Town Hall Meeting

A few housekeeping tips before we start...

Check your speakers so you can hear us

Having trouble? Contact Cristy Balcells communications@barthsyndrome.org

Submit your questions through the chat section of Go To Training Control Panel. Questions will be addressed at the conclusion of the meeting

This call is being recorded. To optimize sound quality of this recording, attendees who are joining the call will be muted.

Reach out to us from the website at www.barthsyndrome.org

Saving lives through education, advances in treatments and finding a cure for Barth syndrome
Saving lives through education, advances in treatments and finding a cure for Barth syndrome
Family Services

Shelley Bowen, Director, Family Services and Advocacy

Saving lives through education, advances in treatments and finding a cure for Barth syndrome
Family Services and Advocacy Update

• Care Management Toolkit
• Emergency Department Guidance
• Medical Binder
  Medical Record Forms
  Health History
  Contacts
• Laboratory Test Results
• Barth Syndrome by System
  Function of systems primer
  Resources to learn more (general and Barth syndrome specific)
Emergency Department Guidance

The local emergency department doctors are unfamiliar with Barth syndrome. I wish I had something I could just hand to the doctors that says DO THIS!
Medical Records

A short pencil is far more reliable than a long memory.
Contacts

80% of the population loses 80% of their cognitive thinking in an emergency situation. You don't know if you are that person until you are in that situation. Be prepared!
Barth Syndrome by System

There is so much to learn. My greatest fear is that I won’t know what I don’t know when I need to know it the most.
Coming Soon

Laboratory Test Results
Filtered by date and by finding
Charts to map trends
I have some questions for you.

Ready Set Poll!
I use the **LISTSERV** to share questions and concerns when communicating with others in the BSF community.

![Barth Syndrome Foundation](image)

**Graph:**

- **True:** 11.00
- **False:** 1.00
- **N/A:** 4.00

**Statistics:**

- **Mean:** 1.562
- **Confidence Interval @ 95%:** [1.125 - 2.000]
- **Standard Deviation:** 0.892
- **Standard Error:** 0.223

*Saving lives through education, advances in treatments and finding a cure for Barth syndrome*
I use the **FACEBOOK GROUP PAGE** to share questions and concerns when communicating with others in the BSF community.

Mean: 1.938 | Confidence Interval @ 95%: [1.519 - 2.356] | Standard Deviation: 0.854 | Standard Error: 0.213
I consider the Barth Syndrome Foundation Website as the “go to” source for reliable information about Barth syndrome.
Behind the Scenes at BSF
Lynda Sedefian, Executive Assistant
Branding Initiative

New look and feel across all BSF communications:

- Website
- BSF Beat
- E-News
- Transition to new email/donation platform
BSF Beat

- A NEW newsletter for everyone in the BSF community
- Distributed May 2019
- Includes a summary of the latest E-News over the past several months
- Sent by email and by postal mail to families and donors
- Check it out on our website News & Events /Newsletter ➔

Did you receive your BSF Beat?
Contact bsfinfo@barthsyndrome.org for your copy!
E-News

• Barth Syndrome Research Brief
• News from Executive Director

Are you receiving E-News?
Visit our website to manage your email preferences.

• Research Brief
• News from Executive Director
• Advocacy Alerts
• Donation Opportunities
• Family Heartbeat Newsletter

www.barthsyndrome.org/gogreen

From the Desk of
Emily Hilligan, MPH, Executive Director

March 28, 2019, marked another important milestone for Barth Syndrome Foundation (BSF). On Wednesday of the week, BSF spent time with representatives from the Pediatric and Congenital Heart Association (PCHA) and the National Heart, Lung, and Blood Institute (NHLBI) to discuss the role of the federal government in funding research and education initiatives in the field. The meeting was an opportunity for BSF to advocate for increased funding for research into Barth syndrome and other rare diseases.

Discovery of viable therapies to alleviate suffering and prolong life for individuals affected by Barth syndrome is the impetus behind the therapeutic research strategy employed by BSF. To that end, the 2018 research grant recipients demonstrated innovative and scientifically rigorous approaches to addressing knowledge gaps in three key areas:

1. Gene Therapies
2. Modification of Cardiopulmonary Management
3. Improving Mitochondrial Function

For 16 years, the BSF Research Grant Program has strategically funded research projects to improve the scientific and medical understanding of Barth syndrome, creating a pathway towards potential therapies. The recent 2018 cycle continues to showcase that legacy, a legacy that led directly to the CARDIOMAN clinical trial in 2019 and more than 25 awards from NIH to advance research about Barth syndrome.
Transitioning to a New Platform

- Newly created donation forms
  - General
  - Tribute
- Autoresponders automatically sent to donor
- Ability to send Ecards to honoree when making a donation in honor/memory

Look for an email asking you to update your profile this month!
New Fundraising Tool called TeamRaiser Coming Soon!

Luminate Online – TeamRaiser

• A new, interactive platform for fundraising!

• Create your own personal fundraising page, send emails and share the link, receive donations from families and friends, and even integrate with Facebook

• Looking for a few volunteers to help us test the platform – contact Lynda If you are interested in setting up your page in June

• barthsyndrome.org/donate
New Fundraising Tool called TeamRaiser Coming Soon!

Want a sneak peek? ➔ TeamBarth.barthsyndrome.org
New Fundraising Tool called TeamRaiser Coming Soon!

Create a Personal Fundraising Page in support of Barth Syndrome Foundation

Thank you - we're grateful for your support!

Please tell us about your fundraiser:

- **Have a Barth Birthday! - No Fee**
  In lieu of unwrapping packages this year, dedicate your birthday to Barth Syndrome Foundation and collect donations that will make a difference in the lives of our friends and family with Barth Syndrome! Not only do community donations support research and family services provided by BSF, your birthday fundraiser raises much needed awareness about Barth syndrome.

- **Create your own fundraiser! - No Fee**
  Create your own personal fundraising page in honor of or in memory of someone with Barth syndrome. Use this tool to customize your page with photos, your personal story, and set a fundraising goal. You can include a live event in your community as well, from a lemonade stand to a community run or a book club, or you can simply share your personal page and ask for donations to BSF to support research and family services for people with Barth syndrome.

- **Go Blue for Barth! - No Fee**
  Challenge your friends to raise a specific amount and when the goal is met, you agree to do something funny or exciting! You can dress as a superhero or cartoon character for a day, dye your hair blue, take a cold plunge, or anything else that you wouldn’t NORMALLY do! Raise awareness for Barth syndrome by setting up a challenge fundraiser!

- **Run for BSF! - No Fee**
  Are you participating in a marathon, a race, triathlon or a physical challenge event? Create a fundraising page to collect donations in SUPPORT of each step, mile or obstacle you will have to overcome. Your hard work on the course can further the mission of Barth Syndrome Foundation!

Set your Personal Fundraising Goal:

$1,000.00

Want a sneak peek? ➔ TeamBarth.barthsyndrome.org
I have some questions for you.

Ready Set Poll!
Are you receiving E-News updates from BSF?

Mean: 1.706  |  Confidence Interval @ 95%: [1.238 - 2.174]  |  Standard Deviation: 0.985  |  Standard Error: 0.239

Saving lives through education, advances in treatments and finding a cure for Barth syndrome
Have you visited our website recently to view the new look and feel of branding?

Mean: 1.176 | Confidence Interval @ 95%: [0.990 - 1.363] | Standard Deviation: 0.393 | Standard Error: 0.095
Research

Matthew Toth, Science Director
Portfolio Review – June 19th

• BSF Board members

• SMAB members

• Three presentations on near-term clinical trials
  • Gene therapy—Drs. Byrne and Pacak
  • Enzyme replacement therapy—Dr. Chin
  • ALCAT1 inhibitors—Dr. Shi

• Four external reviewers
  • Dr. Philip Johnson, former CEO of CHOP, CSO of Limelight Bio
  • Dr. R. Mark Payne, Indiana School of Medicine
  • Dr. Charles Venditti, NIH/NHGRI
  • Dr. Adam Chicco, Colorado State University
Purpose

• BSF has reached an important milestone in our mission to accelerate treatments and find a cure for Barth syndrome.
• Presently, 3 therapeutic ideas are at the cusp of clinical trial implementation.
• BSF seeks to understand specific ways that we can strategically advance the following clinical candidates within the next 1-3 years.
• Our goal is to translate scientific and business considerations of clinical candidates into future actionable steps through strong cross-sector collaboration.
Format

• LOCATION: Boston, MA
• Speakers will present for 40-minutes on topics ranging from technical considerations to partnering and financing challenges that may impact clinical development of potential therapies.
• Each presentation will be followed by question and discussion with BSF’s Reviewers and SMAB members.
• The day will wrap up with a guided discussion among the Reviewers, SMAB, and BSF Board.
• Meeting debrief will take place on June 26th at which time the Board will evaluate next steps.
Therapeutic Pipeline Grows to 5 Clinical Candidates

- Stealth/Elamipretide
- Bezafibrate
- Company X/Gene Therapy
- Enzyme Replacement
- ALCAT1 Inhibitors
I have some questions for you.

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

2018 - JULY
PFDD Meeting
BSF launches agenda to advocate for the approval of therapies and overcome the unmet medical need of individuals living with Barth syndrome.

2019 - FEBRUARY
FasterCures PFDD Guidance
BSF co-authors guide for non-profit sector on common practices, lessons learned, and considerations for planning PFDD meetings to shape R&D.

2019 - MARCH
Voice of the Patient (VOP) Report
BSF publishes the summary report that reflects the perspectives of patients and caregivers who participated in the July 18th public meeting.

LATE 2019 (?)
NDA pre-submission meeting

2018 - SEPTEMBER
Preferred Partnering in R&D
BSF joins NORD's membership and speaks to peer organizations about best practices for partnering with industry to advance therapies.

2019 - FEBRUARY
The Power of BSF’s Registry
BSF invited to speak on a distinguished panel during World Rare Disease Day in Bethesda to champion the importance of patient registries.

2019 - MARCH
FDA listens to BSF
BSF meets with more than 20 regulators to deliver VOP report and educate FDA on challenges of living with or caring for someone with Barth syndrome.
BSF Strategic Plan 2017-2020

1. Expand science and medicine program to further encourage finding treatments or a cure
2. Enhance the capacity of Barth Syndrome Foundation to better meet its mission
3. Build confidence and trust in Barth Syndrome Foundation’s community and resources

ORGANIZATIONAL GOALS 2019

- Develop and promote BSF’s therapeutic strategy and research priorities
- Successfully implement and adopt tools to broaden and deepen engagement in BSF’s mission
- Establish BSF as the premier organization advocating for therapies and policies that positively impact individuals affected by Barth syndrome
BSF Impact Assessment

**ORGANIZATIONAL GOALS 2019**

1. Develop and promote BSF’s therapeutic strategy and research priorities

2. Successfully implement and adopt tools to broaden and deepen engagement

3. Establish BSF as the premier organization

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SUCCESS METRICS 2019
*(selected examples)*

- Portfolio review successfully executed
- BSF publicizes therapeutic vision
- Contract negotiations initiated for new strategic partnership
- Family e-news launched
- Communications platform implemented and adopted by Q3
- Development committee holds 1st meeting
- BSF invited member of ≥ 2 working groups
- VOP report published to FDA.gov
- Meetings with ≥ 20 new companies and academic institutions to cultivate partnerships
I have some questions for you.

*Ready Set Poll!*
Have you read any of the “Research Brief” Monthly newsletters through email?

Mean : 1.400  |  Confidence Interval @ 95% : [1.027 - 1.773]  |  Standard Deviation : 0.737  |  Standard Error : 0.190
How would you describe the reading level content within the “Research Brief” newsletters you have received through email?

Mean: 1.929 | Confidence Interval @ 95%: [1.046 - 2.811] | Standard Deviation: 1.685 | Standard Error: 0.450

Saving lives through education, advances in treatments and finding a cure for Barth syndrome
What monthly “Research Brief” newsletter interested you the most?

- December (Research Grant Program, Cardioman, PFDD Meeting): 1.00
- January (Gene Therapy, Highlights for use of Bezafibrate): 5.00
- February (Musculoskeletal aspects in BTHS, clinical trials): 1.00
- March (2018 Research Grant Recipients): 3.00
- N/A: 2.00

Mean: 3.000 | Confidence Interval @ 95%: [2.237 - 3.763] | Standard Deviation: 1.348 | Standard Error: 0.389

Saving lives through education, advances in treatments and finding a cure for Barth syndrome
Do you want to see the monthly “Research Brief” newsletter about RESEARCH to continue?

Mean: 1.000 | Confidence Interval @ 95%: [1.000 - 1.000] | Standard Deviation: 0.000 | Standard Error: 0.000
What’s on your mind?
How satisfied are you with BSF representing your needs, interests, goals and desires in the area of **ADVOCACY**?

Mean: 2.111 | Confidence Interval @ 95%: [1.006 - 3.216] | Standard Deviation: 1.691 | Standard Error: 0.564

Saving lives through education, advances in treatments and finding a cure for Barth syndrome
How satisfied are you with BSF representing your needs, interests, goals and desires in the area of RESEARCH?

Mean: 2.385  |  Confidence Interval @ 95%: [1.455 - 3.314]  |  Standard Deviation: 1.710  |  Standard Error: 0.474
How satisfied are you with BSF representing your needs, interests, goals and desires in the area of **FAMILY SUPPORT**?

Mean: 2.769  |  Confidence Interval @ 95%: [1.877 - 3.661]  |  Standard Deviation: 1.641  |  Standard Error: 0.455

_Saving lives through education, advances in treatments and finding a cure for Barth syndrome_
How satisfied are you with BSF representing your needs, interests, goals and desires in the area of **FAMILY EDUCATION**?

Mean: 1.933  |  Confidence Interval @ 95%: [1.111 - 2.755]  |  Standard Deviation: 1.624  |  Standard Error: 0.419
How satisfied are you with BSF representing your needs, interests, goals and desires in the area of **HEALTHCARE PROVIDER EDUCATION/AWARENESS**?

- **Very satisfied**: 4.00
- **Satisfied**: 6.00
- **Not satisfied**: 1.00
- **Very dissatisfied**: 0.00
- **N/A**: 3.00

**Mean**: 2.429  |  **Confidence Interval @ 95%**: [1.589 - 3.269]  |  **Standard Deviation**: 1.604  |  **Standard Error**: 0.429

Saving lives through education, advances in treatments and finding a cure for Barth syndrome
THANK YOU!