Barth Syndrome Foundation

Barth Syndrome 9th International Scientific, Medical & Family Conference

July 16–21, 2018
Clearwater Beach, FL

POWER UP!

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

Barth Syndrome Foundation
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Welcome to Barth Syndrome Foundation's 9th International Scientific, Medical and Family Conference!

This year's conference is our largest yet, with over 50 Barth individuals in attendance. Our theme is "Power Up," and we are confident that through this week-long series of meetings and workshops we will empower our entire BSF community.

As you review this conference program, there are several areas of note. As in past years, we will be offering:

- **Clinics** (pages 4-5) – We use the word "clinics" to describe research studies being conducted at the conference. This year there are five different studies being conducted over a two-day period. These studies are crucial to furthering our understanding of Barth syndrome appropriate treatment areas. We want to thank all who participate in clinics this year.

- **Science and Medicine Sessions** (pages 10/15) – Over two days, our enthusiastic Barth syndrome researchers and clinicians share with each other and with families the latest research into Barth syndrome and potential treatments for the disorder.

- **Family Sessions** (pages 11/12/16) – Barth individuals and family members can attend sessions on topics most relevant to them.

- **Time to connect** — As you fill your schedule with the above sessions, please remember to take time to bond with those around you who truly understand what life is like with Barth syndrome.

One meeting deserves special attention from every one of us as it is truly a once-in-a-lifetime opportunity (see below). On Wednesday afternoon, Barth Syndrome Foundation (BSF) will hold a Patient-Focused Drug Development (PFDD) meeting with representatives from the U.S. Food & Drug Administration (FDA). This crucial "mini-conference" will give our community the opportunity to speak directly to not only FDA but also to representatives of various biotech and pharmaceutical companies about what treatments **we** would find most helpful. We urge everyone to make time to attend this important meeting!

We at BSF are proud to present this conference to our community and hope that it serves to Power Up our mission:

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

PLEASE PARTICIPATE IN A ONCE-IN-A-LIFETIME SPECIAL MEETING

WITH THE FDA AND OUR INDUSTRY PARTNERS

EXTERNALLY-LED PATIENT-FOCUSED DRUG DEVELOPMENT (PFDD) MEETING

WEDNESDAY, JULY 18, 2018

12:00pm — 5:00pm

SALON D, E, F

YOUR VOICE AS AN AFFECTED INDIVIDUAL OR A CAREGIVER IS CRUCIAL!

Polling App and WiFi Access Code

Throughout the conference we will be using the PollEverywhere app. Please download the PollEverywhere mobile app link prior to the conference through Google Play and the AppStore (https://www.polleverywhere.com/mobile). This app will be used during the Externally-Led PFDD Meeting so all families' voices can be heard from around the world.

- WiFi access code in the conference center at the Clearwater Beach Hilton is: **POWERUP**
- Conference poll page is: **PollEv.com/powerup1**

SMALL GROUP MEETINGS, CLINICS AND CONSULTATIONS

SUNDAY, JULY 15 thru FRIDAY, JULY 20, 2018

Individual Consultations and Clinics will run from Sunday thru Friday. Please check your individual timetable for your allocated slots as you may not be eligible for, or need to attend every session noted below.

Breakaway Quiet Room (Affected individuals age 16+) (Manatee)
When sessions are not scheduled

Portraits by Amanda Clark (Starfish)
Early family arrivals scheduled on Sunday, July 15 ~ *Amanda Clark; Bryttany Clark; Carlisa Murphy*

Vitals/EMTS (Salon G)
Height, weight and blood pressure ~ *Susan Wilkins; Mike Wilkins; Donna Strain; Shelia Mann; SunStar Paramedics*

General Barth Syndrome Questions (Tarpon)
Information regarding biochemical, diagnostic, natural history, general medical or treatment strategy questions ~ *Hilary Vernon*

Genetics (Tarpon)
Information about all aspects of genetics and how family members may be affected; Collection of family pedigrees ~ *Iris Gonzalez; Rebecca McClellan*

Pill Swallowing Workshop (Marlin)
Techniques and equipment for pill swallowing – creating a personalized program in a stress-free environment.
Audience: Affected individuals age 4+ ~ *Stacey Reynolds; Consuelo Kreider*

Investigation into Clinical, Metabolic and Molecular Factors in Barth Syndrome (Ballroom G)
Six-minute walk test and grip strength ~ *Brittany DeCroes Hornby; Arianna F. Anzmann, Ryan Manuel, Hilary Vernon; Rebecca McClellan*

Longitudinal Evaluation of Cardiomyopathy and Outcome in Barth Syndrome (Coral)
Echocardiogram, EKG and questionnaire—for affected individuals who have participated in previous cardiology study led by Dr. Taylor ~ *Carolyn Taylor; Tammy Churchill*

Dysorality in Barth Syndrome (Dolphin)
Assessing the prevalence, causes and severity of eating problems in Barth syndrome ~ *Florence Mannes; Catherine Thibault; Sandrine Aliotti*

Management of Disease Impacts in Barth Syndrome
Age-specific Focus Group Sessions throughout the week to learn how individuals with Barth syndrome cope with the impacts of their disease and how they plan for the future ~ *Iyar Mazar*

Sunday evening join us for an informal gathering for all families at the pool @ 7:00pm!
Monday evening, grandparents will meet on the beach @ 7:30pm!

CHILDCARE ACTIVITIES

Wednesday, July 18, 2018 (11:30am—5:30pm)
Thursday—Friday, July 19—20, 2018 (8:30am—5:00pm)
Saturday, July 21, 2018 (8:30am—12:00pm)
(Water's Edge A, B, C)

Childcare is offered for children ages 12 and under so that parents can attend educational sessions. Children will enjoy fun, age appropriate activities. Registration for childcare will begin at 11:30am on Wednesday in Foyer D, E, F, G.

CLINICS

(Grand Ballroom Foyer - Clinic Registration Desk)

Family participation in clinical research has provided the primary source of clinical data collection since 2002. Findings from these studies have resulted in the publication of numerous peer-reviewed journal articles to educate physicians and researchers about various aspects of Barth syndrome. This year, families will have the opportunity to participate in multiple institutional review board approved research studies. Since most of the segments of these studies do not require a parent to be present, we have scheduled families into four age-specific blocks. A "Study Buddy" will escort minor children to the various sections of the research studies while parents attend small group focus sessions. Clinic check-in will be at the Clinic Registration Desk located in the Grand Ballroom Foyer.

Investigation into Clinical, Metabolic and Molecular Factors in Barth Syndrome (Ballroom G)

Principal Investigator: Hilary J. Vernon, MD, PhD — Associate Professor, McKusick-Nathans Institute of Genetic Medicine, Johns Hopkins University; Kennedy Krieger Institute, Baltimore, MD

Our team will be assessing strength (grip strength, knee extensor strength and functional strength via a sit to stand test), balance (via the Sway application) and functional exercise capacity (via the 6-Minute Walk Test (MWT) in boys and men with Barth syndrome as well as boys and men present at the conference without Barth syndrome. In addition to the physical therapy assessments, we will ask participants to complete questionnaires regarding recent physical activity and historical risk factors for cardiac arrhythmia.

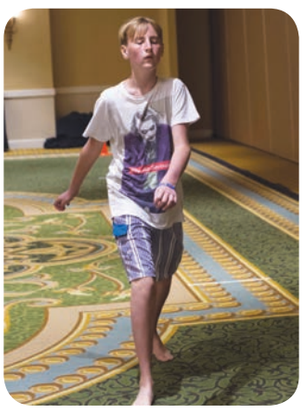
Segment of Study	Who Can Participate	Age Range	Must A Parent Be Present?
Six-minute walk test and grip strength	Affected males and male relatives	3+ years of age	No

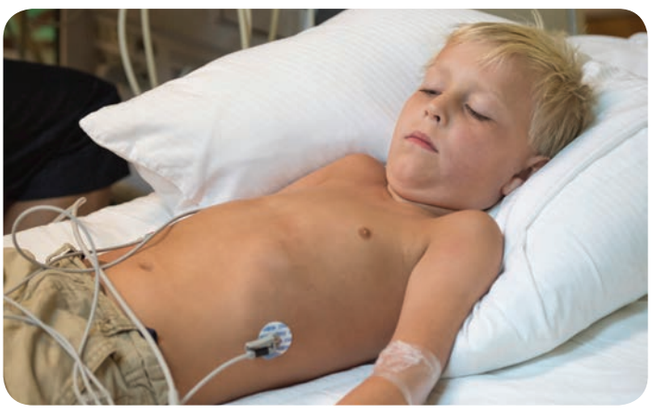
Longitudinal Evaluation of Cardiomyopathy and Outcome in Barth Syndrome (Coral)

Principal Investigator: Carolyn Taylor, MD — Associate Professor, Division of Pediatric Cardiology, The Children's Heart Program of South Carolina, Medical University of South Carolina, Charleston, SC

The purpose of this project is to describe longitudinal changes in cardiac function in boys with Barth syndrome (BTHS). This is an observational study to obtain prospective data and compare it to retrospectively obtained data in order to provide natural history data in subjects with Barth syndrome. The primary purpose of this study is to better understand the longitudinal course of cardiomyopathy in Barth syndrome by analyzing measures of cardiac function and arrhythmia over time. Results of echocardiograms and ECGs that were previously collected will be compared to those that are prospectively collected. We also aim to better understand the relationship between cardiomyopathy and clinical outcome by using data from a brief subject survey. Eligible subjects are any individual with a diagnosis of Barth syndrome who has previously participated in echocardiography studies with Dr. Taylor (Spencer). Subjects who have at least one previous qualifying echocardiogram for review but have since had a cardiac transplant will be included. Those who are currently taking an investigational research study medication will not have echo or ECG data collected but may participate in the survey portion. Exclusion criteria include those who do not wish to consent, those who have not previously participated or do not have a diagnosis of Barth syndrome. The medical survey is estimated to take about 15 minutes to complete. The questions are regarding cardiac transplantation, symptoms, arrhythmia and ICD placement, medications, activity or ability level, stroke history, family history and post-high school graduation activity.

Segment of Study	Who Can Participate
Echocardiogram, ECG, and questionnaire	Affected males of any age who have participated in previous cardiology studies led by Dr. Carolyn Taylor





(L) Pietro, Henry and William participated in research studies conducted at BSF's 2016 conference

(Photos courtesy of Tiffini [2016])

CLINICS

(Grand Ballroom Foyer - Clinic Registration Desk)

Dysorality in Barth Syndrome (Dolphin)

Principal Investigator: Florence Mannes, Chair, Association Syndrome de Barth France, Suresnes, France

Cardiology and hematology are big concerns for Barth individuals; however, many of them face eating difficulties (NG tube, G tube, picky eaters, selectivity, etc.). In this study, I would like to analyze how Barth boys eat, to find some explanation for these difficulties and, ideally, to provide families with guidelines to prevent or improve the situation.

Segment of Study	Who Can Participate	Age Range	Must A Parent Be Present?
Eating disorders etiology in BTHS	Affected males	All ages	Yes (for minor children)

Management of Disease Impacts in Barth Syndrome

Principal Investigator: Iyar Mazar, Doctoral Candidate, Boston College; Research Manager, Adelphi Values, Boston, MA

A research study is underway to gather information about individuals' experiences with goal setting and coping mechanisms related to Barth syndrome. Individuals with Barth syndrome who are 12 years of age and older who participate in select group sessions at the Barth Syndrome Foundation's 9th International Scientific, Medical & Family Conference may choose to agree to have their sessions audio-recorded as part of this research (with parental permission for individuals under age 18). Individuals and caregivers of individuals with Barth syndrome are also eligible to participate in a 60-minute audio-recorded in-person interview at the conference or following the conference via telephone. During the group sessions and interviews, people will be asked to talk about ways they set goals and cope with the impacts of Barth syndrome. The information shared may help others better understand the experience of living with health conditions such as Barth syndrome. The study is entirely voluntary, and none of the sessions will be audio-recorded without the consent of all participating individuals. Individuals can choose to participate in an audio-recorded group session, an individual interview, neither, or both, depending on their preference. Individuals will not receive compensation for participating in this study and no medications will be given as part of this study.

Individuals with Barth syndrome who are 12 years of age and older who participate in select group sessions may choose to agree to have their sessions audio-recorded as part of this research (with parental permission for individuals under age 18). Individuals and caregivers of individuals with Barth syndrome are also eligible to participate in a 60-minute audio-recorded in-person interview at the conference or following the conference via telephone.

Segment of Study	Who Can Participate	Age Range	Must A Parent Be Present?
Disease impact	Affected individuals and caregivers	Ages 12+	Yes (for minor children)

The Relationship Between Sleep and Activity Levels in Males with Barth Syndrome (Marlin)

Principal Investigator: Stacey Reynolds, PhD, OTR/L, Associate Professor, Department of Occupational Therapy, Virginia Commonwealth University, Richmond, VA

The purpose of this study is to determine the nature and degree of sleep problems in children and adults with Barth syndrome. The study will have two parts. Part A will be an online questionnaire that will ask questions about common sleep behaviors and sleep patterns. Parents will be asked to complete this questionnaire for children with Barth under the age of 18; adults with Barth syndrome will complete the survey for themselves. We are hoping for as many families as possible to complete the questionnaire which will take about 10 minutes. Part B of our study will be a pilot study using sleep and activity trackers (an Actigraph watch) to collect real-time sleep data. Boys and men with Barth syndrome will wear the trackers at home for 14 consecutive days (24hr/day) and mail the watches back to the researchers in a pre-paid envelope. We will use the data to look at the relationship between sleep and activity levels in individuals with Barth syndrome as well as examine age-related trends in sleep and activity data. For part B, we are hoping to get at least 10 individuals to participate. Interested individuals or families can contact the research team at: reynoldsse3@vcu.edu.

Segment of Study	Who Can Participate	Age	Must a Parent be Present?
Questionnaire and activity tracker	Affected individuals	Age 4+	Yes (for minor children)

SMALL GROUP MEETINGS, CLINICS & CONSULTATIONS
MONDAY JULY 16, 2018
8:30am–5:00pm

8:30am—2:30pm: Registration (East Foyer)
Pick up badges, t-shirts; Drop items for family "goody bags"; Conference app download and instruction ~ *Lois Galbraith; Sharon Olson; Susan Hone*

9:00am—5:00pm: Portraits by Amanda Clark (Starfish)
Amanda Clark; Bryttany Clark; Carlisa Murphy

9:00am—10:00am: First Time Attendee Orientation (Salon D, E, F)
General orientation and walkthrough meeting for new families and mentors ~ *Donna Strain*

10:00am—11:00am: Barth Syndrome Registry & Repository (For transplanted families only) (Manatee)
Full details about the registry and assistance with enrollment or updating of entries ~ *Shelley Bowen*

11:00am—12:00pm: Affected Individuals Only Discussion (Ages 18+) (Manatee)
Small group meeting with Shelley Bowen

1:00pm—2:30pm: Heart Transplant Meeting (Salon A, B, C)
Informal get-together of families with heart transplanted individuals ~ *Nicole Derusha-Mackey*

1:00pm—2:00pm: Barth Syndrome Registry & Repository (For non-transplanted families only) (Manatee)
Full details about the registry and assistance with enrollment or updating of entries ~ *Shelley Bowen*

2:30pm—4:30pm: Welcome Event (Salon D, E, F)
Overview of conference, family introductions, conference app explanation ~ *Kevin Woodward*

4:30pm—5:30pm: Consent and Assent Signing (All Families & Research Leaders) (Salon D, E, F)
Consents: All adult affected individuals 18 and older, and parents of all affected individuals under 18 years of age; Assents: Affected individuals 12 to 18 years of age. ~ *Shelley Bowen*

5:30pm—6:30pm: Facilitators Meeting (Meeting 1 of 4) (Salon D, E, F)
A chance for all those involved in delivering or facilitating clinics and educational sessions to meet each other in an informal setting. An opportunity to find out who is doing what during the week, fine tune your upcoming sessions with your colleagues, network and generally catch up. ~ *Michaela Damin*

Join us for an informal gathering for all families at the pool @ 7:00pm!
Grandparents will meet on the beach at 7:30pm!

TUESDAY, JULY 17, 2018

8:30am—5:00pm: Registration (East Foyer)
Pick up badges, t-shirts; Conference app download and instruction ~ *Lois Galbraith; Sharon Olson; Susan Hone*

9:00am—5:00pm Portraits by Amanda Clark (Starfish)
Amanda Clark; Bryttany Clark; Carlisa Murphy

8:30am—9:30am: Barth Essential Information (Session One) (Salon A, B, C)
For newer parents and caregivers of individuals with Barth syndrome or those wishing for a refresher course. ~ *Rebecca McClellan; Colin Steward; Carolyn Taylor; Hilary Vernon; Nicole Derusha-Mackey; Michaela Damin*

9:30am—10:30am: Barth Essential Information (Session Two) (Salon A, B, C)
For affected individuals 16+ (no parents) ~ *Rebecca McClellan; Colin Steward; Carolyn Taylor; Hilary Vernon; B.J. Develle*

An informal and interactive session to help prepare attendees for more complex information presented later in the week. Topics include: What causes Barth syndrome; Basic cardiology and what to look for in clinic letters; Heart transplant — basic facts and things to consider; Basic hematology — blood count, what to look for- what the report says, looking at the person; General medical and treatment strategies; Feeding issues; General Q&A.

10:30am—12:00pm: Life as a Carrier or Potential Carrier (Salon A, B, C)
This small group is open to carriers and potential carriers 15 years of age and older. This group will focus on the many and varied issues that present during the lifetime of a woman who is or may be a carrier for Barth syndrome. ~ *Rebecca L. McClellan; Susan McCormack; Brandi Dague; Jessica Wright*

11:30am—1:00pm: Facilitator Meeting Working Lunch (Meeting 2 of 4) (Water's Edge)
A drop-in working lunch for facilitators to meet up again, this time in smaller groups of people who are working towards common or similar goals. ~ *Michaela Damin*

SMALL GROUP MEETINGS, CLINICS & CONSULTATIONS
TUESDAY, JULY 17, 2018 *(cont'd)*

Focus Groups: These small group meetings are split into specific age groups. Explore common challenges and share strategies for success to help with all aspects of day-to-day life with Barth syndrome. Each meeting is open to the whole family (we suggest you make alternative arrangements for children 10 and under though). If your child with Barth syndrome is 10 years or younger, you will have one session, without your child/ren present. If your child with Barth syndrome is 11 years or older, there will be an initial focus group from 1:.00pm–2:00pm which parents/caregivers and their child(ren) attend together, followed by a separate breakaway session for parents/caregivers only immediately afterward from 2:00pm–3:00pm.

1:00pm—2:00pm: Affected Individuals and Parents/Caregivers
Choose from:
1. Middle School (ages 11—14) ~ *Amer Randell* (Salon A)
2. High School (ages 15—17) ~ *Jonathan Stokes* (Salon C)
3. Post High School (ages 18+) ~ *Iyar Mazar* (Salon B)

2:00pm—3:00pm: Parents/Caregivers Only
Choose from:
1. Pre-School (ages 0—4) ~ *Emily Love* (Salon C)
2. Elementary School (ages 5—10) ~ *Ashlee Espensen* (Tarpon)
3. Middle School (ages 11—14) ~ *Amer Randell* (Salon A)
4. High School (ages 15—17) ~ *Jonathan Stokes* (Manatee)
5. Post High School (age 18+) ~ *Iyar Mazar* (Salon B)

3:30pm—5:30pm: Patient-Focused Drug Development Meeting Dress Rehearsal (Salon D, E, F)
For those who are involved in presentations during the Wednesday PFDD meeting only ~ *Emily Milligan; James Valentine*

5:30pm—7:00pm: Translating Science into Medicine or How Does One Find/Discover a Treatment for Barth Syndrome
(Salon A, B, C)
Affected individuals only (age: approximately age 16+)
A simplified summary of potential therapies for Barth syndrome. (Pizza will be served) ~ *Matthew Toth; W. Todd Cade*

Join us for informal social for everyone at the pool at 7:00pm!

WEDNESDAY, JULY 18, 2018

8:30am—5:00pm: Registration (East Foyer)
Pick up badges, t-shirts; Conference app download and instruction ~ *Lois Galbraith; Sharon Olson; Susan Hone*

8:00am—9:00am: Round Table Transplant Discussion (Tarpon)
An informal dialogue between doctors and BTHS transplant recipients and relatives to examine heart transplant issues ~ *Nicole Derusha-Mackey; Carolyn Taylor; Brian Feingold*

8:15am—9:00am: Facilitator Breakaway (Meeting 3 of 4) (Manatee)
~ *Michaela Damin*

9:00am—10:00am: Post-Test Carrier Discussion (Salon A, B, C)
This small group will focus on exploring the impact of learning that you are a carrier for Barth syndrome. How might this impact relationships, future plans, reproductive options, etc. ~ *Rebecca L. McClellan; Susan McCormack; Brandi Dague; Jessica Wright*

10:00am—11:00am: Childcare registration with Corporate Kids Events (ages 0—12) (Foyer D, E, F, G)

10:00am—11:00am: Men's Session (Salon A, B, C)
Small group session to help partners, husbands and fathers support carriers and potential carriers in their family ~ *Rebecca L. McClellan*

11:00am—12:00pm: Luncheon for everyone (Grand Ballroom Foyer)

12:00pm—12:30pm: Youth Check In / Hit the Road (ages 13—18) (Coral)

12:00pm—5:00pm: Externally-Led Patient-Focused Drug Development Meeting (Salon D, E, F)
Emily Milligan; James Valentine
See page 2 for more information.

12:30pm—3:30pm: Little Toot Dolphin Excursion (ages 13—18) ~ *B.J. Develle and other volunteers*

3:30pm—4:30pm: Ice Cream Social (ages 13—18) ~ *B.J. Develle and other volunteers*

4:30pm—5:00pm: Barth Rocks (ages 13—18) (Coral) ~ *B.J. Develle and other volunteers*

5:30pm: Youth Pick Up (Coral) ~ *B.J. Develle and other volunteers*

7:00pm: Post PFDD Meeting Beach Breather (dinner on the deck for everyone) (Sandpiper Deck)

Conference at a Glance: Science/Medicine Sessions

Thursday, July 19	Friday, July 20	Saturday, July 21
7:30am—8:15am **Breakfast** (Salon D, E, F)	**7:30am—8:15am** **Breakfast** (Salon D, E, F)	**8:30am—11:30am** **Scientific and Medical Advisory Board Breakfast & Meeting** *(by invitation)* (Salon G)
8:00am—11:50am **Pathomechanism(s) of BTHS** (Salon G) *Arnold W. Strauss, MD, Chair* • William T. Pu, MD • Christoph Maack, MD • Douglas Strathdee, PhD • Christian Reynolds, PhD • Zujie Liu, PhD • Laura Cole, PhD • Jan Dudek, PhD	**8:30am—11:50am** **Clinical Characteristics of BTHS** (Salon G) *Grant M. Hatch, PhD, Chair* • Carolyn Taylor, MD • Brittany DeCroes Hornby, PT, DPT, PCS • W. Todd Cade, PT, PhD • Anthony Aiudi, PharmD • 2 Poster Presenters • Colin Steward, PhD, FRCP, FRCPCH	
12:00—1:00pm **Luncheon** (Salon D, E, F)	**12:00pm—2:00pm** **Luncheon** (Salon D, E, F)	**12:00pm—2:00pm** **Luncheon & Finale** (Salon D, E, F)
1:30pm—4:50pm **Potential Therapies for BTHS** (Salon D, E, F) *W. Todd Cade, PT, PhD, Chair* • Christina Pacak, PhD • Hilary J. Vernon, MD, PhD • Colin Phoon, MPhil, MD • Michael Chin, MD, PhD • Riekelt Houtkooper, PhD • Hazel Szeto, MD, PhD	**2:00pm—5:50pm** **Cardiolipin and BTHS** (Salon G) *Michael Schlame, MD, Chair* • Ulrich Brandt, PhD • Miriam L. Greenberg, PhD • Two Poster Presenters • Yuguang Shi, PhD • Nathan Alder, PhD • Markus Keller, PhD	
Dinner on your own	**Dinner on your own**	
5:30pm—7:30pm **Poster Session with Hors d'oeuvres** (Grand Ballroom Foyer) *Hilary J. Vernon, MD, PhD, Chair*	**7:00pm—11:00pm** **Friday Night Social with Hors d'oeuvres** (Salon D, E, F)	
8:00pm—10:00pm **Luminaries on the Beach**		

Conference at a Glance: Family Sessions and **Youth Sessions** *(Pick up & drop off in Coral)*

Thursday, July 19	
Family Sessions	**Youth**
7:30am—8:15am: Breakfast (Salon D, E, F)	**7:30am—8:15am: Breakfast** (Salon D, E, F)
8:45am—10:15am: Cardiac Aspects of BTHS (Salon A, B, C) • Carolyn Taylor, MD • Brian Feingold, MD, MS • John Lynn Jefferies, MD, MPH, FAAP, FACC • BTHS Transplant Experiences: Nicole Derusha-Mackey; Andrew Buddemeyer; Tracy Torbert	8:30am—9:00am: **Check In/Hit the Road** (Coral) 9:00am—11:45am: **Build A Boat** (Deck)
10:15am—10:30am: Break	
10:30am—12:00pm: Clinical Research Updates and Their Impact on Treatment (Salon A, B, C) • Kate McCurdy • W. Todd Cade, PT, PhD • Brittany DeCroes Hornby, PT, DPT, PCS • Yoonjeong Lim, PhD, OTR/L	12:00pm: **Youth Pick Up** (Coral)
12:00pm—1:30pm: Luncheon (Salon D, E, F)	**12:00pm—1:30pm: Luncheon** (Salon D, E, F)
1:30pm—4:50pm: Potential Therapies for BTHS (Salon D, E, F) The Family Session is joined with the Science & Medicine Session on Potential Therapies for BTHS *(*See page 8 and 10)*	1:30pm—3:00pm: **Team Building (outdoor water activities)** (Deck) 3:00pm—4:30pm: **Zen Garden Project** (Deck) 4:30pm—4:45pm: **Wrap Up** (Coral) 5:00pm: **Youth Pick Up** (Coral)
Dinner on Your Own	**Dinner on Your Own**
6:30pm—7:30pm: Poster Session (Grand Ballroom Foyer)	
8:00pm—10:00pm: Luminaries on the Beach	**8:00pm—10:00pm: Luminaries on the Beach**
Friday, July 20	
7:30am—8:15am: Breakfast (Salon D, E, F)	**7:30am—8:15am: Breakfast** (Salon D, E, F)
8:45am—9:50am: Neutropenia and BTHS (Salon A, B, C) • Jean Donadieu, MD, PhD • Audrey Anna Bolyard, RN, BS • Colin Steward, PhD, FRCP, FRCPCH	8:30am—9:00am: **Check in/Hit the Road** (Coral) 9:00am—11:00am: **Fishing on the Pier** (Siblings) 9:00am—10:00am: **High School Transitions: Session 2** (Tarpon) 9:00am—10:00am: **Middle School Transitions: Session 2** (Manatee)
9:50am—10:00am: Break	
10:00am—12:00pm: Genetics and Carriers (Salon A, B, C) • Colin Steward, PhD, FRCP, FRCPCH • Rebecca L. McClellan, MGC, CGC • Cynthia James, ScM, PhD	10:00am—11:00am: **What Will it Be Like When I Grow Up** (Water's Edge) 11:00am—12:00pm: **Paracord Jewelry** (Coral) 11:00am—12:00pm: **Post High School Transitions (age 18+)** (Tarpon)
12:00pm—1:00pm: Luncheon (Salon D, E, F)	**12:00pm-1:00pm: Lunch without the parents** (Deck)
1:15pm—4:00pm: Living with BTHS (Salon A, B, C) • Matthew Toth • Stacey Reynolds, PhD, OTR/L • Anthony Aiudi, PharmD • Nicol Clayton, Advanced Specialist Paediatric Dietician • Iyar Mazar, Doctoral Candidate	1:30pm—2:00pm: **Youth Photos** (Deck) 2:00pm—3:30pm: **Photo Scavenger Hunt** (Coral) 2:00pm—2:30pm: **Carrier Pre-Test (15 +)** (Tarpon) 2:30pm—3:00pm: **Genetics (all youth ages 8-12)** (Tarpon) 3:00pm—3:30pm: **Genetics (all youth ages 13-18)** (Tarpon)
4:15pm—4:45pm: Facilitator Wrap Up (Manatee) ~ *Michaela Damin*	4:00pm: **Youth Pick-Up** (Coral)
Dinner on your own	**Dinner on your own**
7:00pm—11:00pm: Friday Night Social (Salon D, E, F)	**7:00pm—11:00pm: Friday Night Social** (Salon D, E, F)
Saturday, July 21	
10:00am—12:00pm: Growing Funds and Friends (Salon A, B, C) • **Outreaches** ~ *John Wilkins* • **Growing Funds and Friends on a Global Scale** ~ *Megan Branagh; Nicole Derusha-Mackey; Steve McCurdy*	9:30am—10:00am: **Check in/Hit the Road** (Coral) 10:00am—10:30am: **Final Thoughts for the Finale** (Coral) 10:30am—11:30am: **Water Balloon Catch** (Deck) 11:30pm: **Youth Pick-Up** (Coral)
12:00pm—2:00pm: Luncheon & Finale (Salon D, E, F)	**12:00pm—2:00pm: Luncheon & Finale** (Salon D, E, F)

SCIENCE & MEDICINE SESSIONS
Thursday, July 19, 2018

The Science & Medicine sessions are designed for doctors and scientists involved in the many aspects of Barth syndrome to discuss the latest underlying scientific developments and clinical insights. They are unique experiences that encourage collaboration and accelerate advances.

7:30am—8:15am: Breakfast for all conference attendees (Salon D, E, F)

8:00am—11:50am: PATHOMECHANISM(S) OF BARTH SYNDROME (Salon G)
Chair: Arnold W. Strauss, MD, Cincinnati Children's Research Foundation, Cincinnati, OH

Introduction — *Benjamin Mann*

Genetically modified mouse models of Barth syndrome ~ *William T. Pu, MD, Boston Children´s Hospital, Boston, MA*

Defective mitochondrial Ca2 + uptake in heart, but not skeletal muscle in Barth syndrome ~ *Christoph Maack, MD, University Clinic Würzburg, Würzburg, Germany*

Activation of the mitochondrial stress response underlies a specific heart phenotype in Barth syndrome ~ *Douglas Strathdee, PhD, Cancer Research UK Beatson Institute, Glasgow, United Kingdom*

Nicotinamide replacement improves mitochondrial function in preclinical models of Barth syndrome ~ *Christian Reynolds, PhD, Wayne State University, Detroit, MI*

10:05am—10:20am: Break

Increased ROS-mediated CaMKII activation contributes to Barth syndrome iPS-CMs pathogenesis ~ *Xujie Liu, PhD, Boston Children's Hospital, Boston, MA*

Altered islet function may promote a lean phenotype in *tafazzin* deficient mice ~ *Laura Cole, PhD, University of Manitoba, Winnipeg, Canada*

Defective mitochondrial cardiolipin remodeling affects HIF-1 medicated response to hypoxia ~ *Jan Dudek, PhD, University Medical Clinic Würzburg, Würzburg, Germany*

12:00pm—1:00pm: Luncheon *(Salon D, E, F)*

1:00pm—1:30pm: **Scientific & Medical Attendees Group Photo** (Sandpiper Deck)

1:30pm—4:50pm: POTENTIAL THERAPIES FOR BARTH SYNDROME (Salon D, E, F)
Chair: W. Todd Cade, PT, PhD, Washington University School of Medicine, St. Louis, MO

Introduction — *Aldo Dunn*

Gene therapy vector optimization and testing for Barth syndrome ~ *Christina Pacak, PhD, University of Florida, Gainesville, FL*

Elamipretide (MTP-131) in subjects with genetically confirmed Barth syndrome (TAZPOWER): A phase 2 randomized, double-blind, placebo-controlled crossover trial followed by an open-label treatment extension ~ *Hilary J. Vernon, MD, PhD, Johns Hopkins University, Baltimore, MD*

Evaluating antioxidant therapies in a *tafazzin*-knockdown mouse model of Barth syndrome ~ *Colin Phoon, MPhil, MD, New York University School of Medicine, New York, NY*

3:35pm—3:50pm: Break

Identification of novel mitochondrial targeting peptides in *tafazzin* and long-term efficacy of enzyme replacement therapy in a mouse model of Barth syndrome ~ *Michael Chin, MD, PhD, Tufts Medical Center, Boston, MA*

Cross-species omics integration identifies new potential treatments for Barth syndrome ~ *Riekelt Houtkooper, PhD, Academic Medical Center, Amsterdam, The Netherlands*

Can elamipretide, the first cardiolipin-protective compound, benefit Barth syndrome patients? ~ *Hazel Szeto, MD, PhD, Social Profit Network, Menlo Park, CA*

Dinner on your own

5:30pm—7:30pm: POSTER SESSION with Hors D'ouerves (Grand Ballroom Foyer)
(5:30pm—7:30pm: Science & Medicine attendees / 6:30pm—7:30pm: Families invited)
Chair: Hilary J. Vernon, MD, PhD, Johns Hopkins University, Baltimore, MD

8:00pm—10:00pm: LUMINARIES ON THE BEACH
Luminaries will light the beach to honor those who are living with Barth syndrome and to remember those who have passed away. (Group photo - all conference attendees)

FAMILY SESSIONS
Thursday, July 19, 2018

The Family sessions are designed to provide the latest information to family members 16 years of age and older. Note: Affected individuals aged 16+ may choose to attend any/all individual sessions within the Youth, Family, and Science & Medicine sessions, according to individual preferences. Note: Family and Science & Medicine presentations may occasionally include more "mature" content.

7:30am—8:15am: Breakfast for all conference attendees (Salon D, E, F)

8:45am—10:15am: CARDIAC ASPECTS OF BARTH SYNDROME (Salon A, B, C)
Chair: Christiane Hope

Carolyn Taylor, MD, The Children's Heart Program of South Carolina, Medical University of South Carolina, Charleston, SC

Brian Feingold, MD, MS, University of Pittsburgh, Pittsburgh, PA

John Lynn Jefferies, MD, MPH, FAAP, FACC, Cincinnati Children's Hospital Medical Center, Cincinnati, OH

Nicole Derusha-Mackey; Andrew Buddemeyer; Tracy Torbert

Discussions/Questions & Answers

10:15am—10:30am: Break

10:30am—12:00pm: CLINICAL RESEARCH UPDATES AND THEIR IMPACT ON TREATMENT (Salon A, B, C)
Chair: Michaela Damin

Clinical trials — An overview ~ *Kate McCurdy, BSF Scientific & Medical Advisory Board, Emerita*

Characterization of the metabolic phenotype in individuals with Barth syndrome with and without cardiac transplantation ~ *W. Todd Cade, PT, PhD, Washington University School of Medicine, St. Louis, MO*

Functional exercise capacity, strength, balance and motion reaction time in Barth syndrome: Outcomes from the 2016 Barth Syndrome Foundation Scientific, Medical & Family Conference ~ *Brittany DeCroes Hornby, PT, DPT, PCS, Kennedy Krieger Institute, Baltimore, MD*

Impact on the family and quality of life: Findings from the 2014 and 2016 surveys ~ *Yoonjeong Lim, PhD, OTR/L, Georgia State University, Atlanta, GA*

Discussions/Questions & Answers

12:00pm—1:30pm: Luncheon for all conference attendees (Salon D, E, F)

1:30pm—4:50pm: POTENTIAL THERAPIES FOR BARTH SYNDROME (Salon D, E, F)
Chair: W. Todd Cade, PT, PhD, Washington University School of Medicine, St. Louis, MO

Introduction — *Aldo Dunn*

Gene therapy vector optimization and testing for Barth syndrome ~ *Christina Pacak, PhD, University of Florida, Gainesville, FL*

Elamipretide (MTP-131) in subjects with genetically confirmed Barth syndrome (TAZPOWER): A phase 2 randomized, double-blind, placebo-controlled crossover trial followed by an open-label treatment extension ~ *Hilary J. Vernon, MD, PhD, Johns Hopkins University, Baltimore, MD*

Evaluating antioxidant therapies in a *tafazzin*-knockdown mouse model of Barth syndrome ~ *Colin Phoon, MPhil, MD, New York University School of Medicine, New York, NY*

3:35pm—3:50pm: Break

Identification of novel mitochondrial targeting peptides in *tafazzin* and long-term efficacy of enzyme replacement therapy in a mouse model of Barth syndrome ~ *Michael Chin, MD, PhD, Tufts Medical Center, Boston, MA*

Cross-species omics integration identifies new potential treatments for Barth syndrome ~ *Riekelt Houtkooper, PhD, Academic Medical Center, Amsterdam, The Netherlands*

Can elamipretide, the first cardiolipin-protective compound, benefit Barth syndrome patients? ~ *Hazel Szeto, MD, PhD, Social Profit Network, Menlo Park, CA*

6:30pm—7:30pm: POSTER SESSION (Grand Ballroom Foyer)
Families invited to attend Poster Session

8:00pm—10:00pm: LUMINARIES ON THE BEACH
Luminaries will light the beach to honor those who are living with Barth syndrome and to remember those who have passed away. (Group photo - all conference attendees)

YOUTH SESSIONS
Ages 13—17
July 19—21, 2018
(Coral for drop off and pick up)

Join your fellow youth ages 13 to 18 for fun and fellowship while learning about the issues that concern young people affected by Barth syndrome. B.J. Develle and other volunteers will be the facilitators of all group activities, unless otherwise noted below.

Thursday, July 19, 2018

7:30am—8:15am: **Breakfast** for all conference attendees (Salon D, E, F)

8:30am—9:00am: **Check in / Hit the Road** (Coral)

9:00am—11:45am: **Build a Boat** (Deck)

12:00pm: **Youth Pick Up** (Coral)

12:30pm—1:30pm: **Luncheon** for all conference attendees (Salon D, E, F)

1:30pm—3:00pm: **Team Building** (outdoor water activities)

3:00pm—4:30pm: **Zen Garden Project** (Deck)

4:30pm—4:45pm: **Wrap Up** (Coral)

5:00pm: **Youth Pick Up** (Coral)

Dinner on your own

8:00pm—10:00pm: **Luminaries on the Beach**

Friday, July 20, 2018

7:30am—8:15am: **Breakfast** for all conference attendees (Salon D, E, F)

8:30am—9:00am: **Check In / Hit the Road** (Coral)

9:00am—11:00am: **Fishing on the Pier (siblings)**

9:00am—10:00am: **High School Transitions: Session 2** (Tarpon) ~ *Jonathan Stokes, Ashlee Espensen*

9:00am—10:00am: **Middle School Transitions: Session 2** (Manatee) ~ *Amer Randell; Iyar Mazar; B.J. Develle*

10:00am—11:00am: **What Will it Be Like When I Grow Up** (Water's Edge) ~ *Ashlee Estenson; Iyar Mazar; B.J. Develle*

11:00am—12:00pm: **Post High School Transitions: Session 2** (Tarpon) ~ *Iyar Mazar; B.J. Develle*

11:00am—12:00pm: **Paracord Jewelry** (Coral)

12:00pm—1:00pm: **Luncheon** (without parents) (Deck)

1:30pm—2:00pm: **Youth Photos** (Deck)

2:00pm—3:30pm: **Photo Scavenger Hunt** (Coral)

2:00pm—2:30pm: **Carrier Pre-Test (ages 15+)** (Tarpon) ~ *Rebecca L. McClellan*

2:30pm—3:00pm: **Genetics (all youth ages 8-12)** (Tarpon) ~ *Rebecca L. McClellan*

3:00pm—3:30pm: **Genetics (all youth ages 13-18)** (Tarpon) ~ *Rebecca L. McClellan*

4:00pm: **Youth Pick Up** (Coral)

Dinner on your own

7:00pm—11:00pm: **Friday Night Social** (Salon D, E, F)

Saturday, July 21, 2018

9:30am—10:00am: **Check In / Hit the Road** (Coral)

10:00am—10:30am: **Final Thoughts for the Finale** (Deck)

10:30am—11:30am: **Water Balloon Catch**

12:00pm: **Youth Pick Up** (Coral)

12:00pm—2:00pm: **Luncheon & Finale** (Salon D, E, F)

POSTER SESSION

THURSDAY, JULY 19, 2018

(Grand Ballroom Foyer)

For 2018, we have a large collection of high quality poster presentations. Four poster presenters will be chosen to talk about their results to the entire Science and Medicine audience on Friday, July 20th. The Poster Session is a perfect opportunity to meet one-on-one with many Barth syndrome researchers, so please take advantage of this opportunity.

5:30pm—7:30pm: Physicians & Scientists	**6:30pm—7:30pm**: Families welcome

POSTER 1: **Cognitive debriefing of the Barth syndrome-symptom assessment (BTHS-SA)**
Jonathan Stokes, **Anthony Aiudi**, Iyar Mazar, Meaghan Elliott, Sarah Dillard, Sarah Ollis, Emily Love, Alan L Shields, Chad Gwaltney
Institution: Stealth BioTherapeutics, Newton, MA

POSTER 2: **Understanding downstream cellular effects of *TAZ* deficiency in a novel CRISPR edited cellular model**
Arianna F. Anzmann, Veronica F. Busa, Hilary J. Vernon
Institution: Johns Hopkins School of Medicine, Baltimore, MD

POSTER 3: **Drosophila *tafazzin* mutants have impaired exercise capacity**
Deena Damschroder, Christian Reynolds, Robert Wessells
Institution: Department of Physiology, Wayne State University School of Medicine, Detroit, MI

POSTER 4: **Exploring the sign and symptom experience of Barth syndrome in adult and adolescent populations**
Jonathan Stokes, Anthony Aiudi, Iyar Mazar, **Meaghan Elliott**, Sarah Dillard, Sarah Ollis, Emily Love, Alan L Shields, Chad Gwaltney
Institution: Stealth BioTherapeutics, Newton, MA

POSTER 5: **An unexpected lack of regulation of superoxide/H2O2 production rates in isolated heart and skeletal muscle mitochondria from a mouse model of Barth syndrome**
Renata Goncalves, Martin D. Brand, Gökhan S. Hotamisligil
Institution: Sabri Ülker Center and Department of Genetics & Complex Diseases and Harvard University T.H. Chan School of Public Health, Boston, MA

POSTER 6: **AAV-mediated *TAZ* gene replacement restores mitochondrial and cardioskeletal function in Barth syndrome**
Silveli Suzuki-Hatano, Madhurima Saha, Skylar A. Rizzo, Rachael L. Witko, Bennett J. Gosiker, Manashwi Ramanathan, Meghan S. Soustek, Michael D. Jones, Peter B. Kang, Barry J. Byrne, W. Todd Cade, Christina A. Pacak
Institution: Department of Pediatrics, University of Florida College of Medicine, Gainesville, FL

POSTER 7: **Aberrant cardiolipin metabolism is associated with cognitive deficiency and hippocampal alteration in *tafazzin* knockdown mice**
Laura K. Cole, Jin Hee Kim, Andrew A. Amoscato, Yulia Y. Tyurina, Hülya Bayır, Valerian E. Kagan, **Grant M. Hatch**, Tina M. Kauppinen
Institutions: Department of Pharmacology & Therapeutics, Rady Faculty of Health Sciences, University of Manitoba; The Children's Hospital Research Institute of Manitoba, Winnipeg; DREAM, The Children's Hospital Research Institute of Manitoba, Biochemistry & Medical Genetics, Center for Research and Treatment of Atherosclerosis, University of Manitoba, Winnipeg, Canada

POSTER 8: **How do women adapt to being a Barth syndrome carrier? Results of a mixed methodological study of psychological adjustment and reproductive options**
Cynthia A. James, Leila Jamal, Rebecca McClellan
Institution: Johns Hopkins University, Baltimore, MD

POSTER 9: **Cardiolipin deficient cells require NAD generated from the fermentation pathway**
Jiajia Ji, Vaishnavi Raja, Christian Reynolds, Miriam L. Greenberg
Institution: Wayne State University, Detroit, MI

POSTER 10: **Cardiolipin is required for optimal activation of pyruvate dehydrogenase, synthesis of acetyl-CoA, and TCA cycle function**
Yiran Li, Wenjia Lou, Vaishnavi Raja, Wenxi Yu, Christian Reynolds, Miriam L. Greenberg
Institution: Wayne State University, Detroit, MI

POSTER 11: **Raising children with Barth syndrome: Impact on the family**
Yoonjeong Lim
Institution: Georgia State University, Atlanta, GA

POSTER 12: **Assessing olfactory functions in patients with Barth syndrome**
Michele Dibattista, **Simona Lobasso**, Sebastiano Stramaglia, Angela Corcelli
Institution: Department of Basic Medical Sciences, Neuroscience and Sense Organs, University of Bari Aldo Moro, Bari, Italy

POSTER SESSION
THURSDAY, JULY 19, 2018

POSTER 13: **A novel role for cardiolipin remodeling in mitigating the effects of cardiolipin peroxidation**
Wenjia Lou, Hsiu-Chi Ting, Christian A. Reynolds, Yiran Li, Yulia Y. Tyurina, Vladimir A. Tyurin, Wenxi Yu, Zhuqing Liang, Detcho A. Stoyanovsky, Tamil S. Anthonymuthu, Joel S. Greenberger, Hülya Bayir, Valerian E. Kagan, Miriam L. Greenberg
Institution: Department of Biological Sciences, Wayne State University, Detroit, MI

POSTER 14: **Management of disease impacts and goal setting in rare, severe, pediatric health conditions**
Iyar Mazar
Institution: Boston College, Newton, MA

POSTER 15: **Legitimization of chronic illness at the intersection of severity, visibility, and control over symptoms: A case study of Barth syndrome**
Iyar Mazar
Institution: Boston College, Newton, MA

POSTER 16: **Studying Barth syndrome's pathomechanism using high throughput screens in yeast**
Diana Antunes, Nofar Harpaz, Maya Schuldiner, **Doron Rapaport**
Institution: Interfaculty Institute of Biochemistry, University of Tübingen, Tübingen, Germany

POSTER 17: **An appraisal of the *tafazzin* knockdown mouse model of Barth syndrome: What have we learned?**
Mindong Ren, Michael Schlame, Yang Xu, Colin Phoon
Institution: New York University School of Medicine, New York, NY

POSTER 18: **A multi-faceted approach to enhance pill-swallowing ability in children and adults with Barth syndrome**
Stacey E. Reynolds
Institution: Virginia Commonwealth University, Richmond, VA

POSTER 19: **Dietary intake and hedonic preferences for sodium in Barth syndrome**
Stacey E. Reynolds
Institution: Virginia Commonwealth University, Richmond, VA

POSTER 20: **AAV9-*TAZ* gene replacement ameliorates cardiac TMT proteomic profiles in a mouse model of Barth syndrome**
Madhurima Saha, Silveli Suzuki-Hatano, Meghan S. Soustek, Peter B. Kang, Barry J. Byrne, W. Todd Cade, Christina A. Pacak
Institution: Department of Pediatrics, University of Florida College of Medicine, Gainesville, FL

POSTER 21: **Unraveling new potential therapeutic targets in iPS-CM model of Barth syndrome**
Erica Fatica, Rohan Shah, **Yana Sandlers**
Institution: Cleveland State University, Cleveland, OH

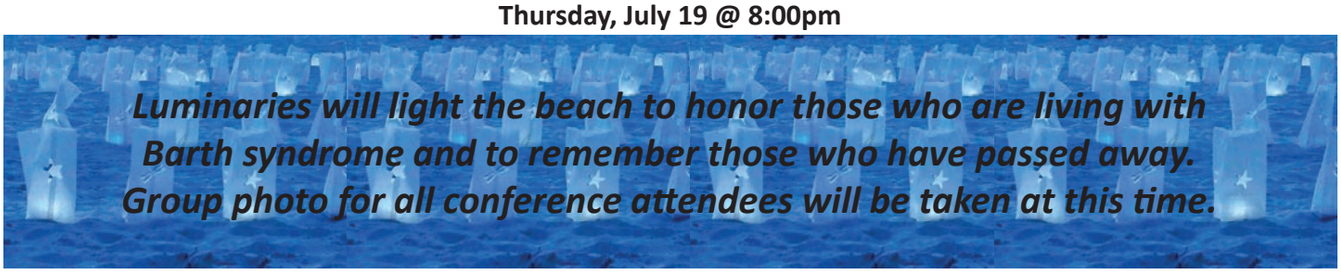
POSTER 22: **Increased anaerobic metabolism during exercise in Barth syndrome may result from augmented liver glycogenolysis**
George G. Schweitzer, W. Todd Cade, Brian N. Finck
Institution: Washington University in St. Louis, School of Medicine, St. Louis, MO

POSTER 23: **Cardiolipin controls oxidative phosphorylation through lactate signaling**
Genevieve C. Sparagna, Hailey L. Chapman, Valerie L. Warkins, Elisabeth K. Phillips, Anastacia M. Garcia, Kathryn C. Chatfield, Iñigo San-Millán
Institution: University of Colorado, Aurora, CO

POSTER 24: **Understanding the life experience of Barth syndrome from the perspective of older individuals**
Jonathan Stokes, Iyar Mazar, Sarah Ollis, Emily Love, Ashlee Espensen, Alan L. Shield
Institution: Adelphi Values, Boston, MA

POSTER 25: **Neutropenia clue in bone marrow of *TAZ* deficient mouse model**
Yang Xu, Mindong Ren, Colin Phoon, Michael Schlame
Institution: New York University School of Medicine, New York, NY

Luminaries
Thursday, July 19 @ 8:00pm



SCIENCE & MEDICINE SESSIONS
Friday, July 20, 2018

7:30am—8:15am: Breakfast for all conference attendees (Salon D, E, F)

8:30am—11:50am: CLINICAL CHARACTERISTICS OF BARTH SYNDROME (Salon G)
Chair: Grant M. Hatch, PhD, University of Manitoba, Winnipeg, Canada

Introduction — *Adam Elwood*

Barth syndrome: Natural history of cardiomyopathy and cardiac conduction ~ *Carolyn Taylor, MD, Medical University of South Carolina, Charleston, SC*

Functional exercise, capacity, strength, balance and motion reaction time in Barth syndrome: Outcomes from the 2016 Barth Syndrome Foundation Scientific, Medical & Family Conference ~ *Brittany DeCroes Hornby, PT, DPT, PCS, Kennedy Krieger Institute, Baltimore, MD*

Characterization of the metabolic phenotype in individuals with Barth syndrome with and without cardiac transplantation ~ *W. Todd Cade, PT, PhD, Washington University School of Medicine, St. Louis, MO*

10:05am—10:20am: Break

Summarized findings from the Barth Syndrome Registry and Repository ~ *Anthony Aiudi, PharmD, Stealth Biotherapeutics, Newton, MA*

Two Poster Presenters (15 minutes each)

Females with Barth syndrome ~ *Colin Steward, PhD, FRCP, FRCPCH, University of Bristol, United Kingdom*

12:00pm—2:00pm: Luncheon for all conference attendees (Salon D, E, F)

2:00pm—5:50pm: CARDIOLIPIN AND BARTH SYNDROME (Salon G)
Chair: Michael Schlame, MD, New York University School of Medicine, New York, NY

Introduction — *Travis Gordon*

The composition and dynamics of the mitochondrial complexome ~ *Ulrich Brandt, PhD, Radboud University Medical Center, Nijmegen, The Netherlands*

Mechanisms underlying TCA cycle defects in *tafazzin* deficient Cells: Potential new targets for treatment of Barth syndrome ~ *Miriam L. Greenberg, PhD, Wayne State University, Detroit, MI*

Two Poster Presenters (15 minutes each)

3:35pm—3:50pm: Break

Targeting ALCAT1 for the treatment of Barth syndrome ~ *Yuguang Shi, PhD, University of Texas Health Sciences Center, San Antonio, TX*

Biophysical approaches toward understanding the molecular mechanism of action of SS-31 (elamipretide) ~ *Nathan Alder, PhD, University of Connecticut, Storrs, CT*

The structural molecular diversity of cardiolipins ~ *Markus Keller, PhD, Medical University Innsbruck, Innsbruck, Austria*

Conference Wrap-up

Dinner on your own

7:00pm—11:00pm: FRIDAY NIGHT SOCIAL (Salon D, E, F)
Join us for a video game themed evening: socializing, music, dancing, appetizers, and cash bar. All conference attendees are invited and encouraged to come!

Saturday, July 21, 2018

8:30am—11:30am: SCIENTIFIC MEDICAL ADVISORY BOARD BREAKFAST & MEETING (by invitation only) (Salon G)

12:00pm—2:00pm: LUNCHEON & FINALE *(All conference attendees welcome)* (Salon D, E, F)

FAMILY SESSIONS
Friday, July 20, 2018

7:30am—8:15am: Breakfast for all conference attendees (Salon D, E, F)

8:45am—9:50am: NEUTROPENIA AND BARTH SYNDROME (Salon A, B, C)
Chair: Michaela Damin

Jean Donadieu, MD, PhD, Trousseau University Hospital, Paris, France

Audrey Anna Bolyard, RN, BS, The Severe Chronic Neutropenia International Registry, Seattle, WA

Colin Steward, PhD, FRCP, FRCPCH, University of Bristol, United Kingdom

Discussions/Questions & Answers

9:50am—10:00am: Break

10:00am—12:00pm: GENETICS AND CARRIERS (Salon A, B, C)
Chair: Christiane Hope

Females with Barth syndrome ~ *Colin Steward, PhD, FRCP, FRCPCH, University of Bristol, United Kingdom*

Overview of all genetic testing from preimplantation genetic testing to postnatal testing; Direct to consumer genetic testing kits ~ *Rebecca L. McClellan, MGC, CGC, Kennedy Krieger Institute, Baltimore, MD*

Tips for talking to your children about the genetics of Barth syndrome ~ *Rebecca L. McClellan, MGC, CGC, Kennedy Krieger Institute, Baltimore, MD; Cynthia James, ScM, PhD, Johns Hopkins University, Baltimore, MD*

Feedback from female side of Barth study ~ *Cynthia James, ScM, PhD, Johns Hopkins University, Baltimore, MD*

Discussions/Questions & Answers

12:00pm—1:00pm: Luncheon for all conference attendees (Salon D, E, F)

1:15pm—4:00pm: LIVING WITH BARTH SYNDROME (Salon A, B, C)
Chair: Shelley Bowen

Translating science into medicine ~ *Matthew Toth, PhD, BSF Science Director*

Problems with sleeping, what is normal, what problems exist, give strategies to help individuals; Global overview on general sensory issues; What can families do ~ *Stacey Reynolds PhD, OTR/L, Virginia Commonwealth University, Richmond, VA*

Summarized findings from the Barth registry interviews: Key points for moving forward ~ *Anthony Aiudi, PharmD, Stealth Biotherapeutics, Newton, MA*

Summary of nutrition in Barth syndrome ~ *Nicole Clayton, Specialist Paediatric Eating Disorders Dietician, Bristol Royal Hospital for Children, Bristol, United Kingdom*

What have we learned from our elders: Findings from the older men Barth study ~ *Iyar Mazar, Doctoral Candidate, Boston College; Research Manager, Adelphi Values, Boston, MA*

Discussions/Questions & Answers

4:15pm—4:45pm: FACILITATOR WRAP UP (For facilitators only) (Manatee)

Dinner on your own

7:00pm—11:00pm: FRIDAY NIGHT SOCIAL (Salon D, E, F)
Join us for a video game themed evening: socializing, music, dancing, appetizers, and cash bar. All conference attendees are invited and encouraged to come!

Saturday, July 21, 2018

10:00am—12:00pm: GROWING FRIENDS AND FUNDS (Salon A, B, C)
Chair: Emily Milligan

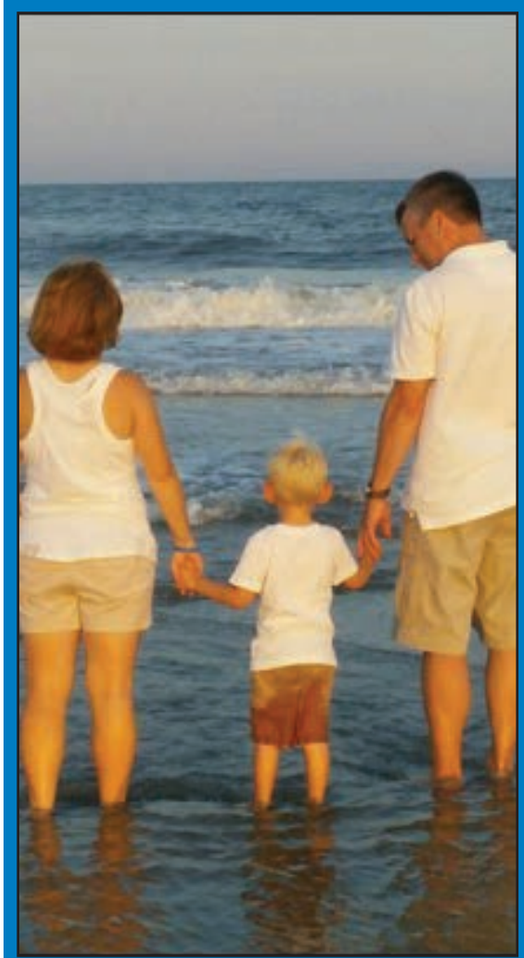
Outreaches ~ *John Wilkins*

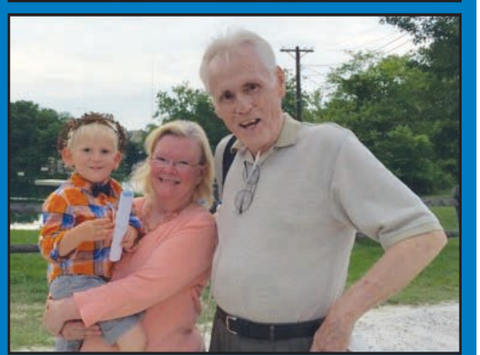
Growing Friends and Funds ~ *Megan Branagh; Nicole Derusha-Mackey; Steve McCurdy*

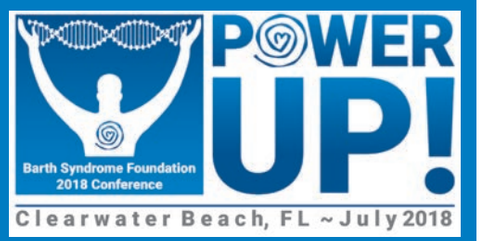
12:00pm—2:00pm: LUNCHEON & FINALE *(All conference attendees welcome)* (Salon D, E, F)

Clinics & Consultations
Science/Medicine Sessions
Sponsored by
Association Syndrome de Barth France

Friends & Family of Connor Woodward are the proud sponsors of the Family Sessions







Professional Travel Assistance for Expert Speakers

Sponsored by Dr. Gerald Cox

Thursday Breakfast

Sponsored by Peter and Susan Osnos

Thursday Refreshment Break

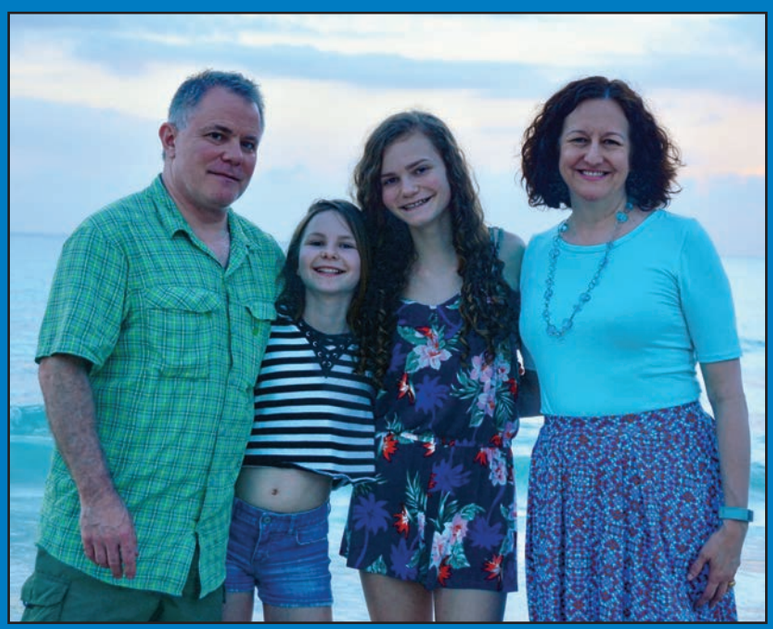
POWER UP!

Sponsored by BSF of Canada

Friday Breakfast
Sponsored by the Wald Family

Friday Refreshment Break
Sponsored by BSF of Canada

Friday Night Social
Sponsored by the McCormack-Marra Family



Lodging for Amanda Clark

Sponsored by The Holly Family

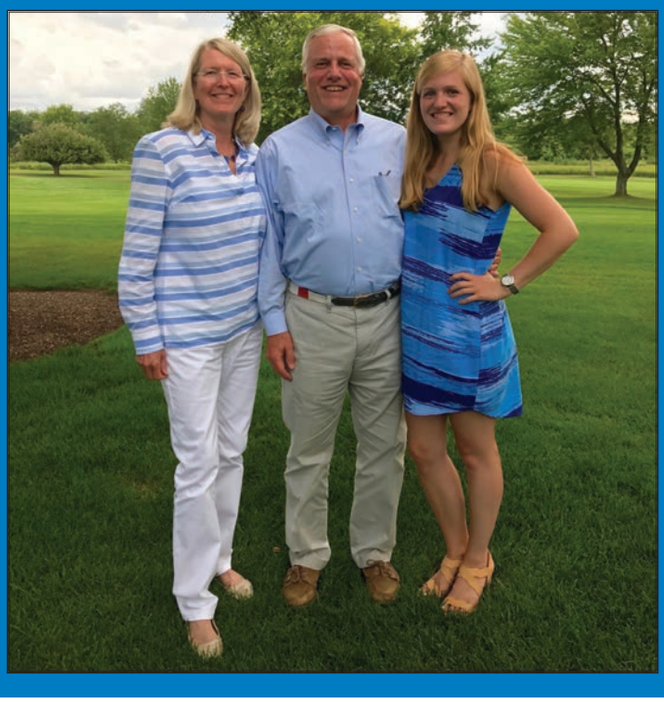
Barth Syndrome Foundation 2018 Conference

POWER UP!

Clearwater Beach, FL ~ July 2018

Saturday Luncheon

Sponsored by The McCurdy Family



Scientific & Medical Advisory Board Breakfast

Sponsored by David, Shelia, English, Benjamin Mann and Allen and Rosa Mann

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Photos from BSF's 2016 conference *(Photos courtesy of Tiffini Allen [2016])*

BARTH SYNDROME FOUNDATION

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Website: www.barthsyndrome.org

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John Wilkins, Board Member, Secretary
Kevin Woodward, Board Member, Treasurer
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Emily Milligan, Board Member, *ex-officio*

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Science Director, Barth Syndrome Foundation

Participant Biographies

Nathan N. Alder, PhD — Associate Professor, Department of Molecular and Cell Biology, University of Connecticut, Storrs, CT, USA

Dr. Alder directs a research program that is focused on understanding the structure, function, and biogenesis of mitochondrial membrane proteins and complexes, including those of the protein transport machinery and the oxidative phosphorylation system. Of particular interest to him is understanding how heritable diseases involving lipid and protein biogenesis impact structure-function relationships within mitochondrial complexes. Research in the Alder Laboratory uses a host of biochemical and biophysical techniques as well as the use and development of model membrane systems. Dr. Alder was awarded a research grant from BSF entitled "Investigation of cardiolipin-dependent respiratory complex activity and development of small molecule lipid analogs" (2013).

Since 2008, research in the Alder Lab has been funded by the National Institutes of Health (NIH), the National Science Foundation (NSF), the American Heart Association (AHA), and the Barth Syndrome Foundation (BSF). Dr. Alder holds a PhD (2002) from the University of California, and a BS (1994) from the University of Utah, and he did his post-doctoral work (2002-2008) at the Texas A&M University College of Medicine. Dr. Alder is an active member of the Biophysical Society and the American Society for Cell Biology.

Anthony Aiudi, PharmD, MBA — Director, Clinical Development, Stealth BioTherapeutics Inc., Newton, MA, USA

Dr. Aiudi is a Director of clinical development and the clinical development lead on the TAZPOWER (Barth syndrome) and MMPOWER (primary mitochondrial myopathy) programs at Stealth BioTherapeutics, Inc. As the clinical development lead, Dr. Aiudi works with regulators and experts to create clinical programs to develop therapies to treat mitochondrial dysfunction associated with genetic mitochondrial diseases.

Prior to joining Stealth BioTherapeutics Inc., Dr. Aiudi was a senior clinical scientist at Merck & Co and Cubist Pharmaceuticals, focusing on the development of novel antibiotics in adults and pediatrics. Dr. Aiuidi holds a PharmD and a MBA from the University of Rhode Island and completed his post-doctoral fellowship at Northeastern University.

Audrey Anna Bolyard, RN, BS — Clinical Manager, University of Washington, The Severe Chronic Neutropenia International Registry, Seattle, WA, USA

Ms. Bolyard manages the Severe Chronic Neutropenia International Registry (SCNIR) which she helped to establish in 1994 to monitor the clinical course, treatment, and disease outcomes in patients with severe chronic neutropenia (SCN). The Registry has the largest collection of long-term data on patients with this condition in the world. Participation in the registry benefits patients, their families and the physicians who treat them by providing the most up to date information to them on the natural history of SCN and its treatment options. Many Barth patients are members of this Registry.

Valerie ("Shelley") Bowen — Director, Family Services & Awareness, Barth Syndrome Foundation, USA

Mrs. Bowen is a founder of the Barth Syndrome Foundation (BSF) and currently serves as Director of Family Services and Physician Awareness. Both of her sons lost their lives to Barth syndrome. She believes it possible for everyone who has Barth syndrome to have an accurate diagnosis and every parent has the capacity to be transformed from a powerless bystander into an empowered advocate when given the proper tools. Mrs. Bowen has worked tirelessly to ensure that not one more child will suffer or perish from the disorder.

Ulrich Brandt, PhD — Professor, Mitochondrial Molecular Medicine, Radboud University Medical Center, Nijmegen, The Netherlands; International Faculty Professor, University Cologne, Germany; Adjunct Professor, Biochemistry, Cluster of Excellence "Macromolecular Complexes", Goethe-University, Frankfurt/Main, Germany

Dr. Brandt's research focuses on the structure and function of mitochondrial respiratory chain complexes.

Dr. Brandt graduated in Biochemistry from the Eberhard-Karls University in Tübingen, Germany and obtained his PhD from the Ludwig-Maximilians University in Munich. Between 1991 and 1993, he was a Feodor-Lynen Fellow of the Humboldt Foundation at the Dartmouth Medical School, New Hampshire, USA. In 1993, he joined the Johann-Wolfgang-Goethe University in Frankfurt am Main, Germany. In 1994, became a Docent and in 1996 a Professor of Biochemistry.

Dr. Brandt was Deputy-Director from 1997 to 2009 and Director of the Centre of Biological Chemistry from 2009-2012 at the Medical Faculty, Goethe-University, Germany. Dr Brandt served as Secretary General of the German Society for Biochemistry and Molecular Biology (GBM) from 1997 to 2012. He is a founding member of the Cluster of Excellence "Macromolecular Complexes" of the Goethe-University. Dr. Brandt has been Editor-in-Chief of the BBA since 2012, and from 2005 to 2013 he was Executive Editor of BBA Bioenergetics.

Megan Branagh — Board Member, Barth Syndrome Foundation, USA

Mrs. Branagh's middle son, Henry, is affected with Barth syndrome. In 2013, she started the Happy Heart Walk, an annual fundraising and Barth syndrome awareness event benefiting the Barth Syndrome Foundation. It is now not only an event, but a week long virtual movement that has grown in participators and funds raised over the years. Mrs. Branagh has a BS in Interior Design from Texas Christian University. She lives in the San Francisco Bay Area with her husband, John, and their three young boys.

Andrew Buddemeyer, Esq. — Bush Ross, P.A., Tampa, Florida, USA

Andrew was born in Detroit, Michigan, raised in Washington, D.C., and attended high school in Tampa, Florida. He went to Florida State University for his undergraduate degree and attended law school at the University of Florida. He currently resides in Tampa, FL and works for Bush Ross, P.A., where he practices in the area of federal and state taxation, corporate law, partnership law, wills, trusts, and estate planning.

Barry Byrne, MD, PhD — Professor and Associate Chair, Pediatrics and Molecular Genetics and Microbiology; Director, Powell Gene Therapy Center, University of Florida, Gainesville, FL, USA

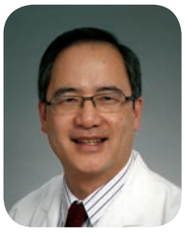
Dr. Byrne is a clinician scientist who is studying a variety of rare diseases with specific attention to developing therapies for inherited muscle disease. As a pediatric cardiologist, his focus is on conditions that lead to skeletal muscle weakness and problems in heart and respiratory function. His Pre-Clinical and Clinical Research Teams have made significant contributions to the understanding and treatment of several rare diseases. The research team has been developing new therapies using the missing cellular protein or the corrective gene to restore muscle function in Pompe and other inherited myopathies. Dr. Byrne was awarded a research grant from BSF entitled "Gene therapy in a mouse model of Barth syndrome" (2010).

Dr. Byrne obtained his BS degree from Denison University, his MD and PhD from the University of Illinois and completed his Pediatrics residency, cardiology fellowship training and post-doctoral training in Biological Chemistry at the Johns Hopkins Hospital. He joined the University of Florida in 1997 and is now the Earl and Christy Powell University Chair in Genetics.

W. Todd Cade, PT, PhD — Professor of Physical Therapy and Internal Medicine, Washington University School of Medicine, St. Louis, MO; Scientific and Medical Advisory Board, Barth Syndrome Foundation, USA

Dr. Cade's research interests include mechanisms and treatments for skeletal muscle and cardiac nutrient metabolism abnormalities in metabolic diseases such as Barth syndrome, HIV-associated metabolic syndrome, diabetes, and pathologic pregnancy. Dr. Cade was awarded the following research grants from BSF entitled "Characterization of the 'metabolic phenotype' in Barth syndrome with cardiac transplantation" (2016); "Effects of resistance exercise training on cardiac, metabolic, and muscle function and quality of life in Barth syndrome: Part II" (2014); "Effects of resistance training on cardiac, metabolic, and muscle function and quality of life in Barth syndrome" (2011); "Safety and efficacy of aerobic exercise training in Barth syndrome: A pilot study" (2009); and "Characterization of nutrient metabolism in Barth syndrome" (2008).

Dr. Cade holds a PhD in Rehabilitation Science/Applied Physiology from the University of Maryland. He also has a BSc in Kinesiological Science from the University of Maryland, an MSc in Physical Therapy from the University of Miami, FL, and is a licensed physical therapist. He completed a post-doctoral fellowship in the Division of Endocrinology, Metabolism and Lipid Research at Washington University School of Medicine.



Michael T. Chin, MD, PhD, FACC, FAHA — Associate Professor of Medicine, Harold T. Dodge-John L. Locke Chair in Cardiovascular Medicine, University of Washington, Seattle, WA, USA

Dr. Chin practices general adult cardiology at the University of Washington Medical Center. He directs a research laboratory focused on understanding the molecular biology of the cardiovascular system. His major research interests include understanding transcriptional control of cardiovascular development, the environment influences that affect the development of cardiovascular disease and developing novel therapies for cardiovascular and myopathic disorders. Dr. Chin was awarded the following research grants from BSF entitled "Enzyme replacement therapy in heart failure associated with *tafazzin* deficiency" (2014); "*Tafazzin* enzyme replacement therapy in a mouse model of Barth syndrome" (2013); and "*Tafazzin* enzyme replacement therapy for heart muscle in Barth syndrome" (2012).

Dr. Chin holds a PhD from the University of Rochester (1988), an AB from Princeton University (1983), and an MD (with Honors), from the University of Rochester (1991). He completed his Residency in Internal Medicine at the Johns Hopkins Hospital, Baltimore, MD (1991-1993).

Dr. Chin is board certified in adult cardiology, a Fellow of the American College of Cardiology, a Fellow of the American Heart Association, an elected member of the American Society for Clinical Investigation and maintains membership in the American Society for Biochemistry and Molecular Biology, the American Physiological Society and the International Society for Heart Research.

Amanda Clark — Portrait Artist, Amanda Clark Photography, Perry, FL, USA

Ms. Clark has always had a passion for photography — a gift given to her by a true love who instilled in her a love for others. To repay this blessing, she gives you, and everyone she photographs, a moment in time, warmth and an emotion that she sees through the lens. These feelings touch her heart and are captured to be yours to cherish for a lifetime. She has attended our conferences almost since the beginning and has given us all incredible gifts by photographing our boys, young men and families.

Nicol Clayton — Specialist Paediatric Eating Disorders Dietician, Bristol Royal Hospital for Children, Bristol, United Kingdom

Ms. Clayton has been a member of the multidisciplinary NHS National Barth Syndrome Service since its inception in April 2010. She manages the dietetic care of all Barth patients in the UK and provides expert advice for many children with Barth syndrome across the world. Ms. Clayton also specializes in the provision of ketogenic diet therapy, a metabolic treatment for children with intractable epilepsy.

Ms. Clayton authored a chapter on feeding in Barth syndrome in the 4th edition of Clinical Paediatric Dietetics (Wiley Blackwell) and has contributed to a number of research publications produced by the Bristol team.

Ms. Clayton's previous roles included working within a multi-disciplinary feeding disorders clinic to manage difficult behaviors around food, including food restriction and food refusal. During this time, she delivered study days to train health and medical professionals in managing children's behavioral problems with food and drink. Ms. Clayton has worked extensively with children with neurological and developmental problems and has a special interest in the nutrition of children with growth disorders.

Laura Cole, PhD — Postdoctoral Fellow, Manitoba Institute for Child Health and Department of Pharmacology and Therapeutics, Faculty of Medicine, University of Manitoba, Winnipeg, Manitoba, Canada

Dr. Cole's research interests focus on the role of cardiolipin remodeling on mitochondrial function in the heart and liver with a specific emphasis on triglyceride metabolism. Dr. Cole is currently funded by Canadian Institute for Health Research (CIHR), Integrated and Mentored Pulmonary and Cardiovascular Training (IMPACT), Manitoba Health Research Council (MHRC) and Manitoba Institute of Child Health (MICH).

Dr. Cole holds a PhD degree in lipid biochemistry from the University of Alberta, Canada.

Brandi Dague — Vice President of Sales and Customer Service, The Americas at HEICO Aerospace, Hollywood, FL, USA

Mrs. Dague is the mother of two beautiful children, Adeline (age 5) and Deacon (age 2). She turned to the Barth Syndrome Foundation when Deacon was diagnosed as an infant in 2016. Mrs. Dague, her husband, Nick, and her children currently reside in Hollywood, Florida.

Mrs. Dague is the Vice President of Sales and Customer Service for The Americas at HEICO Aerospace, the world's largest independent designer, manufacturer and distributor of jet engine and aircraft component parts. She has been with HEICO for over twelve years, which is the latest assignment of her nearly twenty year career in the aerospace industry serving in various technical sales and engineering roles. She holds a Bachelors of Engineering from Georgia Institute of Technology and a Masters of Business Administration from Georgia State University.

Michaela Damin — Founding Board Member, Barth Syndrome Trust (BST), United Kingdom

Mrs. Damin is the founding member of the Barth Syndrome Trust (BST) in the United Kingdom. She has served on the BST Board since its inception in 2003 and has also served on the BSF Board. She, together with Chris Hope and Shelley Bowen, has been involved in organising this year's Family Track of the BSF conference. She has two sons, Nick who is almost 20 years old and who has Barth syndrome, and Matthew, 16.

Nicole Derusha-Mackey — Legislative Aide, Michigan House of Representatives, Lansing, MI; Board Member, Barth Syndrome Foundation, USA

Mrs. Derusha-Mackey has been active with the Barth Syndrome Foundation since attending the BSF conference in 2008. She is the mother of two boys born with Barth syndrome: Nathaniel, who passed away at just two weeks of age in 2002, and Devin, age 13, who underwent heart transplantation as an infant. Nicole has dedicated much of her time with BSF offering her unique perspective of raising a Barth boy after heart transplant surgery. Professionally, Mrs. Derusha-Mackey has worked on numerous political campaigns, ranging from local millage renewal to US Congressional campaign. She currently works as a legislative aide in the Michigan House of Representatives. She, her wife, Sarah, and Devin live just outside of Flint, Michigan.

Bruce J. Develle, MSW — Therapist, Children's Home Society, Lutz FL; Board Member, Barth Syndrome Foundation, USA

Mr. Develle currently works for Children's Home Society with families struggling to maintain the children in their homes and prevent their entrance into the Child Welfare or Juvenile Justice systems. These families are typically struggling to manage to out of control behavior including substance use, illegal behavior and difficult mental health needs. His intense services include crisis support, targeted parenting skills training, individual and family counseling, mentoring and linking families with wraparound services as needed.

Previously, he provided services to families wanting to foster or adopt, as well as to community and foster children with histories of extreme physical and sexual abuse, grief and loss, brain injuries, mood disorders, drug exposure, suicidal and homicidal attempts and psychiatric residential placements, both in the community and within a group home he previously managed.

Mr. Develle graduated from Florida State University with a Masters in Social Work after earning Bachelor degrees in Child Development and Religion. He is currently working towards his Licensure as a Clinical Social Worker.

Mr. Develle lives outside of Tampa, Florida with his wife, Greta, and two girls. Mr. Develle has been a committed volunteer with BSF since 1998.

Jean Donadieu, MD, PhD — Pediatric Hematology Oncology and Epidemiology, Trousseau University Hospital, Paris, France

Dr. Donadieu's major interests are chronic neutropenia and the development of epidemiology for rare disorders in France and also in the European Union (EU) through participation in several EU projects. He now includes Barth syndrome as an interest as a result of the work of Association Syndrome de Barth France.

Dr. Donadieu founded the French Severe Chronic Registry in 1993, initially to evaluate the safety of GCSF in chronic neutropenia, later extended to describe the natural history of any type of syndrome associated with neutropenia, including Barth syndrome. The French SCN registry is currently working with all French hematological centers, with the genetic laboratory of Pitie salpétriére and with many other specialists involved in the care or in the evaluation or research in this field. A biobank is associated to this project in order to allow further research. Dr. Donadieu coordinates the French reference center for chronic neutropenia located in Trousseau Hospital. He also coordinates the French histiocytosis registry and French pediatric reference center.

Dr. Donadieu holds a PhD in Public Health. He trained in pediatric hemotology-oncology and immunology at Necker Hospital, Paris, France.

Jan Dudek, PhD — Junior Group Leader, University Hospital Würzburg, Department of Translational Research Comprehensive Heart Failure Center, Würzburg, Germany

Dr. Dudek's interest is mitochondria and especially how defects in mitochondrial biogenesis and function lead to human diseases. His research interest focuses on the structural and functional aspects of the respiratory chain.

Dr. Dudek holds a PhD in biochemistry from the University of Freiburg, Germany, and has held post-doctoral positions in laboratories at the Beatson Institute for Cancer Research, Glasgow, UK and the University of California, San Francisco, USA conducting research into oncogenic signalling pathways.

Ashlee Espensen, MPH — Research Associate, Adelphi Values, Boston, MA, USA

As a Research Associate, Ms. Espensen holds an interest in patient-centered outcomes research, with a particular interest in the impacts of various health conditions on maternal and child health. Additionally, Ms. Espensen has a background in social work and public health. Her experience in these fields include health equity and program evaluation research, as well as using trauma-informed care as a framework to provide physical, psychological, and emotional safety to families. This work has led Ms. Espensen to working with several of the older men with Barth syndrome in the BSF community, and she is excited to attend the BSF conference to help share their stories!

Brian Feingold, MD, MS, FAHA — Associate Professor, Pediatrics and Clinical and Translational Science, University of Pittsburgh; Medical Director, Pediatric Heart Failure and Transplantation Programs, Children's Hospital of Pittsburgh of University of Pittsburgh Medical Center, Pittsburgh, PA; Scientific and Medical Advisory Board, Barth Syndrome Foundation, USA

Dr. Feingold's research is broadly focused on pediatric heart transplantation and heart failure. He cares for several boys with Barth syndrome in Pittsburgh, both with and without heart transplantation. He has a history of funding by the National Institutes of Health KL2 Clinical Research Scholars Program, the American Heart Association, and the Enduring Hearts Foundation.

Dr. Feingold is board certified in Pediatrics and Pediatric Cardiology. He serves on multiple councils and committees of national organizations including the pediatric heart failure subcommittee of the American Heart Association and the Scientific Council on Pediatric Thoracic Transplantation and Heart Failure of the International Society for Heart and Lung Transplantation.

Iris L. Gonzalez, PhD — Research Scientist (retired), Molecular Diagnostics Laboratory, A. I. duPont Hospital for Children, Wilmington, DE

Dr. Gonzalez's scientific research and clinical interests include molecular diagnostics, research on Barth syndrome and other genetic diseases, and mutation databases. As a molecular geneticist in a diagnostic lab, Dr. Gonzalez performed the genetic test for many patients to confirm a diagnosis of Barth syndrome. In addition, her scientific interests led her to conduct research (with a BSF grant) on the mRNA associated with Barth syndrome. Dr. Gonzalez is also known by Barth families for writing a layman's guide to genetics that has been extremely valuable to BSF families and others. Dr. Gonzalez was awarded a research grant from BSF entitled "A Study of *TAZ* mRNAs in Barth syndrome individuals" (2002), and received the Barth Syndrome Foundation's Varner Award for Pioneers in Science and Medicine (2014).

Dr. Gonzalez received her PhD in Biology in Genetics (1976) and a BA in Biology (1970) from the University of Delaware. Dr. Gonzalez completed a Post-doctoral at the University of Pennsylvania (1982-1985).

Miriam L. Greenberg, PhD — Professor, Department of Biological Sciences, Wayne State University, Detroit, MI; Scientific & Medical Advisory Board, Barth Syndrome Foundation, USA

Dr. Greenberg's laboratory utilizes the powerful genetic, molecular, and cell biological tools of the yeast system and relevant mammalian cell cultures to elucidate the cellular functions of two essential lipid pathways. One project in her laboratory is to understand the role of cardiolipin in essential cellular functions and how perturbation of cardiolipin synthesis leads to Barth syndrome. The second project seeks to elucidate the essential functions of inositol phospholipids and metabolites and the cellular consequences of inositol depleting drugs. These studies have implications for understanding the pathology underlying cardiovascular disorders and the therapeutic mechanisms of action of drugs used in the treatment of psychiatric disorders. Dr. Greenberg was awarded the following research grants from BSF entitled "Cardiolipin is required for mitochondrial protein processing" (2015); "Identification of human cardiolipin phospholipases that are deleterious to *tafazzin*-deficient cells" (2013); "Cardiolipin deficiency leads to defects in the TCA cycle" (partial funding provided by Association Syndrome de Barth France — 2011); "Loss of cardiolipin leads to defective mitochondrial iron/sulfur biosynthesis and iron homeostasis" (2010); "Perturbation of mitophagy in cardiolipin mutants" (partial funding provided by Barth Syndrome Trust — 2009); "The role of *tafazzin* in mitochondrial protein import: Implications for Barth syndrome" (partial funding provided by Barth Syndrome Foundation of Canada —2008); "Perturbation of the osmotic stress response in cardiolipin deficient mutants" (2007); "The role of phosphatidylglycerol in activating protein kinase C mediated signaling" (2006); "Does copper deficiency play a role in Barth syndrome" (2005); and "*TAZ*1 gene function in yeast: A molecular model for Barth syndrome" (2002).

Dr. Greenberg holds a BA in Biology from Reed College, an MS in Microbiology from Loyola University, and a PhD in Genetics from Albert Einstein College of Medicine. She carried out her postdoctoral work in Molecular Biology at Harvard University.

Grant M. Hatch, PhD — Professor, Departments of Pharmacology & Therapeutics and Biochemistry & Medical Genetics, University of Manitoba; Canada Research Chair in Molecular Cardiolipin Metabolism; Director of the Lipid Lipoprotein and Atherosclerosis Research Group (LLARG); Director of the Centre for Research and Treatment of Atherosclerosis; Scientist, Manitoba Institute of Child Health, Winnipeg, Canada; Scientific & Medical Advisory Board, Barth Syndrome Foundation, USA

Dr. Hatch's main research interests have been focused on studying metabolism of cardiolipin — a key phospholipid involved in mitochondrial energy production. He has identified a human protein, MLCL AT-1, which may regulate the fatty acyl composition of cardiolipin. With financial support from BSF, he is investigating if expression of this protein in cardiac cells of *tafazzin* knockdown mice attenuates the loss of cardiolipin and the cardiac pathology observed in these animals. Dr. Hatch was awarded the following research grants from BSF entitled "*Tafazzin* knockdown alters hepatic lipid metabolism" (partial funding provided by Barth Syndrome Foundation of Canada — 2013); "MLCL AT-1 elevates cardiolipin and mitochondrial function in cardiac myocytes of *taz* knockdown mice" (funding provided by Barth Syndrome Foundation of Canada — 2011); "Role of human monolysocardiolipin acyltransferase in Barth syndrome" (funding provided by Barth Syndrome Foundation of Canada - 2009); "Cholesterol metabolism in Barth syndrome" (2005); and "The molecular mechanism of Barth syndrome" (2002).

Christiane Hope — Founding Board Member, Barth Syndrome Foundation of Canada, Ontario, Canada

Mrs. Hope is a founding member of the Barth Syndrome Foundation of Canada and has served on its board for 15 years. She has one surviving son with the disorder and is committed to the mission and vision of BSF.

Brittany DeCroes Hornby, PT, DPT, PCS — Senior Physical Therapist, Outpatient Physical Therapy Department, Kennedy Krieger Institute, Baltimore, MD, USA

Dr. DeCroes Hornby treats patients in addition to striving to advance research produced by the department. She serves as the physical therapist in the Multidisciplinary Barth Syndrome Clinic. Her current research interests include functional ability and quality of life in patients with Barth syndrome.

In 2015, Dr. DeCroes Hornby completed a pediatric physical therapy residency with Kennedy Krieger Institute and the University of Maryland. Following completion of the residency, she earned a specialist certification in pediatric physical therapy. Her professional interests include fitness across the lifespan, standardized clinical outcome measures, mitochondrial disorders and teaching (she currently serves as adjunct faculty of pediatrics for the University of Maryland School of Medicine Department of Physical Therapy and Rehabilitation Science).

Dr. DeCroes Hornby received a Bachelor of Science degree from James Madison University in Harrisonburg, VA where she majored in Biology and minored in Spanish. She received her Doctorate of Physical Therapy from George Washington University in Washington DC. She began working at Kennedy Krieger Institute in 2011.

Riekelt Houtkooper, MSc, PhD — Associate Professor, Laboratory of Genetic Metabolic Diseases, Academic Medical Center, Amsterdam, The Netherlands

Dr. Houtkooper's current research focuses on molecular and translational metabolism, both in the context of inborn errors of metabolism and aging.

Dr. Houtkooper received his MSc in biomedical sciences from the University of Amsterdam, The Netherlands. Following an internship at the Laboratory of Genetic Metabolic Diseases of the Academic Medical Center Amsterdam, he continued for a PhD degree in the same lab, under supervision of Professor Ronald Wanders and Dr. Fred Vaz. Dr. Houtkooper's research centered on cardiolipin metabolism, particularly in relation to the rare mitochondrial disorder Barth syndrome.

In 2009, Dr. Houtkooper joined the lab of Professor Johan Auwerx at EPFL Lausanne (Switzerland) for a postdoctoral project focusing on understanding and treating more common metabolic diseases. During this time, Dr. Houtkooper also initiated projects on the metabolic aspects of aging, leading to several high-impact papers.

In 2012 Dr. Houtkooper started his own group within the Laboratory of Genetic Metabolic Diseases, in Amsterdam receiving funding from the Netherlands Organization for Scientific Research (VENI and VIDI grants) and an ERC Starting grant from the EU. For his contribution to the metabolic field, Dr. Houtkooper received the 2014 NVBMB Prize from the Dutch Society for Biochemistry and Molecular Biology.

Cynthia James, ScM, PhD — Assistant Professor of Medicine, Center for Inherited Heart Disease, Division of Cardiology, Johns Hopkins University, Baltimore, MD, USA

Dr. James' research is focused on 1) psychosocial adaptation to inherited cardiac disease and 2) the role of genetic and environmental factors on disease expression, particularly in patients and families with arrhythmogenic right ventricular cardiomyopathy. Dr. James was awarded a research grant from BSF entitled "How do women adapt to being a Barth syndrome carrier? A mixed methodological study of psychological adjustment and reproductive options" (2014).

Dr. James holds a PhD in Human Genetics and Masters in Genetic Counseling from Johns Hopkins University and is a board-certified genetic counselor.

John Lynn Jefferies, MD, MPH, FAAP, FACC, FAHA — Heart Failure Cardiologist, Cincinnati Children's Hospital Medical Center and the Christ Hospital; Professor, Pediatric Cardiology and Adult Cardiovascular Diseases, Department of Pediatrics, University of Cincinnati; Director, Advanced Heart Failure and Cardiomyopathy, Cincinnati Children's Hospital Medical Center, Cincinnati, OH, USA; Medical Advisor, The Battelle Healthcare Colloquium; Scientific and Medical Advisory Board, Barth Syndrome Foundation, USA

Dr. Jefferies specializes in advanced heart failure and cardiomyopathy care of both children and adults. He has been the Principal Investigator or Co-Investigator for multiple clinical studies funded by the National Institutes of Health (NIH) and industry. He is the author or co-author of over 130 peer reviewed manuscripts, and he currently is the Editor of two textbooks. Dr. Jefferies was awarded a research grant from BSF entitled "Assessment of quality of life, anxiety, and depression in Barth syndrome: Expanding the scope of comprehensive care" (partial funding provided by Barth Syndrome Trust — 2014).

Dr. Jefferies completed a combined residency in Internal Medicine and Pediatrics, a fellowship in General Internal Medicine and a combined fellowship in Adult Cardiovascular Diseases and Pediatric Cardiology. He is certified as a Diplomat in Internal Medicine and Adult Cardiovascular Diseases as well as Advanced Heart Failure and Transplant Cardiology.

Marcus A. Keller, PhD — Assistant Professor, Division of Human Genetics, Medical University Innsbruck, Innsbruck, Austria

Dr. Keller's research focuses on the biochemistry and enzymology required to generate and regulate the structural diversity of mitochondrial phospholipids especially with cardiolipins. This includes the coupling of subcellular phospholipid pools via interconversion and transport processes. As a principal investigator at the Division of Human Genetics at the Medical University of Innsbruck, Dr. Keller develops analytical and data analysis approaches to comprehensively characterize lipid compositions, allowing bioinformatically linking the generated high-content data to their underlying lipid metabolic network. The goal of such integrative approaches is to generate a better understanding for the tissue-specific pathobiochemical rearrangements caused by, as well as, causing aberrant cardiolipin compositions.

Dr. Keller studied Chemistry at the Leopold Franzens University Innsbruck and conducted his PhD thesis at the Medical University of Innsbruck studying rare inborn errors in lipid metabolism. Dr. Keller completed a post-doctoral fellowship at the Cambridge Systems Biology Center in Cambridge, UK, where he studied early origins of the metabolic network structures by means of mass spectrometry and metabolic network modeling.

Consuelo M. Kreider, PhD, MHS, OTR/L — Research Assistant Professor, College of Public Health and Health Related Professions, Department of Occupational Therapy, University of Florida, Gainesville, FL, USA

Dr. Kreider's primary clinical interests are in the areas of pediatrics, specifically in neurodevelopmental disorders impacting learning and information processing. Dr. Kreider's twenty years of clinical experience inform and complement her teaching in the areas of human development and pediatric assessment and intervention.

Dr. Kreider investigates means for promoting and improving the participation, health, and wellbeing of youth who have developmental neuropsychological conditions affecting information processing and learning, such as learning, attention and autism disorders, during the transition to adult roles and contexts with a focus on the interaction of the youth and the social-environmental context for supporting desired young adult outcomes.

Dr. Kreider was awarded a PhD in Rehabilitation Science by the University of Florida in 2013. Dr. Kreider holds a Masters in Health Science (2009) and a Bachelor of Health Science in Occupational Therapy (1999) from the University of Florida. She is a member of the American Occupational Therapy Association, the Society for Research on Adolescence, and the Florida Occupational Therapy Association.

Yoonjeong Lim, PhD, OTR/L — Assistant Professor, Lewis College of Nursing and Health Professions, Department of Occupational Therapy, Georgia State University, Atlanta, GA, USA

Dr. Lim conducts research investigating health-related quality of life of children with rare disorders and the impact that these disorders have on family functioning and family quality of life. Dr. Lim's most recent research examined the relationship of a child's functional ability, family cohesion, and satisfaction with healthcare to quality of life and functioning of families of children with rare disorders. She plans to expand her research to diverse early onset childhood rare disorders and to develop effective strategies to support positive family functioning and improve the well-being of families raising children with rare disorders.

Dr. Lim has worked in pediatric outpatient clinics and school-based settings. She treated children and adolescents with disabilities, including autism spectrum disorders, learning disabilities, and sensory processing disorders. Dr. Lim received her bachelor's and master's degrees in Occupational Therapy from Yonsei University in South Korea and her PhD in Rehabilitation Science from the University of Florida.

Xujie Liu, PhD — Postdoctoral Fellow, Department of Cardiology, Boston Children's Hospital, Boston, MA, USA

Dr. Liu's current research interests are modeling heart disease using pluripotent stem cells, drug screen by genome editing, and heart development.

Dr. Liu holds a PhD (2013) in Cell Biology from Peking University Health Science Center, Bejing, China. Dr. Liu joined the Cardiology Department at Boston Children's Hospital in 2017.

Emily Love, BA — Research Associate, Adelphi Values, Boston, MA, USA

Ms. Love has a background in psychology for which she holds a BA from the University of Massachusetts at Amherst. Her experience in the psychology field includes research in family coping after childbirth as well as research in adult experiences of counseling. Ms. Love has previously worked in a residential treatment program for children ages 9 to 12 years and participated in a program where she counseled inmates in a local county prison. More recently, Ms. Love's work experience has been focused on patient-centered outcomes research during her six years with Adelphi Values, with a particular interest in the impact of various health conditions on quality of life among adolescents. Through this work she has had the pleasure of speaking with several of the men with Barth syndrome ranging in age from young children to older adults and is thrilled to attend her first the BSF conference to meet people in person!

Christoph Maack, MD — Chair of the Comprehensive Heart Failure Center (CHFC), University Clinic Würzburg; Department Lead for Translational Science, Würzburg, Germany

Dr. Maack's work focuses on cellular defects in chronic heart failure, with a special emphasis on the mechanisms of contractile, mitochondrial and metabolic dysfunction in heart failure. Other research areas are epigenetic regulation and adrenergic signaling in heart failure.

Dr. Maack received his MD at the University of Cologne (Germany) in 2000. From 2000 to 2017, he mainly worked at the Department of Cardiology at the University of the Saarland in Homburg, Germany. From 2002 to 2005, he performed a post-doctoral research fellowship in the Department of Cardiology at Johns Hopkins University in Baltimore, MD, USA. From 2006 on, he established his own working group in Homburg with the support of the Emmy Noether Programme (2006-2011) and a Heisenberg Professorship on Cardiovascular Physiology and Bioenergetics (2012-2017) of the German Research Foundation (DFG). In 2017, he became the Chair of the Comprehensive Heart Failure Center (CHFC) at the University Clinic in Würzburg, Germany, where he also leads the Department of Translational Science. Dr. Maack was a Board member of the Heart Failure Association (HFA) of the European Society of Cardiology (ESC) from 2010-2016, where he served as the Coordinator of the Translational Research Committee (2011-2014), as the Chair of the Basic Science section on the Executive Committee (2014-2016) and as the organizer of the HFA Winter Meeting (2012-2016). Since 2015, he is a Fellow of the HFA of the ESC and serves on the Program Committee of the German Cardiac Society. In 2018, he was elected to be on the Council of the International Society of Heart Research (ISHR), European Section (ES).

Florence Mannes — Chair, Association Syndrome de Barth France, France; Board of Directors, Barth Syndrome Foundation, USA

Mrs. Mannes has worked for 12 years as a lawyer for a European bank, after graduating from a French business school. In 2010, she established, with her husband, the French affiliate of the Barth Syndrome Foundation, Syndrome de Barth France. She lives with her family just outside Paris, and has three boys, the younger one, who is nine years old, is affected with Barth syndrome. As she retrains to become a speech and language pathologist, she has decided to write her master thesis on "Feeding difficulties in Barth Syndrome." As a consequence, she will also attend the conference as an investigator during the clinics.

Iyar Mazar — Doctoral Candidate, Boston College; Research Manager, Adelphi Values, Boston, MA, USA

Ms. Mazar's research interests include the areas of medical sociology, social psychology, aging and the life course, qualitative methods, and the measurement of health outcomes. As part of her dissertation, Ms. Mazar is examining the management of disease impacts in chronic health conditions, specifically, the social legitimization of unapparent, severe illnesses, as well as means for goal-setting in tenuous health states, focusing on the experiences of individuals with Barth syndrome.

Ms. Mazar holds an MA in Sociology from Boston College and is a PhD candidate in Sociology at Boston College; she serves as a Research Manager at Adelphi Values, a global healthcare outcomes consultancy.

Rebecca McClellan, MGC, CGC — Genetic Counselor, Center for Inherited Heart Disease, Johns Hopkins Medicine; Division of Metabolism, Department of Neurogenetics, Kennedy Krieger Institute, Baltimore, MD, USA

Ms. McClellan's research interests include further unmasking the phenotype of Barth syndrome, investigation of the challenges faced by carriers of X-linked disease, and the ethical impact and outcomes of the ACMG exome sequencing secondary findings panel.

Ms. McClellan has worked since 2002 in the Metabolism Clinic at the Kennedy Krieger Institute, which specializes in supporting patients with mitochondrial and other rare metabolic conditions. In 2013-2014, Ms. McClellan and colleagues established the only interdisciplinary clinic for Barth syndrome in the United States. She has worked closely over the years with the Barth Syndrome Foundation, speaking at their biennial conferences, providing telephone advice and support, and helping create their carrier support initiative. In 2014, Ms. McClellan and Dr. Cynthia James were awarded a research grant to investigate adjustment to carrier status and decision-making regarding reproductive options among Barth syndrome carriers. Since 2014, Ms. McClellan has split her time between Kennedy Krieger and the Johns Hopkins Center for Inherited Heart Disease, where she is working with families affected by arrhythmia conditions or cardiomyopathy.

Ms. McClellan holds a MGC, Genetic Counseling from University of Maryland School of Medicine and a BS in Psychobiology from Albright College, Reading, PA. She is a board-certified genetic counselor.

Susan McCormack — Chair, Board of Directors, Barth Syndrome Foundation, USA

Ms. McCormack spent 30 years in the finance industry before retiring in 2016 to focus on volunteer work and her family. Most recently, she worked as a mutual fund portfolio manager at Putnam Investments in Boston, MA. She joined Putnam in 1994 from Merrill Lynch, where she was an investment banker in Merrill's Los Angeles office. Ms. McCormack graduated from Dartmouth College in 1986 with a degree in mathematics and received her MBA from Stanford Graduate School of Business in 1990. She is a CFA charter holder.

Ms. McCormack lives in Wellesley, MA, with her husband, Ken Marra, their two daughters and their two mixed-breed rescue dogs. In addition to her work with Barth Syndrome Foundation, Ms. McCormack serves as the Secretary and Co-Treasurer of her daughters' middle school PTO.

Katherine McCurdy — an *Emerita*, Scientific & Medical Advisory Board, Barth Syndrome Foundation, USA

Mrs. McCurdy was a founding member of The Barth Syndrome Foundation's (BSF) Board of Directors and was responsible for establishing the Science and Medicine Program of BSF. She currently serves as *Emerita* member of the Scientific & Medical Advisory Board. Mrs. McCurdy has a BA from Duke University and an MBA from the Harvard Business School. She has worked in both economics and the corporate world and has held positions on the boards of various other non-profit organizations.

Her son, Will, was affected by Barth syndrome and passed away in 2014 at the age of 28, but she, her husband, Steve, and their daughter, Eliza, continue to be strongly committed to the mission of BSF.

Stephen McCurdy — Chairman *Emeritus*, Board of Directors, Barth Syndrome Foundation, USA

Mr. McCurdy is a founding director of the Barth Syndrome Foundation (BSF) and is currently Chairman Emeritus of the BSF Board.

Steve and his wife, Kate, also a founding director of BSF who currently serves as the Board's representative to BSF's Scientific and Medical Advisory Board and on BSF's Publications Committee. Both Steve and Kate have reached their term limits and serve as counsel to the BSF Board only. They are the parents of a young man, Will, who died as a consequence of Barth syndrome at the age of 28 in 2014, and of a 23-year old daughter, Eliza.

Steve earned a BS in Economics from the Wharton School at the University of Pennsylvania and an MBA from the Harvard Business School. He retired in 2014 as a Senior Vice President after a 30-year career with American Express in New York. He continues to serve on philanthropic boards and resides in Larchmont, NY.

Emily Milligan — Executive Director, Barth Syndrome Foundation, USA

Mrs. Milligan has spent her career dedicated to improving the lives of children and their families through scientific advancements and social equality. Trained in public health and international relations, Emily brings years of experience managing research portfolios and transforming business processes. Previously, she worked for the United Nations in Brazil and Nicaragua and was a vital contributor at Columbia University and New York University. She went on to join JDRF (formerly known as the Juvenile Diabetes Research Foundation) where she headed the research operations and scientific teams and oversaw an average annual $100 million research portfolio. Most recently, Emily launched an $80 million, mission-driven venture fund that invests in companies developing life-saving products for individuals living with type one diabetes. She has consulted for many non-profits, including the American Cancer Society and Susan G. Komen.

Emily cherishes the 4 children in her life — Anaclara (21), Leonardo (10), Laura (9), and Camilo (4) — and spends a majority of her down time traveling with her husband, Daniel, and their family. She is an active member of her community in Needham, Massachusetts, and volunteers her time for other organizations focused on social change and alleviating human suffering. She is also an avid cyclist and landscaper and most loves spending time on the family farms.

Christina A. Pacak, PhD — Assistant Professor, Department of Pediatrics, University of Florida, Gainesville, FL, USA

Dr. Pacak's primary focus is to develop adeno-associated virus (AAV) mediated gene therapy and mitochondrial transplantation therapy to treat disorders that affect mitochondrial function, particularly Barth syndrome and Cockayne syndrome. Dr. Pacak was awarded the following research grants from BSF entitled "Correction of mitochondrial dysfunction in Barth syndrome" (partial funding provided by Association Syndrome de Barth France — 2014); and "Optimization of AAV-mediated gene therapy for Barth syndrome" (2016).

Dr. Pacak holds a PhD (2006) from University of Florida and a BS from Ohio State University. She completed a postdoctoral research fellowship at Boston Children's Hospital and Harvard Medical School.

Colin K. L. Phoon, MPhil, MD — Associate Professor, Pediatrics, Hassenfeld Children's Hospital, New York University Langone Medical Center and School of Medicine, Division of Pediatric Cardiology, Department of Pediatrics, New York, NY, USA

Dr. Phoon is investigating the role of mitochondria and cardiolipin in the pathogenesis of cardiomyopathy, with a focus on mouse models to test therapies. As a principal investigator or co-investigator on several projects relevant to a broad spectrum of cardiovascular disease in small animal models, he is especially interested in heart development and heart imaging. Dr. Phoon was awarded the following research grants from BSF entitled "Prenatal cardiac phenotype as a platform for testing Barth syndrome therapies" (2017); "Novel antioxidant therapies in a mouse model of Barth syndrome" (2014); "Role of mitochondria during myocardial morphogenesis in Barth syndrome" (2012); and "Cardiomyopathy in a mouse model of Barth syndrome" (2010).

Dr. Phoon is a pediatric cardiologist at New York University School of Medicine, where he has worked since completing his training in 1996. He was the director of the Pediatric & Fetal Echocardiography Lab from 2003-2015 until he stepped down to focus further on his research. Dr. Phoon maintains Board certifications in both General Pediatrics and Pediatric Cardiology.

William T. Pu, MD — Professor of Pediatrics, Harvard Medical School; Director of Basic and Translational Cardiovascular Research, Department of Cardiology, Boston Children's Hospital, Boston, MA, USA; Scientific and Medical Advisory Board, Barth Syndrome Foundation, USA

Dr. Pu's interest is in regulation of heart development, disease, and regeneration, and in using induced pluripotent stem cell technology to model pediatric heart disease. The goals of his research are: (1) to understand the transcriptional network regulating heart development and disease; (2) to understand cell lineage specification in heart development and regeneration; and (3) to understand genetic contributions to congenital heart disease.

In 2014, Dr. Pu's research on Barth syndrome was recognized by the American Heart Association as one of the top ten cardiovascular disease research advances of 2014. Dr. Pu was awarded the following research grants from BSF entitled "Reactive oxygen species and mitochondrial dynamics in the pathogenesis of Barth syndrome" (2013); "Maturation of Barth syndrome models for clinical translation" (partial funding provided by Barth Syndrome Trust — 2012); "Using induced pluripotent stem cells and modified RNAs to model and correct Barth syndrome" (2011); and "Analysis of metabolic abnormalities in *TAZ*-deficient cardiomyocytes" (2009).

Dr. Pu holds an MD from Harvard Medical School. He completed his internship, residency, and pediatric cardiology training at Boston Children's Hospital and is Board Certified in Pediatric Cardiology.

Christian Reynolds, PhD — Assistant Professor, Department of Emergency Medicine, Wayne State University, Detroit, MI, USA

Dr. Reynolds research focuses on exploring pathophysiologic mechanisms contributing to myocardial dysfunction. His lab is particularly interested in the developing heart and maladaptive responses to genetic, environmental, and neuro-humoral triggers.

Dr. Reynolds' graduate research mainly focused on developing and testing a novel infrared light-based therapeutic technology to limit brain injury following perinatal asphyxia. His work contributed to multiple issued US patents and the formation of a university spin-off company. Ongoing studies are focused on continuing to build understanding of this therapy, large animal testing, and human prototype development with the final goal of clinical translation. Ongoing work is supported by an NIH STTR.

Dr. Reynolds' research efforts, as a postdoctoral fellow in the Greenberg Laboratory, focused on exploring pathophysiological mechanisms that contribute to metabolic dysfunction in Barth syndrome. The approach utilized the powerful yeast genetic model to elucidate the molecular mechanism linking cardiolipin deficiency to metabolic dysfunction. Dr. Reynolds helped to generate and characterize the first-ever cardiolipin-deficient mammalian myoblast cell-line, which is now routinely used in the Greenberg Lab to test hypotheses generated from the yeast model. Since accepting his independent position, Dr. Reynolds has continued to collaborate with Dr. Greenberg and they are currently exploring the therapeutic utility of NAD[H] precursor supplementation in Barth syndrome.

Stacey Reynolds, PhD, OTR/L — Associate Professor, Department of Occupational Therapy, Virginia Commonwealth University, Richmond, VA, USA

Dr. Reynolds' research is focused on characterizing behavioral and physiological patterns of sensory processing in children and adults with neurodevelopmental disorders and examining the effectiveness of therapeutic interventions on various aspects of functional performance. Within the Barth syndrome population, Dr. Reynolds has published multiple studies looking at the impact of taste and smell sensitivities on feeding. Dr. Reynolds was awarded a research grant from BSF entitled "A systematic investigation into sensory and motor-based feeding issues in boys with Barth syndrome" (partial funding provided by Association Syndrome de Barth France — 2013).

Dr. Reynolds completed her PhD in Health Related Science at Virginia Commonwealth University (2007) and her post-doctoral training in behavioral neuroscience at the University of Florida (2012). She is a fellow of the American Occupational Therapy Association.

Michael Schlame, MD — Professor, Department of Anesthesiology, Perioperative Care, and Pain Medicine; Professor, Department of Cell Biology; Director, Cardiac Anesthesia, New York University School of Medicine, New York, NY; Chairman, Scientific and Medical Advisory Board, Barth Syndrome Foundation, USA

Dr. Schlame's subspecialties include cardiothoracic anesthesiology and critical care, and his research interests include Barth syndrome, lipids and mitochondria. His clinical focus includes adult and pediatric critical care, cardiothoracic anesthesia, and pediatric anesthesia.

Dr. Schlame is board certified in Anesthesiology both in the US and in Europe. He trained at Charité University Hospital in Berlin, at New York Presbyterian Hospital, and at New York University Medical Center in New York. Dr. Schlame is a recipient of the Barth Syndrome Foundation's Varner Award for Pioneers in Science and Medicine (2016).

Yuguang (Roger) Shi, PhD — Joe R. & Teresa Lozano Long Distinguished Chair Professor, Metabolic Biology, Barshop Institute for Longevity and Aging Studies, University of Texas Health Sciences Center, San Antonio, TX, USA

Dr. Shi's research focuses on identifying novel molecular mechanisms underlying mitochondrial dysfunction in Barth syndrome and aging-related metabolic diseases, including type 2 diabetes, obesity, diabetic complications, and cardiovascular diseases and then developing novel treatments. His laboratory pioneered the research work on identification and characterization of genes encoding cardiolipin synthesis and remodeling, and demonstrated a key role of pathological cardiolipin remodeling in type 2 diabetes and other aging-related diseases. His work also revealed a novel role of pathological remodeling of cardiolipin by ALCAT1 in controlling mitochondrial etiology of Barth syndrome. Dr. Shi was awarded the following research grants from BSF entitled "Molecular mechanisms underlying a causative role ALCAT1 in the pathogenesis of Barth syndrome" (2015); and "Regulation of cardiomyopathy by ALCAT1 in Barth syndrome" (2012).

Previously, Dr. Shi held various positions at Eli Lilly and Company and Penn State College of Medicine where his research led to identification of SAD-A kinase as a mediator of a novel signaling pathway that regulates incretin effect in islet beta cells, the cloning and characterization of PERK kinase, a milestone work in ER-stress and translational control, and several first in class enzymes involved in synthesis and remodeling of triglyceride and phospholipids, including phosphatidylglycerol and cardiolipin.

Colin G. Steward, PhD, FRCP, FRCPCH — Professor of Paediatric Stem Cell Transplantation, School of Cellular & Molecular Medicine, University of Bristol; Honorary Consultant, Bristol Royal Hospital for Children; Founder of NHS Barth Syndrome Service, Bristol, United Kingdom; Scientific and Medical Advisory Board, Barth Syndrome Foundation, USA

Prof. Steward recently retired from his post as Clinical Lead for the multidisciplinary NHS National Barth Syndrome Service which was established in 2010 and is run in partnership with Michaela Damin and her colleagues at the Barth Syndrome Trust. This will allow him to concentrate on research at the University of Bristol whilst having more time for hobbies such as travel, walking, watching wildlife and gardening. His current Barth syndrome research focuses on the CARDIOMAN Trial looking at use of the drug bezafibrate in Barth syndrome together with his colleagues at Bristol Royal Hospital for Children and mechanisms of neutropenia in conjunction with Dr Borko Amulic.

Prof. Steward is a previous recipient of the Barth Syndrome Foundation's Varner Award for Pioneers in Science and Medicine (2012) and the Global Genes RARE Champion of Hope Award for International Medical Care and Treatment (2017).

Jonathan Stokes, MBA — Volunteer

Mr. Stokes has a devoted interest in understanding and bringing to light the patient voice and perspective, with over 16 years of research study design and implementation experience. His experience is primarily in health outcomes research; specifically, the development and evaluation of clinical outcomes of assessment (COAs) for use in clinical trials intended to substantiate product labeling goals, as well as use of COAs in real world clinical practice. Areas of focus include the evaluation of cardinal signs and symptoms of disease, health-related quality of life, improvements and activation in treatment adherence, understanding unmet need, and exploring the burden of disease.

Mr. Stokes has had the distinct honor of partnering with the BSF since 2016 and remains committed to further understanding the Barth syndrome experience from the perspective of those affected by the condition, including individuals with Barth syndrome and their families. Mr. Stokes holds an MBA from Northeastern University.

Donna Strain, RN — Prince Charles Hospital and Chermside Day Hospital; Mother of Affected Individual, Queensland, Australia

Born in Bristol, United Kingdom, Mrs. Strain moved to Adelaide, Australia as a teenager, with her family, in 1987, where she finished school and joined the Australian Army as a Medic. She was transferred to Brisbane in 1996, where she met her husband. After leaving the army, Mrs. Strain studied Natural Medicine and qualified with a diploma in Naturopathy. After working in this field for a short time, she decided to continue her studies at the Queensland University of Technology completing a dual degree in Nursing and Public Health. As a registered nurse, she has since specialised in gastroenterology/endoscopy and has completed graduate studies in this area.

Mrs. Strain has two boys with her husband and has also been fortunate enough to be a step mother to her husband's two daughters. Their eldest son has Barth syndrome and was diagnosed shortly after birth in 1999. They have been lucky enough to benefit from the support and knowledge of the Barth Syndrome Foundation since this time.

Douglas Strathdee, PhD — Head of the Transgenic Technology Laboratory, The Beatson Institute for Cancer Research, Glasgow, United Kingdom

Dr. Strathdee's research interests focus on generating and studying models of human diseases. The aim of research in his lab is to use stem cells to model the processes underlying cancer and to uncover the roles that novel stem cell and reprogramming factors play in the development of the disease. Dr. Strathdee was awarded a research grant from BSF entitled "Characterization of a conditional knockout of *tafazzin* in the mouse" (partial funding provided by Barth Syndrome Trust — 2013).

Dr. Strathdee studied for a PhD at the Beatson Institute for Cancer Research, under the supervision of Professor Allan Balmain. He was awarded a PhD and a BSc in Immunology (Honors) from the University of Glasgow. This was followed by post-doctoral training in the laboratories of Professor John Clark (at the Roslin Institute) and subsequently Professor Seth Grant (now at the University of Edinburgh). In 2009, he moved back to the Beatson as Head of the Transgenic Technology lab, where study models of human diseases are generated.

Arnold W. Strauss, MD — Professor of Pediatrics; University of Cincinnati and Cincinnati Children's Hospital, , Cincinnati, OH; Scientific and Medical Advisory Board, Barth Syndrome Foundation, USA

Dr. Strauss' research interests include the molecular basis of genetic disorders of mitochondrial fatty acid oxidation and myocardial energetics. He has published more than 240 research and invited articles and served as research mentor for more than 60 graduate students and post-doctoral fellows. Dr. Strauss was awarded the following research grants from BSF entitled "*Tafazzin* function in animal models of Barth syndrome" (2004); and "A mouse gene ablation model of Barth syndrome" (2002).

Dr. Strauss was Director of the Cincinnati Children's Research Foundation and Rachford Professor and Chair of Pediatrics at the University of Cincinnati until 2014. Dr. Strauss previously held positions as James C. Overall Professor and Chair of Pediatrics and Medical Director of Vanderbilt Children's Hospital, Nashville, and Professor and Director of the Division of Pediatric Cardiology at Washington University, St. Louis.

Dr. Strauss holds an undergraduate degree cum laude in philosophy from Stanford University and his medical degree from Washington University, St. Louis. Dr. Strauss's pediatric residency and cardiology fellowship was at St. Louis Children's Hospital and Washington University.

Hazel Szeto, MD, PhD — Director of Research, Social Profit Network, Menlo Park, CA, USA

Dr. Szeto received her MD and PhD in Pharmacology from Cornell University Medical College in 1977 and served on the faculty there for 37 years. In 2004, she discovered a novel class of small molecules that selectively target mitochondria to promote oxidative phosphorylation and improve ATP synthesis. With the collaboration of a vast network of academic scientists, Dr. Szeto demonstrated the efficacy of these compounds in numerous preclinical disease models, including ischemic-reperfusion injury, kidney diseases, heart failure, sarcopenia, diabetic complications, neuropathic pain, and neurodegenerative diseases. Her research has resulted in 200 peer-reviewed publications, numerous invited talks, and more than 60 patents.

Carolyn Taylor, MD — Associate Professor, Division of Pediatric Cardiology, The Children's Heart Program of South Carolina, Medical University of South Carolina, Charleston, SC, USA

Dr. Taylor is a pediatric cardiologist with additional specialty training as a pediatric echocardiographer. Her research and clinical interests are in the areas of imaging and evaluation of cardiac function. Assessment of cardiac performance using echocardiography as well as evaluation of functional capacity in various forms of cardiomyopathy and cardio-skeletal myopathy are central to her clinical practice and research effort. She has been actively involved in the Barth syndrome scientific and research community and has published multiple manuscripts on cardiomyopathy, exercise capacity and results from the Barth Syndrome Registry.

Dr. Taylor holds a medical degree from the Chapel Hill School of Medicine, University of North Carolina, NC, USA.

Matthew J. Toth, PhD — Science Director, Barth Syndrome Foundation, Inc.; Scientific and Medical Advisory Board, *ex-officio*, Barth Syndrome Foundation, USA

Dr. Toth completed his PhD in Microbiology at MIT in 1988 and joined a large pharmaceutical company where he was part of several teams involved with drug discovery projects in the therapeutic areas of inflammation and cardiovascular diseases. His laboratory eventually concentrated on making and testing genetically altered mice as a way to advance drug discovery programs. After 2002, Dr. Toth joined a smaller pharmaceutical company and eventually a biotech company where he led several drug discovery programs in the areas of pain and orphan diseases. Since July of 2006, Dr. Toth has been the Science Director of the BSF, where he uses his experience in guiding efforts towards finding treatments and eventually a cure for Barth syndrome.

Hilary J. Vernon, MD, PhD — Associate Professor, McKusick-Nathans Institute of Genetic Medicine, Johns Hopkins University and at the Kennedy Krieger Institute; Director, Barth Syndrome Clinic, Kennedy Krieger Institute, Baltimore, MD; Scientific and Medical Advisory Board, Barth Syndrome Foundation, USA

Dr. Vernon's research interests include understanding intermediary metabolism in Barth syndrome and in disorders of branch chain amino acid metabolism. Dr. Vernon is the Director of the Barth Syndrome Interdisciplinary Clinic at the Kennedy Krieger Institute. She is the chair of the Maryland State Advisory Council on Hereditary and Congenital Disorders, co-director of the Medicine McKusick-Nathans Institute of Genetic Medicine Clinical Trials Unit, and is on the faculty of the North American Metabolic Academy of the Society for Inherited Metabolic Disorders.

Dr. Vernon received her MD and PhD from Rutgers University, New Brunswick, NJ, USA. She completed residencies in Genetics and Pediatrics at Johns Hopkins University, and a fellowship in Clinical Laboratory Biochemical Genetics at Johns Hopkins University. She is board certified in Pediatrics, Clinical Genetics, and Clinical Laboratory Biochemical Genetics.

John Wilkins — Secretary, Board of Directors, Barth Syndrome Foundation, USA

Being affected by Barth syndrome gives Mr. Wilkins a unique insight into the issues facing the Barth Syndrome Foundation. Mr. Wilkins recently earned an A.S. in Computer Information Technology from Southeast Community College in Lincoln, Nebraska, and works part time as a computer consultant. John lives in Lincoln, Nebraska.

Kevin Woodward — Treasurer, Board of Directors, Barth Syndrome Foundation, USA

Mr. Woodward is an Assistant Vice President and a Technical Solutions Manager at T. Rowe Price Associates, a leading global asset management firm. He oversees an IT group responsible for enterprise content management. Mr. Woodward holds the Project Management Professional (PMP) credential which is the globally-recognized and demanded standard in project management professional certifications. He also brings with him a strong background in graphic design and printing.

Mr. Woodward and his wife, Stacey, live with their two children (Connor and Ryan) just outside of Baltimore, MD. Stacey is also deeply committed to and involved with the BSF. Their older son, Connor, has Barth syndrome. Mr. Woodward's perspective as a parent of a child with Barth syndrome will add a valuable voice to BSF Board as we plan for the future.

Jessica Wright — Regina, Saskatchewan, Canada

Mrs. Wright's brother, Jordan, had a heart transplant when he was two and died at three years old from what was probably Barth syndrome. Her brother, Jared, is 24 and has Barth syndrome and cerebral palsy. Mrs. Wright found out she was a carrier of Barth syndrome when she was 18 years old.

Mrs. Wright interests include cosplay, art, ringette, sewing, video games and reading.

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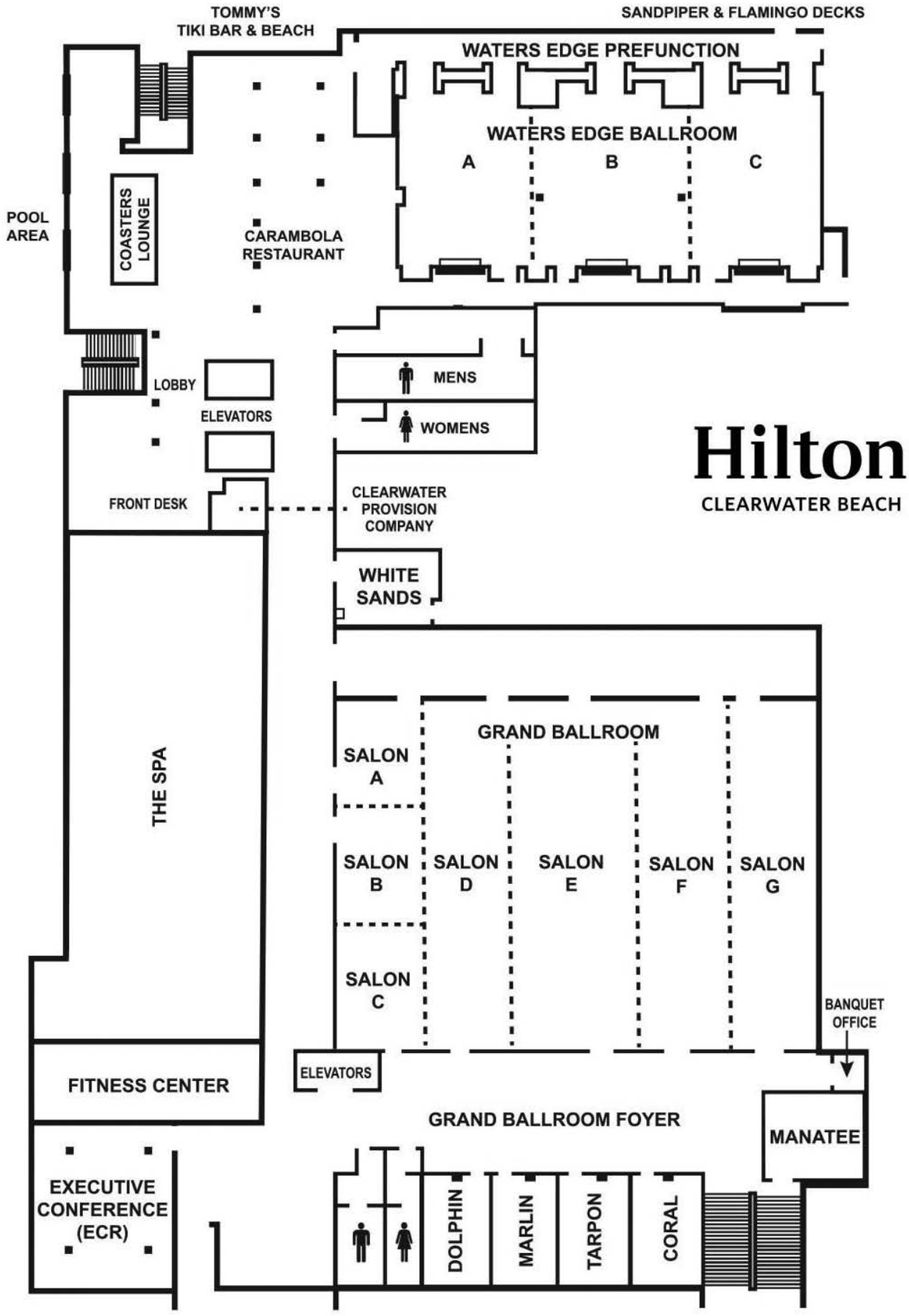
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RESTAURANT
LOBBY
ELEVATORS
MENS
WOMENS
Hilton
CLEARWATER BEACH
FRONT DESK
CLEARWATER
PROVISION
COMPANY
WHITE
SANDS
THE SPA
GRAND BALLROOM
SALON
A
SALON
B
SALON
C
SALON
D
SALON
E
SALON
F
SALON
G
BANQUET
OFFICE
FITNESS CENTER
ELEVATORS
GRAND BALLROOM FOYER
MANATEE
EXECUTIVE
CONFERENCE
(ECR)
DOLPHIN
MARLIN
TARPON
CORAL

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