Barth Syndrome Foundation®

BSF BEAT

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

News and updates from the Barth Syndrome Foundation Fall 2021

We Are Stronger Together

By Emily Milligan, BSF Executive Director

In this issue of the BSF Beat, we share various headlines that BSF has covered over the past few months. Some of these news items bring us great sorrow, others bring us the prospect of hope for a better tomorrow. Yet one message prevails: We are stronger together, no matter what.

You will learn that the FDA refused to file Stealth Therapeutic's new drug application (NDA) for elamipretide, which would have been the first new drug candidate for an indication in Barth syndrome. We share findings from our second clinical trial (CARDIOMAN), testing a repurposed drug bezafibrate, which did not yield meaningful therapeutic benefit for the study trial participants. Both headlines denote significant setbacks in our late-stage clinical pipeline and, with that, tried our emotional and mental resilience.

Despite these disappointments, we at BSF immediately began working to identify a possible pathway for elamipretide, while continuing to "partner with purpose" with academic, nonprofit, and industry collaborators alike on other potential treatments. We firmly believe that through this approach, we will identify and bring new therapies to our community, changing the landscape of drug development and treatments in Barth syndrome.

COVID-19 impacted us not only in our daily interactions as individuals, but also collectively as an organization. We learned that change, despite our wishes, can be reframed as positive. It has to be. The need to change brought us together online for webinars, late-night conversations on the phone, and text messages in greater concentration than ever before, not only because we wanted to, but we needed to as well. Our year-end virtual celebration on December 11th is an example of the resolve to work hard preserving the ties that have brought and keep our community together.

In This Issue

With the advent of vaccines, greater awareness about effective public health protocols, and the reopening of commerce and borders, coming together in person is slowly becoming a real possibility. BSF's Board of Directors unanimously voted to proceed with our in-person 2022 International Scientific, Medical, and Family Conference, because we need to for the sake of our families and our research collaborations.

In this time of both promise and uncertainty, we look ahead with optimism and resolve, towards a future in which we individually and collectively shape BSF. Through the best and worst of times, we are #StrongerTogether and will never ever give up.

Join Barth Syndrome Foundation (BSF) in its cornerstone, multi-track event that brings researchers, clinicians, and families from around the world together in solidarity to further our global mission.

The conference will be held at the Hilton Clearwater Beach Resort & Spa in Florida, July 18-23, 2022.

BSF is committed to doing everything we can to make the conference experience as safe and accessible as possible. We are working with experts to develop guidelines for in-person attendance as well as developing options for those who cannot join us onsite.

Registration opens in January 2022!

BSF Scimed – Partnering with Purpose

We learned during the 2018 Patient-focused Drug Development (PFDD) meeting of the universal and debilitating impact of fatigue on our affected individuals. In 2021 BSF awarded Dr. Stacey Reynolds (VCU) a grant to better understand what it means to be 'Barth Tired'. While one arm of the study aims to understand the context or qualitative aspects of the fatigue associated with Barth syndrome, here we aim to highlight the BSF-industry-academia partnership critical to advancing the quantitative arm of the study.

Dr. Reynolds' past experience with GT9X Accelerometer in studying the sleep and activity patterns of affected individuals showed the high probability of success in being able to conduct a fully remote research effort. Beginning in winter 2019, BSF reached out to the GT9X maker ActiGraph LLC for potential project support for the Barth Tired study, culminating in an in-kind contribution of 25 devices as well as software licenses for the effort. Collaborations where we can leverage the expertise of a highly engaged researcher, partnered with an industry member, are prime examples of the ways BSF is advancing our mission beyond research funding. With the launch of the "Barth Tired" study, we are developing a Barth syndrome-specific clinical measure while also deploying a template for collaboration with future and potential industry partners – because we know it takes a village to address the key challenges faced and voiced by our affected individuals. Please visit our website to learn more or participate in the "Barth Tired" study.

2021 also saw BSF's first ever co-funding partnership with the American Heart Association (AHA). Marking a strategic investment by BSF to broaden our research impact, we joined forces with AHA to accelerate progress through science and education. The two-year postdoctoral fellowship, awarded to Dr. Nanami Senoo in Dr. Steve Claypool's lab (JHU), provides research support to explore the relationship of cardiolipin and the nucleotide transporter ANT1 in cardiac models.

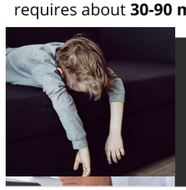
With a shared research interest in cardiomyopathy and cardiolipin, it is our goal that this effort with AHA advances Barth syndrome science while also serving as a collaborative template for other potential non-profit partners with shared clinical indications and research interests. Importantly, this program also provides a funding opportunity for trainees at the pre- and post-doctoral career stage. We look forward to the outcomes of Dr. Senoo's work.

Research Being Conducted: Fatigue in Barth Syndrome

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

You are eligible to participate if you:

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



Interested in participating or want more information?
Email the research team at:
babsoni@vcu.edu or reynoldsse3@vcu.edu

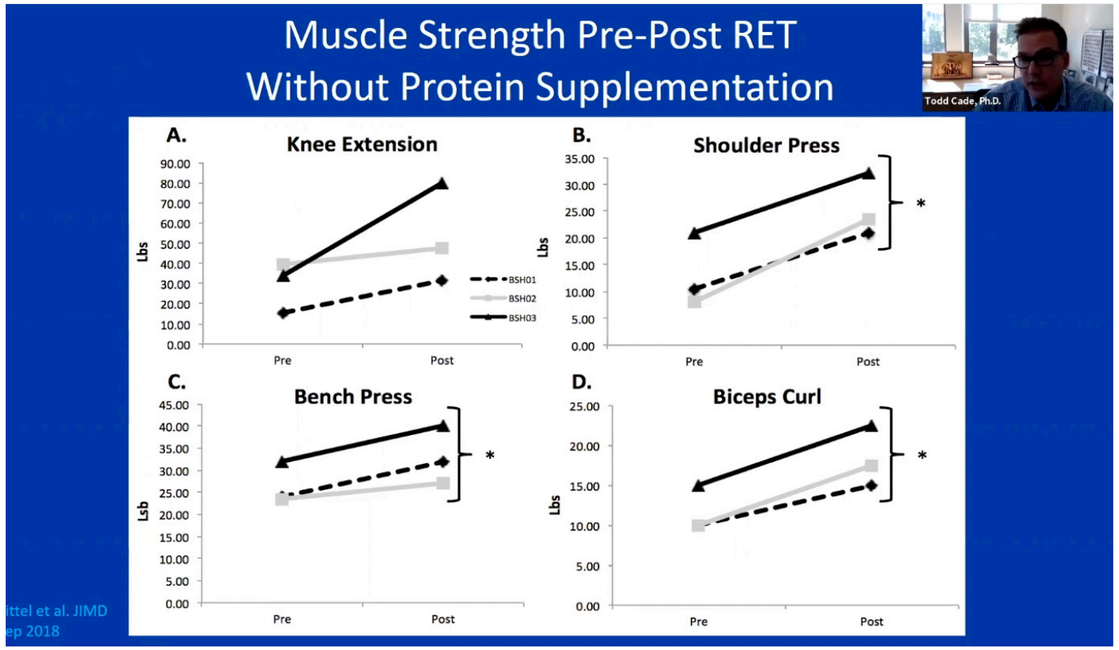
Community Roundtable: A Conversation with Dr. Todd Cade

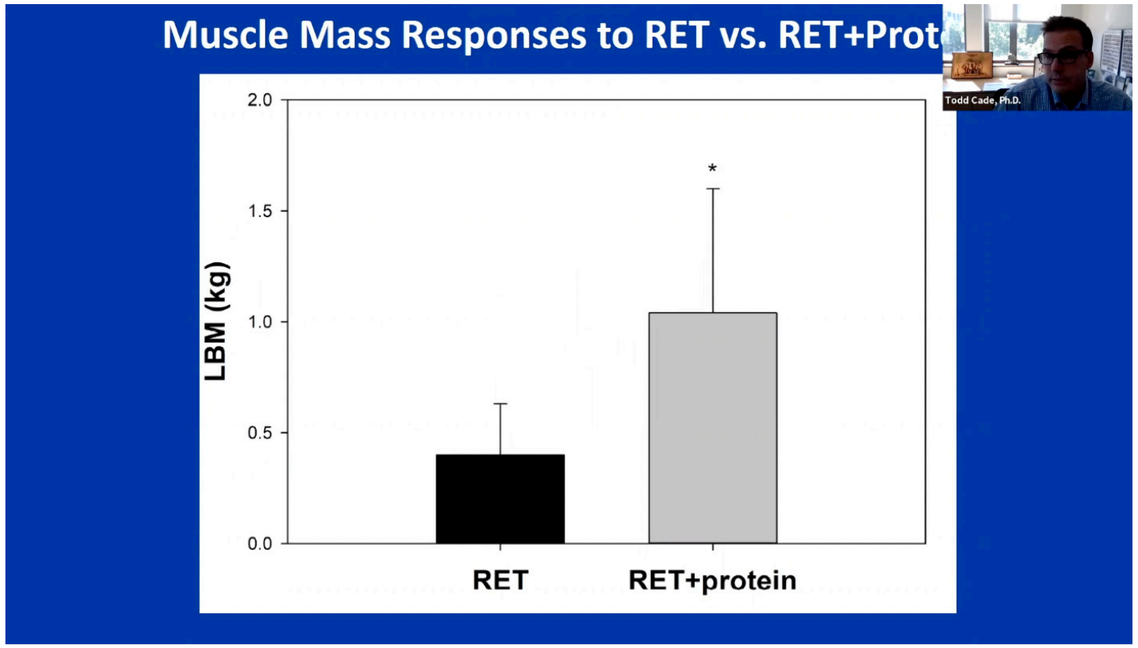
Fatigue and endurance remain critical challenges for our community of affected individuals. On September 24, 2021, Dr. Todd Cade (Duke U.) joined us for a conversation on the role and impact of resistance training in ameliorating these issues. We invited study participants as well as affected individuals who have engaged in resistance training resources and efforts to discuss their firsthand experience with this therapy.

The study was designed to explore exercise as a method to look at physiology as well as change physiology in individuals with Barth syndrome. The team first started looking at aerobic exercise training where participants rode a stationary bike three times per week up to 45 minutes. The participants' quality of life did not improve after aerobic exercise training, so they moved to resistance training.

Resistance exercise training (RET) targets type 2 muscle and working those muscle groups may improve quality of life. RET includes exercises like chest press, bicep curls, leg press, and triceps pull downs. The purpose of RET study is outlined as:

- Determine if RET is safe/feasible in affected individuals
- Characterize changes in muscle strength and performance, body composition, exercise capacity, and quality of life following 12 weeks of RET
- Determine if protein supplementation improves the benefits of RET





From the study we learned that:

- Affected individuals have lower lean tissue volume and bone mineral density and higher fat volume than control subjects.
- Muscle mass responses increased as a result of RET both with and without protein supplements, although the gains were slightly higher with the protein supplements.
- There was a significant improvement in the quality-of-life scores for RET plus protein supplements compared to RET alone.

Additional findings include:

- Modest improvements in exercise tolerance in both groups
- No change in heart function of either group
- No evidence of kidney issues with protein supplementation
- No change in absolute neutrophil count in either group
- Protein supplementation increased plasma glutamate levels but no other amino acids
- There were no significant complaints about taking supplemental protein

Watch the full roundtable, including the question-and-answer period, at:

www.barthsyndrome.org/recordings

SCAN ME

Regulatory Overview of Barth Syndrome Drug Development News

By Kate McCurdy, BSF Board Chair

It has been quite a quarter, comprised of both disappointing news and real learning. I want to shed some light on and offer a bit of perspective on those here.

Back in 2014, BSF approached Stealth BioTherapeutics (Stealth) to encourage them to consider applying their peptide called elamipretide to Barth syndrome, given what we knew about our disease and the molecule's mechanism of action. Over two years later, the company initiated the first-ever clinical trial for Barth syndrome. It was a Phase 2/3 double blind, randomized placebo controlled, crossover study that ultimately enrolled 12 subjects. After the initial study ended with results that did not demonstrate statistically significant improvement, patients were permitted to remain on the drug (giving themselves daily injections) if they wanted in order to generate additional data, and eight of the patients decided to do so. (Several original participants had stopped earlier due to side effects that subsequently were deemed to be manageable). Later, the company also went to great lengths to construct a "natural history" of select components of our disease so they could draw some comparisons These two parts of the trial demonstrated impressive improvements in participants' functional abilities (like walking farther and having more strength) and also produced evidence of changing the course of the disease in terms of some cardiac function measurements. However, because the second portion of the trial was "unblinded" (meaning that the patients knew they were taking the drug) and because a critical cardiac evaluations in the third part had not been validated specifically in our disease, the FDA decided last month not to accept Stealth's New Drug Application (NDA) for full review, stating that, according to Stealth's press release, "the NDA does not contain an adequate and well-controlled trial that provides evidence of effectiveness".

We are all extremely disappointed in both the FDA's decision to cease further review of elamipretide for Barth syndrome and also the process that led to this decision. BSF firmly believes in the FDA's mission of ensuring that all new drugs marketed in the US be proven to be safe and effective. It is clear, however, that reaching these conclusions for treatments of ultra-rare disorders, like Barth syndrome, requires a different set of consistent standards that take into account the limited number of participants eligible for trials as well as the expressed willingness of the community to make informed decisions about the risk/return profile of any treatment. In a listening session with the FDA in March 2021, the Barth community clearly expressed a great willingness to try potential treatments which had no appreciable risk and which, in their view, had great potential to improve their quality of life even if these benefits might not manifest in each individual patient. The FDA said that they had heard.

Neither Stealth nor BSF will give up on elamipretide yet, despite the now less auspicious future path, as we all truly believe that it has real promise for our disease. We will continue to work with the FDA to determine if there are additional data that could be collected that might tip the scales. We all want to chart a productive pathway forward and are working to find a way to do so. Stealth importantly also has agreed to continue to make elamipretide available to all trial participants during this time and is considering also making it available in a few months to some others not in the original trial, at least for as long as the drug is manufactured. Stealth continues to pursue the use of elamipretide for other disorders.

Unfortunately, just a week before Stealth received the negative news from FDA, BSF heard that the CARDIOMAN trial for bezafibrate in the UK did not yield successful results. This was Barth syndrome's second clinical trial and one that tested a drug that has been widely approved for another purpose in Europe for decades. This Phase 2 double blind, randomized, placebo controlled, crossover trial of 11 participants who took an oral pill ended in late 2019, and no Barth patients were still taking the drug. Though these results took a long time to analyze (due in large part to Covid delays), they were anticipated by those in the trial. Yet, it still is disappointing not to have found an effective treatment.

We are deeply grateful to all who were involved in either of these trials; without the participants, this work literally could not be conducted and no treatments would ever be discovered. Without the bench scientists who shed light on the mechanisms by which these drugs work, the idea of trying them in humans with Barth syndrome never would have happened. Without the clinical principal investigators, these trials never would have been designed and executed. And last but by absolutely no means least, without trial participants who learned about the trials and then stepped up to be those who actually took the drug (and the placebo), there is no way that these trials could proceed. It takes a village, as they say; and in these trials, the village is an international community of those who care deeply about finding treatments for our ultra-rare disease and who will not slow down until that goal has been achieved.

Despite the negative news, we will continue diligently on that quest. We have learned a great deal and will continue to prime our drug development pipeline with new early-stage potential therapies for Barth syndrome. Additionally, we are evaluating the lessons we have learned from our first forays into clinical trials and have gained some very useful experience for future endeavors. We have built a widely acknowledged reputation as a very strong patient/community partner for drug companies and as a credible voice within the FDA, and we now have first-hand insight into critical and complex elements such as trial design, endpoint selection and the importance of a strong natural history and advocacy. Our Board recently voted to make some strategic investments in various projects in these areas, and we will be stronger and even better positioned in the future as a result. In addition, Stealth has proven to be a forthcoming and respected commercial partner in this quest and has earned the thanks and appreciation of our community.

Working together as affected individuals and their families, researchers, doctors, volunteers, staff and donors, we will find treatments for this devastating disease, just not quite yet. As I wrote in a recent letter to Barth syndrome families, "...I want to assure you that this marks the dawn of a new beginning, not the end. We need to take the long view and to stay determined and united on our quest for treatments and a cure for our ultra-rare disease. The hard fact is that if we don't do it, no one else will."

BSF Core Values

Credibility • Integrity • Inclusion • Compassion • Professionalism

Barth Syndrome Foundation®

Janet Woodcock, MD, Acting Commissioner
Patrizia Cavazzoni, MD, Director, Center for Drug Evaluation and Research (CDER)
Robert Temple, MD, Deputy Director, Center for Drug Evaluation and Research (CDER)
Peter Stein, MD, Director, Office of New Drugs, CDER
Hylton Joffe, MD, Director, Office of Cardiology, Hematology, Endocrinology and Nephrology, CDER
Lisa Yanoff, MD, Deputy Director, Office of Cardiology, Hematology, Endocrinology and Nephrology, CDER
Norman Stockbridge, MD, PhD, Director, Division of Cardiology and Nephrology, CDER
U.S. Food and Drug Administration
Silver Spring, MD 20993

October 21, 2021

Dear Drs. Woodcock, Cavazzoni, Temple, Stein, Joffe, Yanoff and Stockbridge,

We at the Barth Syndrome Foundation (BSF) heard the devastating news yesterday that the FDA has refused to file Stealth BioTherapeutics' NDA for elamipretide in the treatment of Barth syndrome. Honestly, we are simultaneously both extremely disappointed and also stunned at how profoundly difficult the drug approval process is for ultra-rare diseases. We, as the relevant patient group, have been heavily engaged in Stealth's process all along the way, and we are very familiar with the data that have resulted. We have held multiple meetings with you and offered our patients' perspective, particularly about our increased tolerance for uncertainty of benefit. It is mind boggling to us that the FDA is not even willing to review this application and give it a fair hearing. We have never insisted that you approve the drug, because we firmly believe in the importance of determining safety and efficacy. Instead, we have asked simply that you give us – and the process for an ultra-rare disease with no other treatment options – the respect of fully reviewing the data.

From our perspective, this drug is safe and the totality of evidence is overwhelmingly positive. The results are compelling, and the patients who participated in the trial are completely convinced that their lives have been altered for the distinct good in very significant ways by this treatment. What's more, objective cardiac data have shown that their hearts have been remodeled and have defied the disease's natural history of cardiac worsening. These data

clearly cannot be the result simply of hope bias. Furthermore, we know the data package for elamipretide is stronger than that on which a number of other approvals have been based.

We are incredulous, and this FDA decision is incomprehensible to us. We will encourage Stealth to request a Type A meeting and to "file over protest." We feel that you are committing a serious Type 2 error and that children and young people's lives will be lost as a result.

Ironically, yesterday morning our Barth Syndrome Foundation Executive Director, Emily Milligan, served as a panelist in the EveryLife Foundation 13th Annual Rare Disease Scientific Workshop: Current and Future Barriers to the Utilization of Accelerated Approval Pathway for Novel Rare Disease Therapies. The session was called "How the Accelerated Approval Pathway Transformed Fatal Diseases into Treatable, Chronic Conditions - and Opportunities for Rare," and I strongly urge you to view both the introduction of the subject given by Isabelle Lousada, Founder and CEO of Amyloidosis Research Consortium, (beginning at 4:11 of the video) and Emily's highly relevant 10-minute presentation (starting at 10:28 of the video) as the first speaker. It can be found at: https://www.youtube.com/watch?v=7YBUocPO1_A

We really look forward to working with you to figure out a path forward to approved treatments for our devastating ultra-rare disease – both with this drug and with other treatments in the future.

Sincerely,

Kate McCurdy
BSF Board Chair and Mother of Son with Barth Syndrome (Deceased)

Stealth BIOTHERAPEUTICS | Leading Mitochondrial Medicine

October 1, 2021

Members of the Barth syndrome community:

August 2021 saw the achievement of a shared milestone – the first submission of a new drug application for elamipretide by our team at Stealth, representing the first new drug application for any investigational product for Barth syndrome. This was a Herculean effort by our small team – a new drug application is a massive undertaking even for large companies – and one of which we are exceptionally proud. While we await the FDA's decision whether to review our application (and there is no guarantee that the FDA will review it), I am reminded of the path that led us to this juncture. We would not have reached this point in the FDA process without your voice, your inspiration, and your tireless advocacy and support for the work needed to develop therapeutics for this critical medical need.

BSF leadership first approached us in 2014 to ask that we consider initiating development efforts in Barth syndrome. Quite frankly, we had reservations about the request; it is inordinately difficult to develop drugs for ultra-rare conditions not least because there are so few patients available to participate in clinical trials. Your leadership team persisted, proposing preclinical work with physician scientists dedicated to Barth syndrome research, which we undertook. I recall an early event in Boston at the Paul S. Russell Museum of Medical History and Innovation where Kate McCurdy spoke of the trajectory of the disease and the unmet medical need; attending my first BSF International Scientific, Medical and Family conference in 2014; and watching the first Patient-Focused Drug Development Meeting for Barth syndrome in 2016 (from a hotel room, while traveling on business, with a twelve-hour time difference!). Your leadership team – Kate, Emily, Shelley, your Board, and your scientific advisers – helped further our scientific interest and introduced us to the challenges of the disease. Your community's passion, drive, and optimism, and your love of all your Barth brothers (and sisters!), all underscore the reason we are in drug development in the first place – to help patients in search of potential therapies .

Whatever the outcome of our shared journey from a regulatory perspective, I want you to know that your courage in participating in clinical research and, as importantly, raising your voice and telling your story has resonated beyond your small community. When I speak to my colleagues at our company meetings, I tell your stories. When I speak to scientific and medical thought leaders about our programs, I describe Barth syndrome. You are small, but you are mighty. Your families and members of the BSF leadership team have accompanied us to meetings with the FDA, you have bravely told your stories on social media, and you have made yourselves heard in an unimaginably courageous and compelling way. You've been inspiring and amazing to work with and have invigorated our commitment-to-purpose on this long and sometimes rocky journey to discover and develop therapies for ultra-rare diseases.

You are truly exemplary of strength and solidarity through adversity. We are deeply grateful for your support and partnership.

Warm regards,

Reenie McCarthy
Chief Executive Officer

140 Kendrick St., Building C-West, Needham, MA 02494

Reflections on the Completion of a Clinical Trial in Barth Syndrome

By Hilary J. Vernon, MD PhD

As I reflect on the completion of the clinical trial to evaluate the effectiveness of elamipretide in Barth syndrome, I am most struck by what was learned, what was gained, and the relationships that were made. The knowledge and experience gained from the elamipretide trial has provided invaluable information that will enhance future trials. Scientific dissemination is critical to attract new researchers and clinicians Barth Syndrome; this study has been presented at national and international scientific meetings, during podcasts, in academic publications, and even at a congressional briefing. The study also offered the opportunity to provide specialty care to patients who did not otherwise have easy access to a tertiary care medical center near their homes.

This clinical trial was a true community endeavor. A debt of gratitude is owed to so many people including the Barth Syndrome Foundation and friends and families, the amazing study team at Johns Hopkins and the Kennedy Krieger Institute including Dr. Reid Thompson,v Dr. Brittany Hornby, Ryan Manual, and the nurses, echocardiogram technicians, medical assistants, and scheduling coordinators who made this study possible, and the study sponsor, Stealth Biotherapeutics. Most importantly, an immeasurable debt of gratitude is owed to the amazing study participants and their families. The decision to participate in a clinical trial is not an easy one to make; there are no guaranteed outcomes, side effects may be unknown, and the study activities are time consuming. Choosing to put oneself at the forefront of medical advances is an incredibly selfless act.

What has this clinical trial meant for me as a principal investigator? It represented hope for new treatments for my patients. It gave me the opportunity to really spend time with patients and their families, time that was unrushed, unfettered by constraints of insurance coverage, and time that was really, really fun. Over the 4 years, I watched study participants grow tall, graduate high school, begin college, become uncles, adopt dogs, and start new jobs.

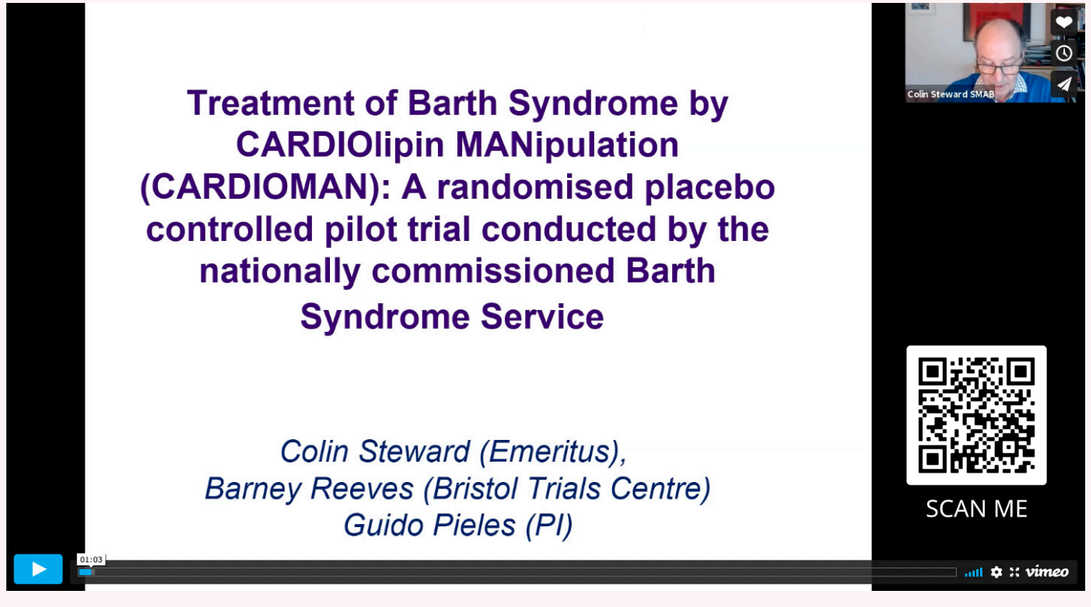
I can't presume to speak to what it has meant to the study participants, because this was a very personal experience for everyone. However, I hope that this adventure (and WOW was it ever an adventure!) has shown that when we come together as patients, families, researchers, and clinicians the possibilities are endless.

Cardioman Clinical Trial Results Update

On November 8, 2021, Dr. Colin Steward (Emeritus, U. Bristol), Dr. Guido Pieles (U. Bristol), Dr. Barney Reeves and Lucy Dabner (Bristol Trials Centre) discussed the research basis, findings, challenges, and successes for the UK-based CARDIOMAN trial. Sponsored by the UK National Institute for Health Research, co-funded by BSF, informed, designed, and led by long-time academic research leaders in our field, and ultimately made possible by the initial participation of eleven affected individuals - this nearly 8-year effort encapsulates the adage that it takes a village.

During this informal discussion of results, the presenters, SMAB members, and long-time researchers in our community joined participants and members of the BSF community to learn firsthand of how the trial did not meet its primary endpoint of improved peak oxygen consumption (VO2 max). Extended discussions regarding study design and secondary cardiac monitoring endpoints indicate the need for further analysis, with the potential to impact Barth syndrome clinical care.

Although CARDIOMAN is the second trial in our ultra-rare space to not have met its primary endpoints, this effort further demonstrates that ours is a clinical trial ready and capable population. And as we identify the scientific and programmatic learnings of both trials in our field, we look forward to applying them towards our next research study, trial, and effort – because we are a village that will never ever give up.



Watch the full recording at www.barthsyndrome.org/recordings

Rare Disease Week on Capitol Hill

More than 600 advocates from 250 patient organizations came together virtually for Rare Disease Week on Capitol Hill that took place July 14-22, 2021. Shelley Bowen, BSF's Director of Family Services and Advocacy, was among those voices advocating for the Barth syndrome and other rare disease communities. "It's important to step up, show up and speak up with peer advocacy groups," Shelley said. "Alone, BTHS affects approximately 300 people in the world. But we amplify our voice when we work with other peer advocacy groups because the issues that are important to us collectively affect 30 million Americans with rare diseases. And that is a voice that cannot be ignored."

This year marked the 10th anniversary of this empowering and inspiring week of action coordinated by EveryLife Foundation. The robust schedule provided educational and networking opportunities for attendees, culminating in "Hill Day," where advocates encouraged members to join the Rare Disease Congressional Caucus, cosponsor the Speeding Therapy Access Today Act of 2021, H.R. 1730/S. 670, cosponsor the Newborn Screening Saves Lives Reauthorization Act, H.R. 482/S. 350, cosponsor the S. 373 the Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act, and cosponsor the Access to Genetic Counselor Services Act H.R. 2144 / S. 1450.

The Diversity Roundtable was another important session during the week. Patient advocates, industry leaders, and community stakeholders participated in inclusive roundtable discussions about the barriers to care for underserved rare disease communities and the policy solutions that can make a difference. Breakout sessions focused on topics including access, representation, clinical trials and therapy development, and newborn screening and diagnostics.
To learn more about Rare Disease Week and Rare Disease Legislative Advocates, visit https://everylifefoundation.org/rare-advocates/

Stay up-to-date on all BSF advocacy efforts at: www.barthsyndrome.org/advocacy

SCAN ME

BSF is Hiring!

BSF is excited to announce the addition of two brand new staff positions to help further our mission and grow as an organization to better support our community. We are currently seeking professionals with experience in the areas of clinical research coordination and development and stewardship management. Following are brief descriptions of each position, which will remain open until filled.

To read the full job descriptions and to apply, visit www.barthsyndrome.org/careers.

Clinical Research Coordinator

BSF is hiring to fill a newly created position to foster and facilitate clinical research efforts across the Barth syndrome (BTHS) field.

In partnership with BSF's Executive Team, namely the Director of Research, this role encompasses the following responsibilities:

- Serve as the participant-facing project liaison for BSF-supported clinical research efforts, facilitating participant recruitment, engagement, and provision of logistical support
- Support multi-institutional, multi-country clinical research efforts in data collection, sharing, and subsequent return of results and findings to participants
- Managing the engagement and access of novel and external researchers to existing BTHS clinical datasets
- Implement, track, and maintain the Foundation's Global Unique Identifier program which will span across all competitive and noncompetitive research efforts and assets
- Develop the framework, protocol, and launch of tissue sample and biorepository program for the collection and use of biosamples BTHS research
- Manage the Barth Syndrome Patient Registry, driving registrant engagement, development of surveys and research tools, analysis of captured data, and subsequent communication of findings to the greater BTHS community
- Stay up-to-date on regulatory affairs that intersect with patient engagement in research or the development of treatment guidelines for BTHS

Development and Stewardship Manager

BSF is seeking a Development and Stewardship Manager to play a key role in the organization's growing philanthropic fundraising and donor stewardship. The Manager of Development and Stewardship will be responsible for collaboratively building a deeper understanding of the philanthropists with whom BSF works, crafting BSF's story to inspire donor engagement, and creating and managing effective stewardship processes that fuel ongoing commitment among our donors.

In partnership with BSF's Executive Team and Fundraising Committee, we seek to raise at least $2M annually, the role of Development and Stewardship Manager encompasses the following responsibilities:

- Tell BSF's story through writing inspiring grant proposals, grant reports, and collateral targeting our existing and potential donor audiences.
- Research and synthesize donor data using a variety of sources to develop excellent donor possibilities, as well as briefs and proposals.
- Communicate value through program outcomes data in a clear and compelling way to drive impact philanthropy narrative.
- Identify and cultivate corporate sponsorships to underwrite BSF programs that bring synergistic value to multiple sectors and audiences.
- Collaborate with Executive Director and members of BSF's team to gather data and key information required in grant proposals and reports.
- Provide excellent customer service by troubleshooting grant and donor needs as they arise.
- Inform capital investments in fundraising platforms to position BSF for successful donor stewardship.
- Manage efficient and smart systems through maintaining up to date activities, campaigns, opportunities and account information
- Implement stewardship strategies and processes to acknowledge donations in a timely and thoughtful manner, as well as support holiday and Giving Tuesday campaigns that increase giving and build loyalty to BSF

Visit www.barthsyndrome.org/careers to apply

Join the BSF community on December 11, 2021 for a virtual year-end celebration!

SCAN ME

This interactive event will be a unique experience featuring pre-recorded videos showcasing the talent of the people within our community.

For more info and to register, visit www.barthsyndrome.org/calendar

Barth Syndrome® Foundation

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Larchmont, NY 10538