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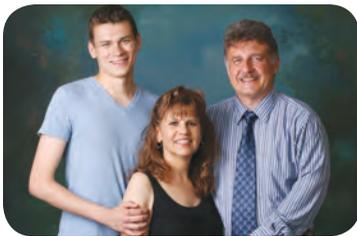
Barth Syndrome Journal

Volume 17, Issue 2 ~ Fall/Winter 2017

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

2018 BSF Conference: Share in the Breakthroughs of Tomorrow, Today

By Marilyn Toth, Wife of Barth Syndrome Foundation Science Director



(L-R) Jeff, Marilyn & Matt

My first BSF conference was in 2008 with my husband, Matt, who is the Science Director of BSF, and our son, Jeff, who was 16 years old at the time. I was finally able to place faces with the names I had heard Matt speak of so often. I not only was able to meet the affected boys and their families but also the scientists and doctors who were working towards a treatment and who cared for many of the boys. I was struck by the strong sense of community and came to realize how different this conference was going to be.

(Cont'd on page 4)

Note from Shelley Bowen, Director, Family Services & Awareness

We have previously highlighted stories about families who have gone to extraordinary lengths to obtain a diagnosis of Barth syndrome. Having a diagnosis provides valuable insights into the underlying condition and its spectrum of risks and also allows for a medical approach that appreciates the entire disorder. In this edition of the newsletter, we are highlighting three stories about remarkable doctors who also took great steps to obtain a diagnosis of Barth syndrome for one of their patients and the enormous difference this has made.

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Our Journey for a Diagnosis

By Leah, Mother of Affected Son (age 5), Arizona

"The information from the Barth Syndrome Foundation's website has given me, as a mother, a sense of empowerment. I am able to provide quality information to my son's doctors and teachers, and this allows Isaiah to have a customized plan of care that fits his unique needs." ~ Leah, Mother of Affected Individual, Arizona



Isaiah

When Isaiah was born in November 2012, I knew becoming a mother would change my life, but I had no idea just how much this little boy would impact me. From the very beginning, Isaiah was a good baby: he ate well, slept well, and fussed very little. Not only was he a good baby, he was also a very healthy baby; each time I would take Isaiah to the doctor for his checkups, he would measure on the small side of the spectrum, but he was growing consistently and he was thriving. Life seemed to be off to a wonderful start for my new family, and we were excited to watch our little boy grow and learn new things.

(Cont'd on page 8)

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E-mail: matthew.toth@barthsyndrome.org
Skype Name: matt_bsf

Au Revoir — Always Part of Team Barth

By Lindsay Groff, Executive Director, Barth Syndrome Foundation

"The last six years have been a time of great personal fulfillment and organizational progress. I have treasured this time being your Executive Director. We have celebrated some incredible moments together, and we have mourned tragic losses." ~ Lindsay Groff, BSF Executive Director



Lindsay Groff
*(Photo courtesy of
Amanda Clark 2016)*

Sadly, this will be my last update as Executive Director of the Barth Syndrome Foundation (BSF). I announced my resignation to the community with a heavy heart, yet an abundance of hope back in October.

The last six years have been a time of great personal fulfillment and organizational progress. I have treasured this time being your Executive Director. We have celebrated some incredible moments together, and we have mourned tragic losses.

While there is still far more work to be done, what we have accomplished over the last several years has, undoubtedly, made great strides in improving the lives of those affected by Barth syndrome and great strides toward our ultimate goal of effective treatments and a cure. This is something we can all take pride in.

As is often the case, it's the people who have made these experiences exceptional. I have especially enjoyed getting to know many of you through the listserv, by email, on the phone, and in person. Spending time together at the last three BSF conferences made for lasting memories that I will take with me. I look forward to seeing the photos from our talented photographer, Amanda Clark, as well as all of the candid photos that will be shared from the 2018 conference.

Moving forward, I am committed to assist BSF over the next couple of months to ensure a smooth transition of my responsibilities. Shelley (Director, Family Services & Awareness), Matt (Science Director), and Lynda (Executive Assistant) will, of course, continue to do all of the great work they do. As well, the Board of Directors will continue to chart the direction of the organization and be available for more hands-on involvement through this transition.

In terms of transition, the Board has already begun the process of finding the next Executive Director for the organization, and I plan to assist in this process in any way that I can. BSF is an amazing organization that is as strong as ever, and I'm absolutely confident that it will continue to flourish in the future. Although I am leaving this position, I will always be a part of Team Barth. You have touched my heart in so many ways. So, this is not goodbye, but merely see you next time, albeit in a different capacity.

With gratitude,

Lindsay B. Groff

Lindsay B. Groff

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.

Embracing Change

By Marc Sernel, Chairman, Barth Syndrome Foundation

Change can be difficult. We all have routines and embrace what we know, and can bristle at the notion of something disrupting what is familiar and comfortable. I, for one, am one of the most change-averse people around — I live in the same town in which I grew up, have worked for the same law firm for nearly 20 years, and enjoy the same routine. But sometimes things have to change, and many times that change can turn out for the best, even if we would not have chosen it in the first place. Starting a new job, going to a new school, moving to a new city, starting a family — all of these life events can be intimidating but also immensely rewarding. Sometimes you need to opt for change, jump at a new challenge, and have faith it will work out for the best.

This past fall each of my three children faced the change of moving on to new schools — our daughter starting high school, and our twin boys moving up to middle school. They each had liked their prior schools and voiced some uneasiness as the new school year approached. My wife and I were particularly worried that our son with Barth syndrome might have less than a smooth transition, given the much larger school and new challenges that awaited him this year. Middle school can be a tough time for any kid, but especially so for a child facing the additional challenge of Barth syndrome. Thankfully, I can report that all three have successfully adapted to their new schools and now profess to like the new schools even more than their previous ones. The change in schools has turned out for the best.

Some change is also in store for the Barth Syndrome Foundation, and we are going to do our best to ensure we make the best of this change too. As many of you know, BSF's Executive Director for the past six years, Lindsay Groff, has chosen to move on to a new challenge in her career. I will especially miss Lindsay because, in my role as Board chairman, we have had weekly and many times daily interactions, and I know first-hand how she has improved BSF and its operations in countless ways. While I might have preferred the status quo of working with Lindsay for a little longer, we all wish her well in her new endeavor and are now focused on embracing this change and finding a new leader who can build on Lindsay's successes.

The search for BSF's next Executive Director is well underway. Susan McCormack, the vice-chairman of our BSF board, is the chair of the search committee, which also includes Susan Osnos, Kevin Woodward, Kate McCurdy and me. We have retained an outside consulting firm to assist in the search process and hope to start meeting candidates in early 2018 and identify the right one soon thereafter. Given all that we have done together in building BSF into what it is today, we understand the importance of hiring a new leader who will embrace our culture and values, while also bringing new ideas and skills to our collective effort.

We will survive and thrive through this transition, just as we have continued to thrive despite the natural turnover on the BSF board. Due to term limits recommended by the National Health Council and imposed by our by-laws, we have seen many longtime board members roll off the board in recent years. I will join that list in April 2018 and will be replaced as chairman by Susan McCormack. It is thus vital that we continue to find new and talented board members, and in that regard I am happy to report that Megan Branagh was recently elected to a three-year board term. Many of you have met Megan and her family at the last two conferences or read about Megan's amazing fundraising efforts with her annual Happy Heart Walk that she started when her son was diagnosed with Barth syndrome. I am excited to have another can-do person like Megan on the board to help propel this organization forward.

I believe that the Barth Syndrome Foundation is as strong as it ever has been. That strength is due to the unbelievable efforts of many dedicated people, some of whom have been with the organization since the very beginning. But that strength has also come about because the organization has been willing to change, adapt, and re-invent itself to tackle new challenges. We embrace the change and the challenges that our success has brought us, and we look forward to elevating the organization to even greater heights in the years to come. As always, we thank you for your support.

Barth Syndrome Foundation (BSF)

2005 Palmer Avenue #1038
Larchmont, New York 10538
Toll Free: 855-662-2784 or 855-NO-BARTH
Local Line: 914-303-6323
Fax: 518-213-4061
E-mail: bsfinfo@barthsyndrome.org
Website: www.barthsyndrome.org

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2018 BSF Conference: Share in the Breakthroughs of Tomorrow, Today

(Cont'd from page 1)

"...The biennial meetings of which I was privileged to attend the first three have become very popular, attracting many affected families, medical specialists and scientists, all interested in Barth syndrome."

~ Peter G. Barth, Emeritus professor of congenital diseases of the nervous system, Emma Children's Hospital, University of Amsterdam, the Netherlands

Jeff and I attended a variety of the Science and Medicine talks which we found to be very interesting. Since I have a nursing background, it was especially informative to speak personally with some of these scientists and learn more about their work. It was impressive to see all the different areas of research presented according to the symptoms which impact Barth syndrome individuals. The clinics held at the conferences are a way to gather information that is useful and critical to doctors, scientists, and families. My son, Jeff, although not affected by Barth syndrome, has participated in some of the clinic studies to help better understand this disease.

As each day of the conference progressed, Jeff and I were able to chat with some of the families at mealtimes. The social, which is held on the last evening of the conference, was a fun way to get to know and dance with the BSF community members. It was heartening to see the boys having a great time. BSF is really like one big family with the same hopes and dreams for a better tomorrow. On the last afternoon of the conference, I remember feeling so touched by the slideshow presentation of all the BSF families. It was an enjoyable conclusion to a special conference that has the potential to enrich every participant's life.

Since 2008, Jeff and I have attended more BSF conferences and enjoyed meeting old friends and making new ones. Each time, I am overwhelmed with emotion as I participate in the conference. It amazes me to see how everyone involved works tirelessly in their diverse roles to make these conferences a great success each time. As for the boys and their families, I have the greatest regard for their perseverance and their abilities to overcome the obstacles they face daily. These families are my true superheroes.

At the conference families are able to share their personal stories with one another making it an event that is valuable and encouraging. I believe that we gain so much from meeting individuals who have been through a certain experience. The knowledge received from them is priceless. It also brings some level of comfort to speak with others who have had a similar experience.

As the 2018 BSF conference approaches, why not come and share in the knowledge, experiences, and love that this unique community possesses.



Conference attendees at BSF's 2016 conference (Photos courtesy of Amanda Clark 2016)

SAVE THE DATE!

9th International Scientific, Medical & Family Conference

July 16-21, 2018

Clearwater Beach, Florida, USA

Save the date! The 2018 Barth Syndrome International Scientific, Medical & Family Conference is scheduled for July 16-21, 2018 at the Hilton Clearwater Beach Resort located in Clearwater Beach, Florida. The hotel is right on the beach with two pools, many restaurants, ample shopping, and exciting activities all within walking distance.

The Barth Syndrome Foundation (BSF) is soliciting speaker abstracts for the Scientific & Medical Sessions of the 9th International Barth Syndrome Scientific, Medical & Family Conference. The Sci/Med sessions will take place on Thursday and Friday, July 19 and 20, and presentations should cover clinical and scientific areas directly related to Barth syndrome. Invited speakers will have 30 minutes to present. **The deadline for Speaker Abstract submission is March 1, 2018.** Please submit your Poster Abstract to Matthew Toth at: Mtothbsf@comcast.net or matthew.toth@barthsyndrome.org.

We expect to invite ~ 18 speakers to describe their work caring for Barth syndrome individuals, or how their work furthers the goal of finding a specific treatment for Barth syndrome or addresses aspects that relate to the pathophysiology of Barth syndrome. In order to have a fair and orderly process, we are asking the potential speakers to provide us with a one-page abstract (less than 400 words) describing in general terms what they will present. Based on these abstract submissions we will choose the speakers and assemble the agenda for the SciMed sessions. The Barth Syndrome Foundation will provide travel expenses, hotel accommodations, and common meals for the invited speakers, unless they are able to generously use their own funds for this purpose. In addition, the 2018 Conference will have a Poster session (on Thursday afternoon) along with a Poster stipend program that can help defray the cost of attendance if one is not invited as a speaker. For 2018, we expect to ask four poster presenters to speak at the Friday afternoon session for 15 minutes each.

Call for Poster Abstracts

The Barth Syndrome Foundation 2018 Scientific and Medical Conference Organizing Committee (COC), comprised of members of the Barth Syndrome Foundation international Scientific & Medical Advisory Board, invites the submission of abstracts for poster presentations related to the scientific and/or clinical aspects of Barth syndrome. **The deadline for Poster Abstract submission is May 15, 2018.** All submitted abstracts will be peer-reviewed by the COC. Once accepted, the submitting author will be expected to present his/her corresponding poster at a specific time during the Conference. Please submit your Poster Abstract to Matthew Toth at: Mtothbsf@comcast.net or matthew.toth@barthsyndrome.org.

All Conference registrants are encouraged to submit abstract(s)/poster(s) of their work. Poster presenters are also encouraged to apply for a stipend to help defray the cost of their attendance. **The deadline for Poster Stipend submission is May 15, 2018.** Program and application information will be available at www.barthsyndrome.org.

Scholarship Program

The Barth Syndrome Foundation offers a limited number of travel scholarships for qualifying physicians, clinical residents/fellows/students, nurses, and other allied health professionals to help defray the cost of attending the 2018 Conference. This program is designed to encourage medical practitioners to increase their knowledge about and improve their care of Barth syndrome individuals. **The deadline for Poster Stipend submission is May 15, 2018.** Program and application information will be available at www.barthsyndrome.org.

Why You Need To Attend:

“My attendance at BSF’s conference was invaluable in learning about patients with this disorder and about scientific progress into the mechanisms of disease and genotype-phenotype correlations.” ~ Arnold W. Strauss, MD, BK Rachford Professor and Chair, Department of Pediatrics, University of Cincinnati College of Medicine; Director, Cincinnati Children’s Research Foundation; Chief Medical Officer, Cincinnati Children’s Hospital Medical Center, Cincinnati, Ohio

Clinical Trials — Our New Reality

By Mathew J. Toth, Science Director, Barth Syndrome Foundation

"We need our Barth brothers to step up and volunteer as much as they can. These volunteers not only have the potential to help themselves, but they also will help their fellow Barth brothers who cannot participate, and they may help other people afflicted with similar serious diseases." ~ Mathew J. Toth, PhD, Science Director, Barth Syndrome Foundation



Dr. Matt Toth

It has been almost 12 years since I first came to the Barth Syndrome Foundation (BSF) and attended its biennial conference. At the 2006 BSF conference, I was introduced to many of you, and I spoke briefly about what we all wanted to hear — how will we find a treatment for Barth syndrome? Though it has taken a long time, the hope I spoke about in 2006 is now coming into focus.

At the 2016 BSF conference (and before), we began to hear about the term clinical trials, and several other BSF members and I spoke to many of you about what clinical trials were and how important they are in the process of finding a specific treatment. I gave a presentation about eight distinct therapeutic ideas that were being pursued. In April of 2017, the company Stealth Biotherapeutics initiated a clinical trial with Barth syndrome individuals testing their lead compound Elamipretide — the TAZPOWER study. That study is soon to be followed by another pharmaceutical clinical trial using the pharmaceutical Bezafibrate which will take place in the United Kingdom — the CARDIOMAN study. We anticipate a third trial starting soon that involves gene therapy, which is very exciting due to its revolutionary approach for treating human disease. Clinical trials of other therapies are also being planned that will keep up the therapeutic assault on this cruel disease. BSF is very fortunate and blessed to have researchers, clinicians, and members who have worked hard and struggled to be where we are now — enrolling volunteers to test specific treatments for Barth syndrome.

Just as BSF asks for donations, BSF now asks for volunteers for these clinical trials. What I said to you in 2006 has now reached a point where therapies have to be tested. Our small community needs to realize that without the timely volunteering for clinical trial testing, nothing will be advanced. If we do not complete these initial clinical trials, there will not be any more of them. Researchers and drug developers will lose interest and enthusiasm for finding a treatment for Barth syndrome. Guys with Barth syndrome will never get better.

Clinical trials are experiments on humans that test the usefulness of a particular therapy in a scientific way. All clinical trials benefit from what has come before, and volunteering for any clinical trial has great value. No one can predict whether one therapy or another (or a combination of therapies) will be useful if at all, but the process of performing any clinical trial gives the healthcare and research community a great deal of information about the disease it is tested on. We need our Barth brothers to step up and volunteer as much as they can. These volunteers not only have the potential to help themselves, but they also will help their fellow Barth brothers who cannot participate, and they may help other people afflicted with similar serious diseases.

I recently attended several meetings at the major funding and drug approval arms of the US government — the National Institutes of Health (NIH) and the Food and Drug Administration (FDA), respectively. There is new thinking taking hold at both of these organizations that heralds a renaissance in how advances in healthcare are made. A remarkable consortium of academic, industrial, and patient-advocate organizations is focusing their efforts on the patient and on rare disease sufferers in particular. The NCATS division (National Center for Advancing Translational Science) of the NIH has put together a “toolkit” to do the very things BSF has been doing for years, and it describes what we should be and what we are doing in this era of clinical trials for Barth syndrome. No rare disease will be left behind by NCATS. Patient advocate organizations like BSF are an integral and essential part of this consortium. BSF represents the only people who can volunteer to test these new drugs/treatments for Barth syndrome in the clinical trials.

The vision I described at the 2006 BSF conference is here now. This reality of the need for volunteers from our community to test therapies is exciting but sobering. The research that the BSF has supported over the years through its grant program, through its conference clinics that members have participated in, and through the scientific-medical discussions/debates that take place at the conferences and beyond, have all contributed to where getting us we are now. BSF is in a position to finally reap the rewards of what it has carefully sown and cultivated over the years. The major medical research and pharmaceutical approval agencies of the US government are now explicitly encouraging and endorsing what BSF has been doing since its inception — fostering a vibrant and caring scientific-medical-patient community to help our boys, and others like them. We are united in a struggle to lessen the suffering of not only our own members, but that of others with similar diseases. Please make sure to help us make the future better and consider volunteering for clinical trials.

(Photo courtesy of Amanda Clark 2016)

Opportunities to Participate in Barth Syndrome Research

A Phase 2 Randomized, Double-Blind, Placebo-Controlled Crossover Trial to Evaluate the Safety, Tolerability, and Efficacy of Subcutaneous Injections of Elamipretide (MTP-131) in Subjects With Genetically Confirmed Barth Syndrome

Details can be found at: <https://www.clinicaltrials.gov/ct2/show/NCT03098797?term=barth&rank=4>

Barth Syndrome Registry

The BRR empowers every person who has Barth syndrome and family members around the world to make a difference in the fight to conquer Barth syndrome. By participating in the BRR and completing your profile survey about your own unique experience with Barth syndrome, you are contributing to a global database about the accessibility of diagnosis, care and treatments, and disease severity of Barth syndrome. The BRR is a centralized resource that is vital to helping researchers learn more about BTBS, accelerating the development of new research and treatments, identifying issues that need research, and improving the care of all those with Barth syndrome.

Do You or a Loved One Have Barth Syndrome?

Dr. John Lynn Jefferies of Cincinnati Children's Research Foundation is doing a research study concerning the assessment of quality of life, anxiety, and depression in Barth syndrome. Please consider the relevant information and contact Dr. Jefferies directly if you decide to help. (*Please see below.*)

The Female Side of Barth Syndrome — Calling All Adult Women!

Dr. Cynthia James and Rebecca McClellan of the Johns Hopkins School of Medicine, Baltimore, Maryland are doing a study concerning how Barth syndrome carrier women navigate the family, reproductive, and psychological implications of being a carrier. Please consider the attached information and contact Dr. James or Rebecca McClellan directly if you decide to help.

As more boys and men are correctly diagnosed with Barth syndrome, mothers, sisters, daughters, and grandmothers face their own challenges. This questionnaire study builds on interviews we did with Barth syndrome carriers to measure the emotional, family, reproductive, and psychological implications of having a relative with Barth syndrome. We are asking ALL ADULT WOMEN to join. We hope study results will help both health care providers and patient organizations provide better care to women in families with X-linked conditions, especially Barth syndrome! (*Please see below.*)

Please visit BSF's website to learn more of these opportunities to participate in Barth syndrome research (<https://www.barthsyndrome.org>)

Our Journey for a Diagnosis

(Cont'd from page 1)

“I feel fortunate to have such an easily accessible and well maintained website to turn to for questions or family resources. Many genetic conditions do not have such a helpful foundation, and it can make it more difficult to get reliable and trustworthy information for parents and families.” ~ Kristin E. Lindstrom, MD, Genetics & Metabolism, Phoenix Children's Hospital, Phoenix, Arizona



Dr. Lindstrom & Isaiah

The day Isaiah turned six months old is a day that is so clear in my mind. It was a Friday, and I had scheduled Isaiah's six-month checkup for that day. We arrived at his pediatrician's office and went through the usual routine of measurements, and then we were sent into a room to wait for the doctor. It took longer than usual for the doctor to enter the room, and when he finally did, he explained to me that Isaiah had gained almost no weight in the last two months. He also expressed concern with the way Isaiah was breathing. He listened to Isaiah's heart for a long time and told me everything sounded fine, but he ordered a chest x-ray, just to be certain. The following Monday we took Isaiah to have his chest x-ray, and we waited to hear from the pediatrician.

The next day, I received a phone call from Isaiah's pediatrician, and my world was turned upside down. Isaiah's heart was severely enlarged, and he needed an echocardiogram immediately. The echocardiogram showed that his heart was extremely enlarged and was barely squeezing. Isaiah was admitted to the cardiac ICU at Phoenix Children's Hospital, where we were told he had severe dilated cardiomyopathy. Tests and medications began immediately, and we watched our little boy's health deteriorate very rapidly. His heart's ejection fraction was 13%, and his doctors began to discuss the possibility of a heart transplant.

Isaiah soon was unable to eat because it caused too much strain on his heart. He was losing weight rapidly and relied on a feeding tube for nutrition. The doctors also expressed concern about Isaiah's muscles, stating he had low muscle tone. The doctors ran countless tests and asked us what felt like millions of questions, but nothing seemed to give us any answers. Isaiah's cardiomyopathy was labeled as "idiopathic," or no known cause. All we knew was our boy was extremely sick, he was quite honestly knocking on death's door, and we didn't know why. All we could do was treat his symptoms, trust his doctors, and pray for God to give us a miracle.

Years went by, and we received our miracle. Isaiah's heart improved, progress was slow but steady. Isaiah has spent years recovering his ability to eat by mouth, and he has worked hard in physical therapy. Although he was significantly behind, Isaiah eventually reached each of his milestones. We are thankful for his progress, but one piece has always felt like it was missing. Why did my baby get sick? What caused his heart to fail, and, if I had another baby, would that child be at risk for the same kind of health problems? It seemed we might never have answers to these questions, and as a mother, that was something that weighed heavily on my heart.

Our answers came nearly three years after Isaiah was initially diagnosed with dilated cardiomyopathy. In December of 2015, we met with one of the geneticists at Phoenix Children's Hospital, Dr. Kristin Lindstrom. She reviewed Isaiah's medical records and looked over our family history. Dr. Lindstrom felt strongly about the possibility that Isaiah's condition was caused by a genetic disorder, and she found some abnormalities on biochemical blood and urine studies that suggested Isaiah could possibly have Barth syndrome. This led to genetic testing of the TAZ gene in February of 2016, which found a mutation but one that had never been seen before in other people with Barth syndrome. Since all of Isaiah's symptoms seemed so consistent with Barth syndrome, she felt that this was still probably the correct diagnosis. To be absolutely certain, however, she sent a special blood test for cardiolipins, which was abnormal and definitively confirmed that Isaiah had Barth syndrome.

Dr. Lindstrom not only found a diagnosis for Isaiah, but she sought out further information to help us learn what a diagnosis of Barth syndrome really meant for our boy and his future. Dr. Lindstrom found the Barth Syndrome Foundation's website and shared it with us. She used information from this site to help us learn how to deal with Barth syndrome. She was able to find a cornstarch dosing chart as well as a growth chart that has been created for children with Barth syndrome. All of this information has made a huge impact on the quality of my son's life. Isaiah will probably never grow according to standard charts, and he may never have the same nutritional needs as other children. When asked about the Barth Syndrome Foundation's website, Dr. Lindstrom said, "I feel fortunate to have such an easily accessible and well maintained website to turn to for questions or family resources. Many genetic conditions do not have such a helpful foundation, and it can make it more difficult to get reliable and trustworthy information for parents and families."

(Cont'd on page 9)

Our Journey for a Diagnosis

(Cont'd from page 8)

The information from the Barth Syndrome Foundation's website has given me, as a mother, a sense of empowerment. I am able to provide quality information to my son's doctors and teachers, and this allows Isaiah to have a customized plan of care that fits his unique needs.

No parent ever wants to hear there is something "wrong" with their child, but, throughout our journey with Isaiah, I have found that knowledge really is power. If we don't know what is wrong or why things are happening, we can't be properly equipped to deal with the road ahead of us. Dr. Lindstrom certainly seems to share the same way of thinking as we do, and we feel extremely fortunate to have the opportunity to work with her. Dr. Lindstrom didn't stop at just finding a diagnosis. She gave us a wealth of information by recommending the Barth Syndrome Foundation website to us, and she found us an amazing support system in the Barth Syndrome Foundation itself. Dr. Lindstrom did so much more than give my son a diagnosis, she changed our lives, and I know my son's quality of life will be so much greater because of her. *(Photos courtesy of Leah 2017)*

Proud Parents of a Very Rare Child

By Branislav, Father of Affected Individual (age 5 months), Slovakia

"The results of genetic tests were ready one month after the first heart failure, and BTHS was confirmed. ... I contacted the Barth Syndrome Foundation, and thanks to Shelley Bowen, we gained contact with other families and could start to learn how to live with all the complicated health problems. We were surprised how many people offered help, which we needed." ~ Branislav, Father of Affected Individual, Slovakia



Branislav, Jr.

A year ago, we were a family with two daughters and had celebrated 10 years of marriage, but then a big surprise came. My wife was pregnant. Our happiness became greater when we found out we were going to have a son. Everything was fine, and Branislav Jr. was born on May 29, 2017. After three weeks at home, my wife said, "Look at his colour." When I saw the purple-gray skin of our son, I took him to the pediatric ambulance immediately. The ride took me about three minutes, as we needed a doctor to help him with oxygen. After one hour of resuscitation, he was taken to the ER at the Children's Hospital in Bratislava, Slovakia, about 100 km from where we live.

I told my wife only parts of the story right away, as I didn't want to scare her too much. We had to wait about three hours to see Branislav, and then the biggest shock came. The doctors told us that he had little hope of living. Sometimes miracles happen, and,

in seven days, he could breathe without ventilation. As we "came up for air" after this strange week, we were "drowned" so fast after another three days when our son's heart failed again. After this moment, children's cardiology admitted him.

Back then, Dr. Kunovský said something about Barth syndrome. My wife called me from the hospital about it. I tried to google it, but I didn't understand her at first and had typed "Bart syndrome" (without the H). I didn't believe what I read, because Branislav was born with skin. This misunderstanding was explained, and I started studying the "right syndrome."

Unfortunately, our son's heart failed again in two weeks due to bad cardiomyopathy, and we thought we had no hope after another resuscitation lasting for seven minutes. The results of genetic tests were done at almost the same time, and the diagnosis of Barth syndrome (BTHS) was real for us.

It was then that we had the first interviews about heart transplantation, and we learned about a collaboration between the Pediatric Cardiac Center in Bratislava and Children's Hospital in Philadelphia, USA. The most complicated and rarest cases are presented and consulted with American experts through video conference hosted by the U.S. Embassy in Bratislava. Later the U.S. Embassy donated equipment to the Bratislava Pediatric Cardiac Center so that the Center's doctors are now able to consult directly with international experts and better treat their acute patients.

(Cont'd on page 10)

Proud Parents of a Very Rare Child

(Cont'd from page 9)



Branislav, Jr.

The results of genetic tests were ready one month after the first heart failure, and BTHS was confirmed. Unfortunately, geneticist Laszlo Kovacs died from a heart attack before our son's diagnosis. I contacted the Barth Syndrome Foundation (BSF), and thanks to Shelley Bowen (Director, BSF Family Services), we gained contact with other families and could start to learn how to live with all the complicated health problems. We were surprised how many people offered help, which we needed.

Two months later, both our daughters, my wife and my wife's mother were confirmed as carriers of BTHS, so we have to take our son's illness as preparation for our future grandchildren.

Branislav is at home now, and he makes us proud parents of very rare child. As we enjoy every little thing, other things, important in the past, became irrelevant, and long-term plans don't exist for us, as it seems. We are thankful to all family members, friends and the Barth Syndrome Foundation for contact with people sharing the same fate, who help us to live with this health condition. (Photos courtesy of Branislav 2016)

Contacting BSF — A Very Good Decision

By Christian, Father of Affected Individual, Denmark

"It gave us hope when we saw the other boys playing around and being happy." ~ Christian, Father of Affected Individual, Denmark



Atlas (age 2)

Atlas was born on the 27th January 2015, with his umbilical cord wrapped twice around his neck and as a stargazer. He quickly recovered though and everything seemed just perfect. Liva, our daughter, was so happy that she had a baby brother, that for the next three months, she only responded to "Sister" as her name. We felt so happy that we had two beautiful healthy children, and they clearly had a good chemistry from the beginning.

To begin with, we felt that Atlas was a boy like all others (much more beautiful and perfect, of course), but as time passed we saw that he was not growing at the same rate as his peers, and he was getting sick quite a lot. At that time, we felt that his periods of sickness were the reason for him not growing that much; also his motor development was slow, he had eating difficulties, sleeping issues, was a sensitive child, and had frequent diarrhea.

He was always a happy child with the cutest smile, but that changed a bit when he was around 8-9 months old. His mind was evolving, but his physique was not developing at the same rate. That left Atlas frustrated because he wanted to move, but he had no power or muscles to do so. He crawled when he was 14 months and walked when he was 18 months old.

Time passed and Zascha's maternity leave was coming to an end in December 2015, so we had to enroll Atlas at a daycare facility. After being in the daycare center for four days, Atlas got sick for two weeks, and, after that, he went back only to get very sick three days later. This time he was hospitalized for five days in isolation, and the doctors were running tests. They found his neutrophil count was very low (0.2). This was the start of one and a half

(Cont'd on page 11)

Contacting BSF — A Very Good Decision

(Cont'd from page 10)



Atlas takes Nick's dog, Mojo,
on a walk while visiting the Damin family

years of testing for all kind of diseases when his neutrophil numbers were between 0.1 and 0.7 (though nobody knew to focus on that at the time).

In Denmark we have, in theory, a very good healthcare system — a system with equal rights for all, and everything is free. The problem with “free” is that the whole mindset is “how do we save money — because free is very expensive.” That meant that testing was slow and the doctors were testing for the cheapest options first. After a year, they said they did not know how to diagnose him. But lucky for us, one doctor decided to run a genetic test. So, in April 2017, we got the diagnosis of Barth syndrome. Atlas is the only one that we know of living with Barth syndrome in Denmark and that means they do not know much about the syndrome. They told us that there was a website called barthsyndrome.org and we could go there for information. The doctor who told us about the syndrome got much of her knowledge from the same place. They then suggested that we meet for the next consultation three months later. We went home from the hospital being very unsure and not really knowing what to expect. That same night when I turned on my computer and realized what Barth syndrome meant, I was in tears. Until that moment I did not realize the seriousness of the disease. My son was to live with this the rest of his life, and I felt like I had absolutely no support at all. I felt like I had nowhere to go. So at 23:58, I wrote to bsfinfo@barthsyndrome.org, “My son, Atlas, has been diagnosed with Barth syndrome today — I feel like crying.”

That turned out to be a very good decision because not long after, I got in touch with Shelley Bowen (Director, BSF Family Services), and, for the first time, I felt like there was someone who understood me and took my concerns seriously. I also found out that there was a whole community in the world and Atlas was not the only one to have this syndrome.

We really wanted to meet somebody who understood the syndrome and who had some real-life experiences with it. Unfortunately, the conference in the US was not before 2018 and “privacy protection policies” did not allow Shelley to tell me if anyone close by to me lived with Barth syndrome. Then we found out that there was a gathering one and a half months away in England, and we were so happy. Finally, we would meet families in the same situation as ours.

June 2017 finally arrived, and we were off to England. In the preparations, we had been talking to Michaela Damin (Chair, Barth Syndrome Trust), and we were blown away by her commitment and her willingness to make the trip the best experience possible for us. We arrived by train from London to Southampton, and there we were met with open arms by Michaela who took us to our hotel. That night she took us to meet her family at the local pub. Here we met her husband and her two sons, and that was such a nice evening for us. For the first time, we met a family who had gone through the same as us, and for the first time, we met a boy with Barth syndrome. For Michaela's son Nick, who has Barth syndrome, it must have felt a little like an interrogation, but for us, it meant the world — and what a nice guy. Nick's conversation was the best, and what he is lacking physically (due to the syndrome) he makes up with his fantastic personality — a little like our son, Atlas.

That was the start of a fantastic four days for us. The next day we spent most of the day with Nick until we were off to Avon Tyrrell where we met all the other families who were attending. And what a giving group of people who made us feel very welcome; the chance for us to see so many boys with Barth syndrome was extremely satisfying. Now we could see that Barth syndrome was so many things, and it gave us hope when we saw the other boys playing around and being happy. Talking to all the boys, it was a very giving trip, not only for us but also for our daughter, Liva, who now could relate to Barth syndrome and better understand the challenges Atlas is going to face in the future.

After talking to Dr. Colin Steward by phone and all the families at the gathering, we realized that we had to try GCSF for Atlas even though our doctors were not recommending it. We also got additional heart scans.

Atlas has been on GCSF for three months now and seems so much better. His mental energy levels have risen, and, in the three months, he has evolved so much. He has started talking a little, wants to play with other children, and seems happy.

We especially want to thank Michaela and her family for making us feel so welcome, and we can't wait for the next invitation to come to England to meet all the fantastic Barth boys. (Photos courtesy of Christian ²⁰¹⁶)

Farewell to Our Friend and Doctor, Professor Colin Steward, Bristol, United Kingdom

By Michaela Damin, Chairperson, Barth Syndrome Trust



Prof. Colin Steward & Michaela

My story with Professor Colin Steward (though he is Colin or Dr. Colin to nearly everyone, so please forgive my informality from here on) began in 2001 when his lab diagnosed my son, Nick, with Barth syndrome. Desperate for information about this ultra-rare disease, we travelled to Bristol to meet him. His journey had started some time before when, as a recently promoted consultant, a colleague described the disease to him and he, along with a few other key colleagues, began investigating old cases in an effort to discover whether they might have missed a diagnosis of Barth syndrome since it was so unknown at the time. Their efforts were to prove fruitful and soon after he started finding, as well as diagnosing, new cases. He has been one of our most eloquent and persuasive advocates for the issue of under-diagnosis of our rare disease ever since.

In 2004, he organized the first-ever dedicated Barth Syndrome Clinic in Bristol to which ten affected boys were invited. Nick was one of the ten, and this meeting was to be the first of many regular and successful clinics where young people could be seen by diverse specialists. Doctors were learning from patients and their families, and families were learning from each other as lifelong friendships were forged.

Colin said at the time, "The children who attend this clinic provide wonderful examples of bravery in the most difficult circumstances. But other affected boys have lost their lives due either to late diagnosis or lack of effective treatments for the disease. Better education of health professionals and families is needed if we are to learn to recognize and beat this disease."

In 2009, knowing that his volunteer clinic was under threat, he put together a pioneering proposal to create the first national center of excellence for Barth syndrome. This patient-led center of clinical excellence was funded by the National Health Service (NHS) starting in 2010. Before this service existed, patients were often cared for by physicians who had no experience of Barth syndrome, never having seen other patients with the same rare disease.

Working with people affected by Barth syndrome and their families, Colin crafted a service which incorporates diagnosis, genetics, cardiology, metabolics, hematology, neurology, physical therapy, occupational therapy, psychology, and specialist nurses. At the core of this service are the patient and their family. People from all over the United Kingdom and Europe can access this service and receive expert multi-disciplinary, patient-centered care.



Boys and Sarah at first-ever Barth Clinic in Bristol 2004

Colin and his lovely wife, Chris, have made themselves available to patients and families, attending family outreaches and conferences. He treats every family with respect and breaks down traditional barriers by actively listening to, and learning from, the families. He is honest even when his message might be hard to hear. This has helped make him a trusted, informed, and caring physician who is a true champion of affected individuals and their families.

He has built a multi-disciplinary team of dedicated experts who will carry on his work after his retirement in late 2017 to ensure that families affected by Barth syndrome in the UK continue to have access to quality care.

In addition, he has served on the International Scientific and Medical Advisory Board (SMAB) of the Barth Syndrome Foundation since its formation as an instrumental source of advice and experience. He has also co-authored many publications about Barth syndrome including a pivotal review of this disease (Clarke et al., *Orphanet J Rare Dis.* 2013; 8: 23).

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Farewell to Our Friend and Doctor, Prof. Colin Steward, Bristol, United Kingdom

(Cont'd from page 12)



Boys & Colin at Family Information Day
(Photos courtesy of BST 2016)

In September 2017 he was awarded the Global Genes RARE Champion of Hope Award in Medical Care and Treatment – International, winning this award from among over two hundred nominations from all around the world. Together with Shelley Bowen and Lindsay Groff from BSF, he attended this prestigious event in Irvine, California to receive his award and further public awareness of Barth syndrome. An accolade which we all agree is well deserved!

As a parent of a young man affected by Barth syndrome, I have been honored to know Colin Steward and to have worked alongside him in our fight to help families with this rare disease. We will all miss him when he retires. Although we may not see him at the Bristol clinic in the future, we are happy to note that he will still serve on the SMAB, act as the Principal Investigator for the CARDIOMAN bezafibrate clinical trial, and use some of his free time to pursue his research into Barth syndrome. So this is not a final farewell, rather a chance to say thank you to an extraordinary physician and friend as he continues to search for new ways to improve the lives of those affected by Barth syndrome.

A Huge Thanks To Those Who Have Helped Raise Money

By Lindsay Groff, Executive Director, Barth Syndrome Foundation

Many of you were moved to give because you know someone struggling with Barth syndrome. They may have told you about BSF and the support they have found here. Your decision to donate is a personal one meant to help them... and in your giving, you join a special community that cares for one another.

We want you to know that we work hard to ensure that every dollar you give is used wisely. We have stretched your donations to do more each year. BSF proudly displays the logos of the Better Business Bureau Wise Giving Alliance and the National Health Council, meeting every one of their 20 Standards of Accountability and 38 Standards of Excellence, respectively. Their certification means we run a tight ship. The accelerating pace of scientific progress, and our growing and supportive community of affected families, means we are making a positive impact. Your continued support means that we have earned your trust and confidence. Thank you.

As we come to the end of another incredible year, it is time to celebrate and to take stock of our blessings and our success. May we ask you to do two things before the end of the year? First, please make your contribution to BSF. And second, please spread the word! Tell one person about BSF and why you give. Tell your story. Introduce a friend or two to our community and help us find a cure faster. You can make a difference!

A Huge Thanks to Those Who've Helped Us Raise Money

Every single dollar raised for BSF matters. Thank you for finding creative ways to raise money to help those affected by Barth syndrome. Below you'll see how some of our friends and family have worked hard to make a difference.

Sweet Travis Turned Four – Family Celebrates with Gifts to BSF

In honor of Travis' fourth birthday, his family asked for donations to BSF in lieu of gifts. Travis' parents wanted people to know that a large part of his life is spent seeing multiple specialists, dealing with long waits, vomiting, feeling sick, checking his weight, and enduring pokes. Travis sees the people at the lab, nurses, and the pharmacist, more than friends and family. On his special day, they wanted to honor their son by requesting donations to BSF instead of toys. Happy Birthday, Travis!

(Photo courtesy of Amanda Clark 2016)



Travis (age 4)

(Cont'd on page 14)

A Huge Thanks To Those Who Have Helped Raise Money

(Cont'd from page 13)



Members of Team Will

Team Will Remembers Will McCurdy at the Westchester Triathlon

Once again, members of Team Will participated in the Westchester Triathlon on September 24th. As many of you know, for the past 11 years, members of Team Will have competed in triathlons to raise money for the Barth Syndrome Foundation. This race will mark their third year racing in memory of Will McCurdy rather than in his honor. Will's passing has motivated Team Will to try to raise even more funds to help find a cure for this dreadful disease. Team member, Heather, said, "Team Will continues to grow as the next generation of triathletes joins the ranks. The momentum is strong...and Will McCurdy is in our hearts every step of the way." Thank you, Team Will! (Photo courtesy of Heather Segal 2017)



Stacey & Kevin

Baltimore Running Festival Together We Can Do Hard Things!

Together Kevin & Stacey Woodward ran 39.3 miles in the Baltimore Running Festival on Saturday, October 21st. Kevin took on the half marathon and Stacey the full 26.2 mile marathon. They run because their son, Connor, can't.

To get ready for race day, Kevin and Stacey put in an average of 20-40 miles of running per week. All this, in addition to working full-time and raising two kids. That's dedication! Their friends and family also showed their dedication by donating to BSF in Connor's honor! As Stacey and Kevin say to their sons, "Together, we can do hard things!" Yes, we can! (Photo courtesy of Stacey 2017)



Team Will's race continues

Stefan Tunguz's Participation in Santa Rosa Ironman in Memory of Will McCurdy

Stefan only started doing triathlons 10 years ago. With encouragement, training, and support, he quickly became a part of Team Will. Stefan turned 60 this past April and wanted for his birthday to do "one more" ironman and have his kids and grandchildren at the finish line. This was Stefan's fifth ironman. And, at all of them, Will McCurdy has been with him — in person or in spirit. Stefan often talks about the influence of Will and the other Barth boys. He feels blessed to have known Will. Stefan attended BSF's 2014 conference and was honored to meet many of the boys and their families. They have been a source of inspiration to him. Despite his original plans, Stefan said, "Gary and I signed up for the Wisconsin Ironman in September 2018. Will and the boys will race again in 2018!" (Photo courtesy of Stefan Tungez 2017)

In Loving Memory of Mary Stenson

Mary Stenson, 89, was a devoted mother of four and a grandmother to Matthew, Kevin, Jamie, Mary Kate and Anna Baffa who supported Kevin and his family through all their Barth related endeavors. The family has requested donations be made to the Barth Syndrome Foundation in lieu of flowers. (Photo courtesy of Baffa family)

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Rosemary Baffa & Mary Stenson

Power of Kindness

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A Huge Thanks To Those Who Have Helped Raise Money

(Cont'd from page 14)



Nathaniel
09/09/2002 — 09/23/2002

In Loving Memory of Nathaniel

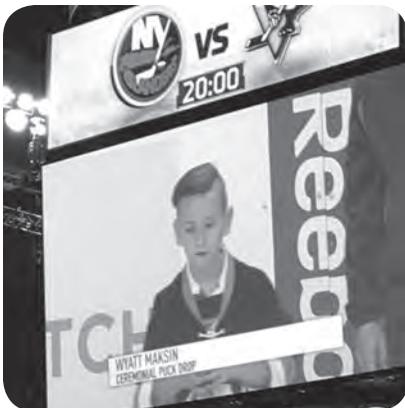
For the past several years, Nicole Derusha-Mackey honors her son, Nate's memory by requesting donations to BSF on his birthday. This year, Nate would have been 15 years old, so Nicole requested donations of \$15. Nicole says, "I'm so glad I started asking for donations on Nate's birthday — I only wish I had thought of it sooner! It definitely gives me something to look forward to on his birthday, rather than dreading it every year." (Photo courtesy of Nicole 2002)

#GivingTuesday by the Numbers — November 28, 2017

We did it. In ONE DAY, BSF raised over \$60,000. We did it together, and we did it with LOVE. Thank you all for pushing hard to make a difference in the lives of these boys and men affected by this horrible disorder. That's thanks to you, our beautiful BSF family. Imagine the possibilities in what we'll tackle next!

Barth Night at the New York Islanders

On February 9, 2018, the New York Islanders will host the second "Barth Night" where fans will learn about Barth syndrome and how they can help. A short video called a public service announcement (PSA) will be shown to all in attendance, with the goal of increasing both awareness and raise funds. Hockey super fan, Devin, will represent BSF in the video and in-person at the game. Be sure to tune in to see all of the fun! ([L]Photo courtesy of New York Islanders 2016; [R] Photo courtesy of Amanda Clark 2016)



Wyatt (age 10) featured on
Jumbotron at New York Islanders'
Barth Night in 2016



(L-R) Nicole & Devin (age 13)

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 **144** 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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"I have worked with the Barth Syndrome Foundation for more than 15 years as a physician and a researcher. They are well organized and focused on education and advancing treatment for Barth syndrome for families, scientists, physicians, and health professionals. They have a terrific record of funding research, including clinical, basic science and translational research. They have a unique conference every two years that brings together families, scientists and clinicians. The foundation continues to grow and is a model foundation for rare disease advocacy." ~ Carolyn Taylor, MD, Associate Professor of Pediatrics, Medical University of South Carolina; Director, Pediatric Echocardiography Laboratory, Children's Hospital, Charleston, SC, USA

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Barth Syndrome Foundation

2005 Palmer Avenue #1033
Larchmont, NY 10538
Telephone: 855-662-2784 or 914-303-6323
Email: bsfinfo@barthsyndrome.org
Website: www.barthsyndrome.org

Barth Syndrome Trust (UK)

1 The Vikings
Romsey, Hampshire SO51 5RG
United Kingdom
Telephone: +44 (0) 1794 518 785
Email: info@barthsyndrome.org.uk
Website: www.barthsyndrome.org.uk

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92150 Suresnes
France
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“As mother to a son with Barth syndrome this foundation is a total life line, and I mean that literally! This condition is rare and complex, so to know that there is a forum to share wisdom and expertise not only from other parents but also with medical and scientific specialists and experts in their fields and from around the world is nothing short of remarkable, you can't imagine how supportive that is and how there can never be enough thanks given!” ~ Parent of Affected Individual

Barth Syndrome Trust

Family Weekend — A Perfect Introduction

9th — 11th June 2017

By Hayley Smith, Clinical Nurse Specialist, Barth Syndrome Service Team



Hayley Smith, CNS

Having successfully applied for the Barth Syndrome Clinical Nurse Specialist (CNS) role and anticipating my start date (16th June), I was invited to the Barth syndrome family weekend at Avon Tyrrell Activity Centre.

With a little nervousness and a healthy dose of excitement, I put my weekend bag in the car and drove to the New Forest to meet the families I would soon be working with and for.

My nerves were soon dispelled as I was welcomed to the group! Over the next three days, I met two thirds of the patient group and their families for a series of fun events, such as Bush Craft (making our own fire and doughnuts!) and archery which got very competitive and has spawned a new hobby for one of the boys!

For my interview, I read as much as I could about Barth syndrome, but I learnt so much more from meeting the families. Taking part in activities demonstrated the impact of the muscle fatigue and how this affects the boys. Joining the boys at mealtimes highlighted the complexity of eating and the challenges of such an everyday activity on health, social interaction and weight management, and talking to the parents in a relaxed environment (not a hospital!) was invaluable.

This weekend for me was such a positive experience, seeing first-hand what living with Barth syndrome really means. I thoroughly enjoyed chatting with the boys, their parents and siblings and having the opportunity to get to know them a little before formally taking up my post. It was also the perfect opportunity to introduce myself and to discuss the needs of the families and how I can best support them.

My favourite memories of the weekend were Atlas's face when he saw ponies (Hest Mama! – horse in Danish) whilst we searched for hidden clues in the Treasure Hunt, Joe's excitement as he demonstrated a hidden talent for archery, and the crazy game of table tennis with the teens at 22.00 at night.

This was a perfect introduction to the guys, families and my role. I am loving the role and am grateful for the insight this weekend provided. (Photos courtesy of BST 2017)



(L-R) Matthew, Marco & Nick (age 19) at the raft making activity



(L-R) Ashley (age 13) & Dave waiting for the high wire



Barth boys enjoying a chance to sit down during a busy Activity Weekend

Family Fun at Activity Weekend

By Isabel Easterbrook, Mother of Affected Individuals, Wales

Aneira: "That was really fun, it was the best weekend ever."

Alex: "It was great. So many people to talk to, so little time!"

Ieuan: "It was good to catch up with everyone, just very tiring."

Ralph: "Who won the rugby?"



(L-R) Mitchell (age 11), Dillon (age 12)
& Alex (age 7) enjoying the electric go-kart
(Photo courtesy of BST 2017)

It was with some trepidation that we travelled to the Avon Tyrrell Outdoor Activity Centre, as we are not the most adventurous family, and all the activity options on offer seemed a little too energetic for us. Unfortunately, we arrived late because of the distance and traffic, but hot food was waiting which cheered us up no end. After finding the bar and catching up with everyone (in that order), we checked our timetables to see what events we were participating in over the next couple of days.

Our first activity was the swimming pool - the other family who were supposed to join us (and who shall remain nameless) said it was freezing, so we had the pool all to ourselves. We survived, as luckily for us the wind was warmer than it normally is in Wales in summer. Even the rain was quite warm.

We then learned how to set forests on fire with the minimum of effort (this is called bush-craft apparently), relaxing whilst feeling the warm glow of hot chocolate and eating freshly made doughnuts.

After lunch, it was decided that teaching the children to shoot arrows was a good idea. We don't know who came up with this, but we were prepared to go with the flow. It turned out to be a barrel of laughs, with no one getting killed, or even hurt, other than Ralph who will definitely wear an arm-guard next time.

It was around this time that Aneira decided she wanted to be adopted, and other than for rare sightings (mostly at meal-times), she was not seen again by the Easterbrook family until it was time to leave.

The youngsters found enormous fun taking turns whizzing around the grounds on electric go-karts, and, as far as we could tell, the watching adults were not jealous or embittered at all.

The new Barth team from Bristol also came to meet us in these more relaxed surroundings. Hayley, the specialist nurse, made her way around to everyone, meeting the children and organising clinics. Also, in the large marquee, we met Dr. Germaine Pierre, one of the metabolic specialists taking over Dr. Steward's role, with a question and answer session taking place as well as discussions around the future direction of the Barth Syndrome Trust (BST).

On Saturday evening, we got together (in or near the bar, naturally) and had a raffle organised by Suzy Green, who, as usual, was intent on raising more vital funds for BST. (Point to note — if in a room with Suzy keep moving or you might find yourself offered as a "prize," and keep your hand on your wallet!!). Also on sale were T-shirts, running tops and other Barth memorabilia. Buy one, get another one for exactly the same price — can't go wrong really.

On Sunday morning, we took part in the treasure hunt. If we had followed the lead of Ieuan and Alex, we would even now be somewhere slightly to the west of Blackpool. Great fun though, and the chocolate treats at the end made everything worthwhile.

Ieuan was now feeling the effects of such a full weekend, and he decided to relax around the house, talking with friends and generally chilling. The remnants of the Easterbrook clan were taken on an extended tour of the grounds with historical background provided by the friendly and knowledgeable guide. When it was time to vacate our rooms, most families packed their bags and stored them in their cars, because as with most Barth events, no one really wanted to leave. A great weekend was had by all — old and young alike.

Josh

By Claire, Mother of Affected Individual, England

On 23rd February 2015, we were blessed with a perfect baby boy who we named Joshua. After nine difficult months of pregnancy, we were over the moon to finally meet our newest bundle of joy. This outlook was shared wholeheartedly by his big brother, Jamie, who was immediately besotted with Josh.

The expectation of a challenging new baby routine didn't seem to transpire. Josh was sleepy, and he struggled to feed, taking 45-50 minutes to finish 1-1.5 ounces of milk. We consulted with community midwives daily about our concerns, but it felt at times they thought we were just fussy parents. Call it intuition, but we knew that there was something not quite right, and on day four of his life, we took Josh back to the hospital. He had refused feeds for a large part of the evening, and had started to sound like he was grunting with every breath. He was admitted as a matter of urgency, and we later found that Josh had enterovirus sepsis. For the first few days, he was so critically ill that we were told there was a risk we could lose him. We were terrified. And yet all we could do was sit beside him willing him to get better.

Incredibly, after 11 traumatic days in hospital, we were overjoyed to have Josh return home to us safe and well. We were so extremely grateful for the quick response and the dedication shown by the all the medical staff who had helped him to get through this horrific ordeal, and we were very aware of just how lucky we were to have our little family back together. It was at this point we saw just how incredibly strong and determined our beautiful boy really was.

A ventricular septal defect (VSD) had been identified in his heart during his hospital stay, so we were appointed a cardiologist. Devastatingly, at his first "routine" appointment, Josh was found to have dilated cardiomyopathy and was admitted immediately to undergo tests and assessment. We were distraught: How had this happened? We hadn't noticed anything to indicate there were any issues, let alone something of this magnitude. We just couldn't believe that our little boy had yet another battle ahead. Josh, on the other hand, well, he just took it all in his stride. After a week of observation, his cardiologist was happy for us to go home, on the understanding that we would need to manage his condition with drugs every day and be vigilant in identifying any risk or sign of heart failure.

Cautiously, we began to settle into a routine, and Josh thrived. He fed much better, with the use of high energy milk and without the need for an NG tube. He was happy and very active, and he was



Josh (02/23/2015 — 04/27/2016)
and his big brother, Jamie

absolutely infatuated with his big brother, Jamie. Life with our boys was amazing, and we started to relax a little, hoping that the worst was behind us.

This was not to be.

At a cardiology appointment, bloods taken showed that Josh was neutropenic. At first, it was assumed his drugs were at fault, though it was at this point that Barth syndrome was first mentioned to us. The doctor explained it was an unlikely scenario, as it is an extremely rare condition, but as Josh was neutropenic and had dilated cardiomyopathy and as these were associated with Barth syndrome, they would carry out the test to rule out this diagnosis.

In June 2015, at the age of four months, Josh was diagnosed with Barth syndrome. There was initial confusion over his results, but we were contacted by Professor Colin Steward soon after who confirmed that Josh had tested positive for Barth syndrome. In a state of total disbelief, we had to face the reality of just how complex and problematic this disease was. Now, in addition to the ever present cardiac worries, Josh faced the risk of infection as a result of his severe neutropenia, as well as motor and development complications due to his muscle weakness. It was just so cruel and unfair that our precious boy, who had already endured so much, had so much more to contend with.

Josh was still very well, however. He was still very active and extremely determined. On the surface, he seemed to be holding his own, and we were incredibly proud of how well he was doing. Most importantly, we had two happy little boys, and we continued to enjoy each day with them. We were determined not to let Barth syndrome rule completely, and, with the introduction of G-CSF, we found that we were able to take baby steps towards a more "normal" life.

Given the enormity of the risk and all the associated complications, Josh seemed to just soldier on. We had numerous appointments, hospital visits, hospital stays – even an NG tube: something we had hoped would not be needed. Josh, on the other hand, did not approve and, after only 12 hours, decided to remove it himself! He went on to feed appropriately thereafter and was discharged shortly after following observations.

Our boy showed such incredible strength and courage time after time, that we were almost lulled into a false sense of security. In October 2015, Josh became unwell again at home. It was nothing

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Josh

(Cont'd from page 20)

to be instantly fearful of, but his feeds had reduced, and we knew he had picked up a virus of some description. We rang our local hospital and were advised to bring Josh in to be assessed. On 18th October, we remained in hospital overnight as a precaution, though doctors did not seem too worried. By the next afternoon, he was refusing feeds altogether. The hospital intervened, and he was monitored more closely. We approached the doctor on call that night about our fears that Josh was going into heart failure. He was showing many of the symptoms we had been made well aware of and was working very hard with his breathing.

On Tuesday, 20th October, Josh deteriorated rapidly and was transferred urgently to the Cardiothoracic unit of the Freeman Hospital. We were advised that he was in a very bad way and was close to suffering a cardiac arrest. It was decided that ECMO was the only solution, and Josh remained on this for just under two weeks. Medical staff warned there was a chance that he might not be able to manage without this. Once again, we began to fear the worst, and, once again, we sat beside our son and willed him to fight.

Josh was weaned successfully from ECMO, surprising everyone, I suspect. We allowed ourselves to be a little more optimistic. Sadly, only a few short days later, it was decided that mechanical support was necessary, as Josh's liver was severely enlarged. It was not a conversation we had ever imagined, even with all of the frank discussions we had previously held with Josh's doctors. The left ventricular assist device (L-VAD) was successfully placed, and we sat beside Josh for what felt like a lifetime waiting desperately for him to show us he was okay. In the weeks that followed, he began to improve day by day. An attempt was made to transfer Josh to a Berlin heart, but his clotting was an issue, and so he reverted back to a Levitronix circuit. He was placed on the urgent heart transplant list, and that was how he remained for six months.

The battle continued daily. He suffered from regular infections, and a case of flu very nearly took Josh from us in January 2016. He fought on, though, and his strong will remained. By Easter 2016, it seemed that things were looking up. Josh looked amazing, and his cheeky personality became much more apparent; from a medical point of view, things were obviously improving. We established a daily routine — not the stereotypical, but one that worked for our situation. We made sure our boys spent as much time as possible together. Jamie really



Sam & Josh

was the best remedy for his baby brother; it was clear to see how much they adored one another. The situation was difficult, but we believed that every day spent in hospital was simply another closer to Josh receiving what he needed in order to come home to us again.

Josh was truly inspirational. His strength; his courage; his determination. He fought so hard, I never once considered that we could lose him.

Devastatingly, on the 27th April 2016, we lost our beautiful, brave hero.

We are left now with a void that will never be filled. Our boy completed us, and filled all of our lives with immense joy and happiness. We will always be so grateful for every single amazing moment with him, but will forever ache without him.

Before Josh's diagnosis, we had no idea about this complex and devastating condition. Though painful, we want to help raise awareness, and help to prevent other families from experiencing such heartbreak.

On 2nd September, my fiancé, Sam, and his best friend, Chris, decided to raise funds for Barth Syndrome Trust by completing a mammoth walk across the full length of St. Cuthbert's Way (Melrose, Scotland to Holy Island, England) in two days. This is a 100+km walk normally taking four to five days. Walking has helped Sam immeasurably over the last year and a half, and this opportunity also meant he could raise valuable funds while honouring and remembering our Superhero, Joshua. (<http://samlaing64.blogspot.co.uk/?m=1>)

Amazingly, they arrived intact (just!) on Holy Island, having covered 67.5 miles in 29 hours, and as of the end of September have raised £2,220 for Barth Syndrome Trust. The initial target was £1000, but the generosity of friends and families has been overwhelming.



Sam & Chris

The walk had its difficult moments, but throughout, what kept Sam going was the memory of our amazing Joshua. Everything we do for the rest of our lives will be to love and support Jamie, and to make sure that Josh is loved and remembered for the happy, cheeky and courageous little boy he was. *(Photos courtesy of Claire 2017)*

Barth Syndrome Foundation of Canada President's Report

By Susan Hone, President, Barth Syndrome Foundation of Canada



(L-R) Board members: Susan, Chris, Cathy & Lynn

As the season of giving and love is upon us, I would like to begin by thanking all our donors, families, doctors, scientists and researchers for making the Barth Syndrome Foundation of Canada (BSFCa) the great Foundation it has become. Never have I been a part of an organization that has made me feel like family from day one. The willingness to help each other, no matter what time of day, or what else is going on in their lives is something I treasure. For those of you who provided support to our family over the last three years when Jared was going through so much, I will be forever grateful. He is now back to his normal cheerful healthy self and loving life again.

I was very fortunate to have the opportunity to meet our newest Barth family in British Columbia this summer, Caleb is the youngest affected individual we are aware of in Canada His Mom shares their story in this issue.

In September, the BSFCa Board had its yearly planning session, followed by our annual family Outreach. We reviewed 2017 and made our plans for 2018. We met at Cathy's house and discussed issues such as the 2018 conference, what the future of BSFCa looks like, budget planning and numerous other issues. It was a long, tiring day which was made easier by the great group of women involved. The following weekend, we were fortunate to get together again, only this time for fun. We were joined by Jan, R.J., and Sharon from Florida. A relaxing and work free time was held at Lake Kashabogg, Ontario.

Keeping updated on the clinical trials happening and just around the corner is my number one favourite Barth activity this year. I am so grateful that Canadian individuals were able to partake in the current Stealth clinical trial. To all those individuals everywhere who are participating in the trial, thank you from the bottom of my heart. It is a truly unselfish act. *(Photo courtesy of BSFCa 2017)*

Fun with Lasers and Locks

By Susan Hone, President, Barth Syndrome Foundation of Canada

The Barth Syndrome Foundation of Canada (BSFCa) held its annual Family outreach this past September in Toronto, Ontario. We had surprise special guests, Jan, R.J., and Sharon from Florida, who decided if they had to evacuate due to hurricane Harvey anyway, they may as well come and have some fun.

As in past years, we started our afternoon off with some fun and games. Following a request from last year, this year's activity for the young (and youngish) was a couple of games of laser tag. Our boys and men suited up, and sent their laser beams flashing. At the end of the games, scores were analysed, and the shots and strategies were excitedly rehashed.

The more sedate (and cerebral) group chose to challenge their wits in an Escape Room. The group was locked in a room, and, using clues, figured out puzzles in order to find the key to escape. It was right down to the wire, but we made it!



(L-R) Sheldon (age 16), Adam (age 27), Jared (age 23), Robert (age 31), & R.J. (age 19)

After the entertainment, it was off to a restaurant where we met up with additional family members. During dinner, families were able to relax and catch up with each other. The group was then updated about the latest Barth syndrome news, including current research

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Fun with Lasers and Locks

(Cont'd from page 22)



(L-R) Cathy, Chris, Jan, Susan & Jared (age 23)

and the exciting clinical trials, as well as BSFCa's latest efforts and plans.

A special presentation of a picture collage of some of her volunteer activities encased in a handmade frame was made to Lois Galbraith who retired from active volunteering earlier this year. Lois brought a lot of enthusiasm and caring to our Foundation, and we miss her a great deal.

We have found that these annual meetings are very important to our families. Not just for the fun activities, but they also give families the chance to ask questions, get suggestions and support each other as we continue to work towards giving everyone with Barth syndrome the best quality of life possible. (Photos courtesy of BSFCa 2017)

Caleb's First Year

By Jasmine, Mother of Affected Individual, British Columbia

"Finding the Foundation gave us comfort and hope and strength that we needed to continue on the journey alongside Caleb." ~ Jasmine, Mother of Affected Individual, British Columbia

On the morning of October 27, 2016, we were scheduled to welcome our second child into the world. What was a routine C-section quickly took a devastating turn and changed the way we thought things were going to go. Our son Caleb was born, and it was quickly apparent that he was having difficulties moving blood to his lungs as he was very blue at birth. The doctors soon decided he needed to be sent to Vancouver. Within two hours of his birth, he and my husband were airlifted to BC Children's Hospital. At this point, I had yet to meet my new son. Because I had just undergone major surgery, I was unable to fly with my son and was sent to Vancouver late that evening by ambulance; it would be 15 hours before I was able to meet him.

The first 24 hours, Caleb seemed to just need a little assistance in the form of low flow oxygen. At about 3am on the second day of his life, though, Caleb went into cardiac arrest and needed 20 minutes of chest compressions. As a result, he was placed on a heart and lung life support machine. Caleb's kidneys were badly affected from the cardiac arrest, but the rest of his organs were okay. Due to the damage to his kidneys, he was ineligible for a heart transplant. For the first five days that Caleb was on life support, his heart did not beat on its own. After 13 days on life support, we were informed that he needed to be removed from the machine due to high risk of a stroke and that he was not expected to live. The pain and agony that washed over us was numbing. We could not understand how it was that this precious little boy had been dealt this.



Caleb

Caleb exceeded expectations and survived coming off life support; he even showed some significant improvements. His kidneys returned to normal function, and his heart began to show a little progress; it began to rock back and forth rather than do nothing at all. Caleb did, however, remain on a lung support machine as his heart was not strong enough to support his lungs for breathing. Over the next few months, Caleb had some significant setbacks caused by a common cold virus, extreme swelling of the torso and head, and an infection caused by bacteria in his breathing tube. There were many times when we were sat down and told that he would not make it. There were times when we stood by and watched as people worked in a flurry to bring him back from what seemed to be the edge.

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Caleb's First Year

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“Caleb's diagnosis means that his future is uncertain, but we know that we do not face that uncertainty alone. We have the love, the prayers, the knowledge and expertise; we have the support and the guidance of a community that is living and has lived what we are living. We have the Barth Syndrome Foundations, and they have proven how mighty a community they are.” ~ Jasmine, Mother of Affected Individual, British Columbia



Caleb (age 1)

There were days when a team of 15 plus members with amazing medical reputations were stumped and puzzled as to why this was happening and what to do next. And then on November 15th, just 20 days after Caleb's first breath, we received a diagnosis of Barth syndrome.

Our family knew nothing of this syndrome before the diagnosis, and none of Caleb's doctors were familiar with the syndrome at the time. When we got the diagnosis for Caleb, there was an overwhelming feeling of isolation. A feeling like nobody "got it," nobody knew what we were feeling and facing. We were overwhelmed and exhausted from all the research we were trying to do. Caleb was given a diagnosis that in a sense is invisible to most people. The world would not understand our challenges, they wouldn't feel our pain and joy from the smallest bit of progress. The weight of this diagnosis, the uncertainty of his future, it stole our breath and exhausted us.

And then we found the Barth Syndrome Foundation website and sent out an email. Just a few days later, we received emails and phone calls from a family we never knew we had. We had contact from Barth Syndrome Foundation of Canada, and to know that there was someone else, someone so close, helped with some of those feelings of isolation. These ladies sent us packages of information, names of doctors for our

team to contact, and made personal calls to us to see what help they could offer. In finding the Barth Syndrome Foundation and Barth Syndrome Foundation of Canada, we found a community. A family that lives in this same world we'd entered, that understood our pain and our fears, a family that could give us resources, answer questions and share real life experiences. Finding the Foundation gave us comfort and hope and strength that we needed to continue on the journey alongside Caleb.

At three months of age, Caleb's heart had progressed enough that he was able to breathe unassisted, and his breathing tube was removed. At this time, he was moved from the PICU up to the cardiac ward where he stayed for three weeks before being discharged with a feeding tube in his nose and a line in his chest that provided his heart directly with a medication. Over the last eight months at home, Caleb has been admitted to the hospital three times for common colds and infected lines. He returns for monthly heart monitoring in Vancouver and has to have monthly blood draws. It is not uncommon for me to have to watch and hold him as nurses have trouble with access lines and have to re-poke him several times. Caleb remains on multiple medications and a rigid feeding regimen. He still has trouble eating food, so he continues to depend on his NG tube for nutrition. Caleb cognitively is developing beautifully, however he continues to struggle to gain weight. He is small in stature and developmentally behind. Despite the battles he has endured and the battles he will undoubtedly encounter, Caleb continues to bring light to everyone who is blessed to meet him. He is such a happy, loving one year old little boy.

Caleb's diagnosis means that his future is uncertain, but we know that we do not face that uncertainty alone. We have the love, the prayers, the knowledge and expertise; we have the support and the guidance of a community that is living and has lived what we are living. We have the Barth Syndrome Foundations, and they have proven how mighty a community they are. (Photos courtesy of Jasmine & BSFCA 2016-2017)



(L-R) Susan, Jared (age 23), Chris, Mark, Joshua, Jasmine, Caleb (age 8 months) & Jordan

Association Syndrome de Barth France Behind the Scene

By Florence Mannes, Chair, Association Syndrome de Barth France

" BSF is both a family — loving, helpful and supporting, and a huge source of information. Even if our son is affected by a rare disease, we never feel alone. BSF gives us the strength to live with Barth syndrome, and gives us hope that the future of our kids will be brighter" ~ Florence, Mother of Affected Individual, France



Winners of the Barth Poker Tournament, with Philippe Mannes

In the very first years of Barth France (now called Association Syndrome de Barth France), we were so enthusiastic about spreading awareness about Barth syndrome and about raising funds that we tended to have one (small) event each month. Each event gave us the opportunity to raise some money (between USD \$1,000 USD to \$2,000). This was amazing to us, as this was a lot of money... but it was a LOT of effort too! I remember a garage sale week-end, when we woke up at 6am on Saturday and came back home at 8pm on Sunday – all for less than USD \$1,000. And I cannot forget the way our three boys looked at us when we came back home; they had missed us as much as we had missed them.

Our ultimate goal when creating Association Syndrome de Barth France was to do our best to ensure that we would be able to spend as many years as possible with Raphael; this was not supposed to cost us so many everyday moments with him.

That's when we decided that we should organize our fundraising slightly differently. It seemed more efficient to spend our energy on three main big events than on 12 or 15 small ones.

Indeed, we can now say that we have them:

- August 2017: 7th Golf Tournament – 110 players
- November 2017: 6th Poker Tournament - 110 players
- February 2018: 4th Black Truffle Gala Dinner – 210 attendees

This is still a lot of work; however, we are very lucky to have a very dedicated family and dedicated friends who are always willing to help or even to organize the main part of the event! In 2016, more than half of the total amount raised came from those three days.

Last weekend was one of these BIG weekends: the Poker Tournament, which requires much organization. The room has to be prepared to look like a true casino (whereas it takes place in a garage), and the bar has to be ready to sell drinks and food to the 110 players plus non-players. This involves cleaning the place, installing the lighting, tables, computers, bar, and buying the drinks, food, etc. This represents two FULL days of hard work, and I am so grateful to have a brother and a sister-in-law who take care of it with such talent and efficacy. Last Friday was a success. Players said they had a great time, and we raised about USD \$15,000. It was worth staying up until 5 am (time for the tournament to end plus some cleaning...)!

As this was meant to be one of our "Big Barth Weekends," at the same time, a fireman friend of ours and team member of



Setting up for the Barth Poker Tournament

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Association Syndrome de Barth France Behind the Scene

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Ironman4barth, agreed to give the French Barth families and the Ironman4barth team “first aid training.” There were four different sessions available for up to twelve people each time. We were happy to welcome to this training Florian, who is 9 years old and affected by Barth syndrome, with his parents and his 14 year old sister. Many adults attended the training sessions, but there were also children.... you're never too young to learn lifesaving procedures. In addition, this was an occasion for French Barth families to meet, which doesn't happen as often as we would like. Since fundraising is so important to us, in order to help funding medical research, we were really happy to be able to offer something different, which is SO useful to anyone, whether affected by Barth syndrome or not.

Well, it's now time to start working on the Black Truffle Gala Dinner. It's endless... until a cure is found!



(L-R) Raphaël (age 9 - BTHS), Yohan Villedieu (Fireman - Trainer), & Florian (age 9 - BTHS)



First-aid training session w/ Yohan Villedieu



Attendees at Barth Poker Tournament

(Photos courtesy of Association Syndrome de Barth France 2017)

Associazione Barth Italia Activities Fall/Winter 2017

By Paolo, Father of Affected Individual, Italy

On October 29th, Barth Italia organized “The Halloween Barth Fair” in Monza. In the medieval central square of our town, we set up a market and sold carved Halloween pumpkins and other monstrous gadgets to raise funds and draw attention to Barth syndrome. In addition, in the nearby cemetery, children could draw, play and have terrific facial makeup done.

In the afternoon, more than 130 masked children with their families and masked Barth volunteers took part in a treasure hunt through the streets of the town. It was amazing and very important for us to see our friends helping us and working together for the success of Barth Italia’s initiatives.

In the coming months, we will be organizing fundraising and awareness events at two Charity Christmas Markets, as well as a Burraco tournament and a great charity dinner at the end of February for the World Day for Rare Diseases. We wish to support families financially to allow them attend the Barth Syndrome Foundation’s international conference this summer with the other boys. *(Photo courtesy of Associazione Barth Italia 2017)*



(L-R) Paolo & Mad Hatter

My Meeting with Barth Italia

By Carmela, Mother of Affected Individual, Italy



(L-R) Vincenzo (age 1) & Carmela

I am Carmela, the mom of Vincenzo who is one year old and is affected by Barth syndrome.

We learned about his cardiomyopathy after a simple pediatric examination. This meant the beginning of our terrifying long days — rides to the hospital and scary hours with Vincenzo in the intensive care unit. This fear threatened to break my soul, but you can’t choose some battles. Sometimes, you are thrown into some fights, and you have no choice but to go ahead. I spent time doing some medical reading, trying to understand technical words and researching the best medical center to deal with.

When Vincenzo was five months old, we arrived in Rome at the Child Jesus Hospital where we met an excellent medical team. They saved him and us!

In April, though, we had bad news again — the proposal of changing therapy and also the shocking proposal of a heart transplant. Then we met a geneticist who, just looking at Vincenzo, immediately suspected Barth syndrome. Vincenzo was finally diagnosed at the age of 11 months. At that time, we didn’t know anything about Barth syndrome. The only thing we knew was that it was extremely rare. We were alone, tired, angry...

On a blog that allows cardiopathic children’s parents to share their journey, I met Elena, another Barth mamma. She immediately reassured me and put me in contact with Paola, President of Barth Italia, who gave us new hope, and I will never stop thanking her. We met Paola and her family, Margherita and her husband, and another English family in Martina Franca at the Cardioliipin Medical Conference. The meeting was attended by so many scientists interested in Barth syndrome, among whom was Dr. Colin Steward. He gave us much advice and precious information. We found that we are not alone. We learned that many doctors are working to change our children’s lives, and we are part of a beautiful family who welcomed us and suggested some useful insights to manage Vincenzo’s life. We realize that we have to do our best to spread information about Barth syndrome and to raise funds.

Now Vincenzo is fine, and he is having therapy. We know that, in the future, we will have to fight many battles, but this will not prevent us from being happy. Facing pain through these experiences, makes you able to understand the true sense of life. All together we have climbed troubles as high as mountains, but from here, up so high, the landscape is really beautiful. *(Photo courtesy of Carmela 2017)*



Barth Syndrome Foundation

Barth Syndrome Journal Return Address
104 Stone Ridge Court
Altamont, NY 12009
Toll Free: 855-662-2784 or 855-NO-BARTH
Local Line: 914-303-6323
Fax: 518-213-4061
Email: bsfinfo@barthsyndrome.org
Website: www.barthsyndrome.org

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*)



Ty (age 3)



Kevin (age 28)
(Photos courtesy of Amanda Clark 2016)