorities; things were happening!

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Association Syndrome de Barth France

My "Meeting" with Barth

(Cont'd from page 32)

From then on, our objectives were the following:

- Prepare a questionnaire for the families who would be present at the conference, in order to collect general information and medical history, and information more directly related to orality, in particular eating behavior;
- Precisely define the evaluation "tests" following these axes: fine motor skills, swallowing, phonation, breath-ventilation, "gnosia" (explained below), tactile and olfactory sensoriality, intra-oral examination, oral-facial praxis;
- Plan the logistics regarding our departure (video recording equipment, test equipment ...).

Meanwhile, on the other side of the Atlantic Ocean, Shelley Bowen, Barth Syndrome Foundation Director of Family Services & Awareness, was helping us in an invaluable way informing families about our study and scheduling appointments with those who agreed to participate.

Saturday, July 14, 2018, French National Day and Eve of the World Cup Soccer Final: Departure for Clearwater Beach, Florida

Florence, Catherine and I boarded our flight to Florida on this national day, ready to meet the families the very following day (and incidentally to watch the performance of our national team in the FIFA World Cup Final).

From Sunday to Friday, we conducted 30-minute interviews with 42 patients, whose ages ranged from 8 months to 36 years, and their families. During these interviews, we sought to observe the following elements, guided by Catherine's acute clinical sense:

- **Breathing:** Does the patient breathe through mouth or nose? It is common knowledge that oral breathing can cause a number of disorders.
- **Intra-oral examination:** Is the soft palate mobile? This plays a fundamental role in the articulation of sounds, breathing and swallowing. In dental terms, what kind of bite does the patient have (the relative positions of mandible, jaw and teeth ...)?
- **Praxis:** What is the mobility and tonicity of the face and mouth muscles (especially of the tongue)? We can easily understand their role in speech, but also in chewing, swallowing ...
- **Phonation:** Through repetition of sentences (read by the parents), reading a text or the naming of objects presented on pictures, we try to evaluate the articulation; we seek the presence of loss through the nose during speech (taking into account the fact that nasal sounds are more common and normal in English than in French)
- **Fine motricity:** How does the patient write; how does he reproduce a sequence of gestures?
- **Gnosia:** Gnosia is the recognition of an object through one of the senses; in this specific case we wanted to test lingual gnosis: does the patient recognize a geometrical form put in his mouth?
- **Sensoriality:** What is the attitude of the patient when he is presented sensory material (balls of various shapes, "slime paste" ...)?
- **Breath:** How does the subject direct his breath? How does he adjust the airflow? What is its power?
- **Olfactive sensoriality:** What is the patient's reaction to different smells presented? What reaction do they evoke in him?



Caleb (age 2, BTHS) participates in Florence's research study with the help of his brother, Jordan

Then the conference ended, after a memorable closing "Power Up" theme party, and we flew back with twenty hours of video recordings and several thousand responses to analyze. This will keep us busy during the coming months.

But for my part, and I believe that Florence and Catherine will not disagree, I left Florida enhanced by meeting amazing people touched by the very strong link that unites these families, sad to leave each other and happy to say that they will meet again in two years. (Photos courtesy of Florence ²⁰¹⁸)

¹Institut National de la Santé et de la Recherche Médicale, French public scientific and technological institute which operates under the joint authority of the French Ministries of Health and Research. The institute is dedicated to biomedical research and human health.

Association Syndrome de Barth France

Barth Syndrome on the Finish Line of the Ironman World Championship!

By Quentin Kurc Boucau and Benjamin Proux, Ironmen

On Saturday, October 13, 2018, we were in Hawaii. This was a triathlete paradise, with the IRONMAN world championships finale. The program includes: 3.8km swim in the Pacific Ocean, 180km bike ride on Queen K, and 42.2km run also on Queen K.

Any world triathlete practicing Ironman dreams about Hawaii...but you need to deserve it! The world championship finale is only reachable with a "slot," a qualification. To obtain it, you must rank in the top three of your age group during one of the Ironman races around the world.

This year, we had two representatives for Barth France. There was Benjamin Proux, 39 years old, who earned his slot at Ironman Cozumel in Mexico and Quentin Kurc Boucau, 30 years old, who earned his slot at Ironman Panama City in Florida.

We are two very different triathletes, but we fight with the same motivation and with the same involvement for the same cause: to develop Barth syndrome awareness.

We were only two racing on D-day, but we felt like 15 or 20 or even more, as the Barth family is so united and there is so much strong energy emerging from this association that motivates us.

Hawaii is one of the most demanding Ironman contests, particularly because it brings together the best triathletes worldwide. We started on a long and challenging day, but it was full of pleasure as we got to practice our hobby.

During the Ironman preparation, and even more when we were in Hawaii, we thought about our friend, Raphael, who hopes to race one day. We thought about his parents who fight every day to bring him a better life. It's a driving strength that helped us move forward in the many tough times that we faced during such a long race. Thinking about Raphael in front of his computer following our progress during the race gave us a stronger will to succeed than did the desire to perform for a ranking.

We went there with and for anyone affected by Barth syndrome. We fought for them with the hope of seeing them heal one day and grow happy and carefree. Raphael, we love you! *(Photos courtesy of Florence 2018)*



Syndrome de Barth Team getting ready to run the Ironman World Championship



(L-R) Benjamin Proux and Quentin Kurc Boucau
Last training on Ali'i Drive (Hawaii)



Benjamin Proux crossing the finish line with the Barth flag

Barth Italia Onlus

Barth Italia Store a HUGE Success!

By Paola and Paolo Muller, Barth Italia Onlus

Last summer Barth Italia was at BSF's 9th International Scientific, Medical & Family Conference with three Italian families and five researchers: it was really important for us!!

Despite the language barrier, our families had a wonderful time with the amazing BSF community: Matei, Teodor, and Valerio met their "Barth brothers" for the first time, and Pietro had a great time with old and new friends.

First-time conference attendees Elena and Simona participated in the carrier sessions and had the opportunity to meet other Barth moms who shared their stories, while Carlo and Catalin, along with Paolo, met other Barth dads in the dads' session.

It was an important time to keep in touch with our own feelings and all of us allowed ourselves to feel our pain together with the others.

It is difficult to explain the emotional intensity of the conference experience.

It is also paradoxical to feel completely at home with new people we have just met because we share the worst thing that has happened in our lives – Barth syndrome.

Every time we meet our Barth family, we feel we have a big mission to fulfill. So, before taking part in our second conference, we decided to try something new, the Barth Italia Store.

We have a beautiful logo, everybody loves it, not only Barth people: in our town a lot of people know it because the jewelry with which we had some successful fundraising.

At the traditional fundraising event that we have in June in Monza to support the Italian doctors' conference, we presented a lovely fashion show, in which Pietro and his friends were Barth models, for one night.

It was funny and such a real success!! Everybody enjoyed the show and supported us by purchasing our PowerUp t-shirts.

Since people in Monza love our logo so much, we wondered how successful it would be in the Barth community, where it's so significant.

So, we shipped boxes and boxes of our Barth merchandise to raise money in support of this very important conference. It was a HUGE success, and it was really appreciated.

It was also so rewarding seeing the children wearing coloured t-shirts with our PowerUp logo, moms wearing our Barth jewelry, and dads... sometimes a little bit worried about their credit cards...

I wish to thank everybody for your interest in our Barth Italia Store and for helping us to support the conference.



Barth Italia was very present at the conference



Paola, William (age 7, BTHS) and Paolo proudly display Barth Italia merchandise



Barth Italia store raises funds to support BSF conference
(Photos courtesy of Paola 2018)



Barth Syndrome Foundation

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Barth syndrome (BTHS; OMIM #302060) (ICD-10: E78.71)

A rare, life-threatening genetic disorder primarily affecting males around the world. It is caused by a mutation in the *tafazzin* gene (*TAZ*, also called G4.5), resulting in an inborn error of phospholipid metabolism.

Though not always present, cardinal characteristics of this multi-system disorder often include combinations and varying degrees of:

- **Cardiomyopathy** (*usually dilated with variable myocardial hypertrophy, sometimes with left ventricular noncompaction and/or endocardial fibroelastosis*)
- **Neutropenia** (*can be chronic, intermittent, cyclic, or not present*)
- **Low muscle mass and muscle weakness**
- **Growth delay** (*short stature in the early years, followed by accelerated growth in mid- to late puberty*)
- **Exercise intolerance** due to early fatigue
- **Feeding problems** (*e.g., difficulty sucking, swallowing, or chewing; aversion to some food textures; selective or picky eating; frequent vomiting*)
- **Cardiolipin abnormalities**
- **3-methylglutaconic aciduria** (*variable but typically a 5- to 20-fold increase*)



Henry (age 1)
(Photo courtesy of Amanda Clark 2018)



Mario, Jr. (age 15)
(Photo courtesy of Amanda Clark 2018)

The personal opinions expressed in this newsletter are those of the authors of each article and do not necessarily reflect the views of the Barth Syndrome Foundation.