



# BSF Beat

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

News and updates from the Barth Syndrome Foundation

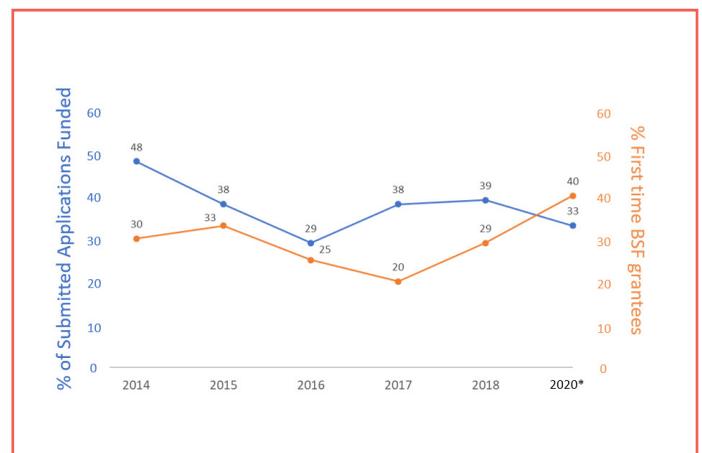
Fall 2020

## Research: 2021 BSF Grant Cycle

The BSF Grants Program welcomes innovative applications that address the basic, translational, and clinical research challenges of Barth syndrome. For the 2021 grant cycle, we are excited to announce that 13 applications have been submitted to us, spanning metabolism, disease management, research tools, and novel therapies for Barth syndrome. "The peer review process has begun, and we anticipate reporting which projects BSF will support in March of 2021," states Erik Lontok, BSF's Director of Research.

During the review process, submitted applications will be scored on the basis of three (3) criteria:

1. Importance and Impact - the extent to which the research outlined could make a significant contribution to the understanding of Barth syndrome or the treatment of the disorder.
2. Feasibility - the qualifications of the investigator(s) to carry out the proposed work based on expertise, experience, and commitment; as well as the availability of all the elements required for the project (such as technology, reagents, and research participant engagement and recruitment).



3. Prospect for Long-term Funding - the likelihood of subsequent NIH or other grant funding institution to support follow-on work.

Through this grant program, BSF seeks to provide seed grant funding to young and established investigators in order to generate the preliminary data required for successful follow-on funding available from major grant-making institutions such as the National Institutes of Health (NIH).

Updates on the BSF Grant Program will also be posted on our website as well as shared via email. If you would like to get connected, visit [www.barthysndrome.org](http://www.barthysndrome.org)

# BSF Responds to COVID-19



## 2020 Virtual Scientific and Medical Symposium

Although BSF had to cancel this year's conference due to COVID-19, we quickly went to work planning a meaningful alternative for our community. Convened on July 23-24, 2020, BSF presented its first ever virtual Scientific and Medical Symposium.

With over 300 unique viewers and composed of presenters and attendees from across the world, this event demonstrated the global scope of the BTHS community of patients, families, researchers, and clinicians. Research topics included cardiolipin and metabolism,

BTHS cardiomyopathy, pathology of BTHS, and neutropenia in BTHS.

In addition to the research presentations, two individuals with Barth syndrome, Andrew and Cameron, shared their firsthand experiences of cardiomyopathy and neutropenia, respectively.

All of the rich and diverse research presentations did not just live in the moment, as they are preserved and available for viewing on BSF's website at [www.barthsyndrome.org/research](http://www.barthsyndrome.org/research).

COVID-19 changed the world dramatically and BSF will continue responding to those changes through innovative and meaningful initiatives. Together, we #StayBarthStrong!

## Community Roundtables



BSF recognized that this has been and continues to be an extraordinary time and that the COVID-19 situation is extremely stressful and frightening for most people in our community. Parents, caregivers, grandparents, teens and adults with Barth syndrome have experienced isolation and feelings of disconnectedness during the pandemic. BSF responded to the needs of our community, and the virtual community roundtables were born.

The roundtables were developed to be community conversations and provide an opportunity to connect with others while sharing and receiving meaningful information. Since April, the community has gathered for over 25 roundtables, with particularly steady attendance over the summer. Discussion

topics included Barth syndrome carrier issues, eating/feeding challenges in BTHS, metabolic issues, family planning, and many more, each led by an expert in the respective field.

During the Zoom calls, community members had the opportunity to ask questions and share experiences, which was primary to the design of the roundtables, thereby increasing opportunities to connect. This way, they not only have been informative but also have helped mitigate isolation in the age of COVID.



You can find the schedule of upcoming roundtables as well as other BSF events by visiting [www.barthsyndrome.org/newsevents](http://www.barthsyndrome.org/newsevents)

# Advocacy: Elamipretide Update

We are an ultra-rare community, but we demonstrated the global reach of our #BarthSTRONG family in our recent advocacy efforts in support of a new drug application for elamipretide. We want all Barth syndrome patients to have access to this potential new treatment that we think is safe and has shown positive effects during the recently completed clinical trial. With your help, we received over 4,200 signatures and 730 testimonials on our BSF Community Petition that was submitted to the U.S. Food and Drug Administration (FDA) and which can be found on our website at [www.barthsyndrome.org/petition](http://www.barthsyndrome.org/petition).

We've heard of how the debilitating and constant fatigue experienced by our affected individuals can profoundly impact every daily activity. It is our aspiration that the improvements in energy, stamina, and quality of life in addition to the important positive changes in certain cardiac measures found in the assessment of elamipretide for Barth

syndrome ultimately will translate into access for our entire community. It is heartening that, with your signatures and stories, we have an opportunity to make our collective voice heard to the FDA and Stealth BioTherapeutics.

In addition, there also has been an effort on the part of key physicians and other experts with specific knowledge about caring for those with Barth syndrome to support access to elamipretide. Twenty-six prominent experts signed a letter of support stating, in part, that "we want to have the current opportunity to prescribe elamipretide to our patients with Barth syndrome" that also has been sent to the FDA and also can be found on our website at [www.barthsyndrome.org/petition](http://www.barthsyndrome.org/petition).

Though our advocacy has only begun, our community-wide efforts have supported our goal to get approval for the first-ever potential therapy for Barth syndrome.

## Q&A with Dr. Richard Kelley

### Q: At what age may a child begin taking cornstarch before bedtime?

As the most slowly digested of starches, uncooked cornstarch can sustain the blood glucose level longer than any other food given at bedtime---usually for between 4 and 6 hours. Most nutritionists and gastroenterologists do not recommend giving cornstarch until age 6 months, because the enzyme needed to digest cornstarch, pancreatic amylase, does not develop until then. However, there is variability in the amount of pancreatic amylase made between ages 3 and 6 months, and some infants cannot adequately digest cornstarch until the end of their [first year](#) (2 teaspoons of cornstarch for children aged 12-18 months). Therefore, for Barth children in their first year, bedtime cornstarch should be gradually introduced up to the calculated amount, starting at, for example, 0.2 g per kilogram and increasing in steps of 0.2 g per kilogram every 3 or 4 days.

When cornstarch is incompletely digested, there can be constipation caused by the increased stool volume from intestinal bacterial metabolism of undigested cornstarch. Whether or not to start cornstarch between ages 3 and 6 months should be discussed with the child's physician or nutritionist, who can best determine if there is a medical need for cornstarch.

Reference: Shulman RJ. Starch Malabsorption in Infants, JPGN Vol 66, Suppl 3, June 2018, S65 – S67 and references therein.

Richard I. Kelley, MD, PhD  
30-Oct-2020

You can find the cornstarch fact sheet as well as many others by visiting: [www.barthsyndrome.org/family\\_resources](http://www.barthsyndrome.org/family_resources)