Inspiration and Determination

Barth Syndrome Foundation
2012 Annual Report

Saving lives through education, advances in treatment, and finding a cure for Barth syndrome.
“The science presented at the Barth Syndrome Foundation 2012 Conference was incredible. I consider this among the most important work I have done in my career.”

Colin Phoon, MD, MPhil, New York University School of Medicine and Langone Medical Center, New York, New York

Photos on pages 1 and 2 courtesy of Amanda Clark ~ 2012
Dear Friends,

The scientific and medical advances that we helped facilitate in 2012 continue to make us optimistic about reaching our long-term goal of finding a cure for Barth syndrome. We are also proud of our successes in increasing awareness, as well as finding and caring for families affected by this life-challenging disorder. While we do not know where the next major breakthrough will come, we are giving ourselves the best chance with the best people to be “lucky” by pursuing a broad range of options. We have worked diligently to keep our global community of affected families, clinicians, scientists, donors, volunteers, and staff focused on the mission. Furthermore, we extend our reach beyond our community, as we engage in broader initiatives. The following are some of the bright spots from 2012:

- Increased awareness within the medical community and the general public
- Empowered newly identified families to become informed advocates; continued care for existing families
- Hosted our sixth highly-successful International Barth Syndrome International Scientific, Medical & Family Conference
- Funded nine research grants for a total of $341,002
- Selected as a National Institutes of Health (NIH) pilot participant in the Global Rare Diseases Patient Registry and Data Repository (GRDR) for the next phase of our Barth Syndrome Registry and Repository; expanding the accessibility and breadth of the information captured, while reducing the cost to BSF
- Raised over $870,000, maintaining our strong financial position
- Expanded use of the “Barth mouse” as a mammalian model of this human disease to more than 10 laboratories around the world
- Facilitated development of two lines of human-induced pluripotent stem cells from donors with Barth syndrome and supported initial work done with these cells
- Initiated development of a clinical trial with the drug bezafibrate in collaboration with the Science Moving Towards Research Translation and Therapy group (SMARTT) at the National Heart, Lung and Blood Institute of the NIH
- Added four new members to BSF’s international Scientific and Medical Advisory Board

BSF continues to blend the best characteristics of a family-centered and a professionally managed organization. We have so much to celebrate; you deserve our greatest respect and deepest appreciation.

Thank you!

Lindsay B. Groff     Stephen B. McCurdy
Executive Director     Chairman

December 2012: Barth syndrome by the numbers

| Known Individuals living with Barth syndrome (BTHS) | 162 in 21 countries |
| Increase in number of individuals identified since December 2011 | 9.3% |
| Grants funded since BSF was established | 72 grant awards totaling $2.7 Million |
| Individuals registered in Barth Syndrome Registry & Repository (BRR) | 78 |
| International Scientific, Medical & Family Conferences held | 6 |
| Percentage of 2012 peer-reviewed journal articles relevant to BTHS that specifically acknowledge support from BSF and/or affiliates | 50% |
Less than 200 living individuals with Barth syndrome are known to us, but anyone who loves someone with this disorder will tell you that it touches the lives of thousands when you consider parents, siblings, grandparents, aunts, uncles, cousins, friends, and neighbors.

The magnetism of these boys and young men is undeniable. Numerous articles have featured the heroic stories of their journeys. Christopher and Henry exemplify how many boys and young men affected with Barth syndrome captivate those who know them, becoming ambassadors for even broader causes. Articles and appearances like the two below help to raise awareness about this rare genetic disorder.

**Christopher** kept busy in 2012, serving as the Children’s Miracle Network Hospitals® Mississippi Champion. He served as an ambassador for Batson Children’s Hospital and all sick and injured children in the state of Mississippi. His story of beating the odds after being given only six months to live has increased awareness of Barth syndrome from his home state all the way to the White House! (Photo courtesy of Batson Children’s Hospital, Jackson, MS.)

**Henry**’s story melted hearts in the Riley Messenger which came out a few days before National Doctors’ Day. As a result of the article and National Doctors’ Day, Henry and Dr. Grzegorz Nalepa shared their story and created awareness for Barth syndrome during radio and television interviews. (Photo courtesy of Riley Children’s Foundation and photographer, David Jaynes.)
Family Services

"The Foundation and the Conference gave us the hope we needed — that Bryn would not have to follow in his brother’s footsteps — that he could grow up." ~ Kate, Mom

Bryn

The first indication of trouble with the pregnancy showed on the last scheduled fetal echo. All of the measurements were normal but the heart "looked thick." A repeat echo ended with the same frustrating result, so an echo was suggested during the first week of life. Bryn was born on December 15, 2011, and, although he was tiny, he was deemed “gestationally appropriate” and large enough not to be put on any watch lists. He also scored a 9-9 Apgar! He latched well and was a sweet, quiet, ever-watchful baby. We were hopeful that the echo would prove he was healthy—for how can a child with a cardiac problem score a 9-9?! However, the echo showed a decline in his heart function from “normal” in the fetal echo to 33% ejection fraction at day two. He also suddenly started showing signs of lethargy, and tests revealed thickened blood and a possible kidney infection. He was transferred to the Intensive Care Nursery at Children’s Hospital at Dartmouth in Lebanon, New Hampshire where he spent the next 11 days.

Our world was rocked again. Bryn’s heart issue appeared different than Rhys’, but how could both our boys have different cardiac problems? Because Barth syndrome (BTHS) was still a suspect for Rhys’ passing, Bryn was referred to Dr. Gerald Cox at Boston Children’s Hospital immediately. A cardiomyopathy genetic panel was drawn, and the waiting game began again. Biochemical urine tests were done, and roughly one year after Rhys’ passing, we got the call from our genetic counselor: lab results from Amsterdam indicated BTHS for Rhys, the urine tests for Bryn indicated the same. They were 99.9% sure—the tafazzin (TAZ) mutation was really just a formality. We got the final genetic results for both boys in early April.

We struggled with the issue that, while Bryn looked so good, he had a disease that had claimed his brother. Friends and family alike could not fully comprehend our anxiety and would, understandably, get frustrated with our reclusiveness. Then, we discovered an entire community of people who not only got the “chronic illness” thing, but understood BTHS as well. Our experience at BSF’s 2012 Conference was nothing short of amazing. Interacting with other affected families showed us that these boys could thrive. Meeting the people who are treating and researching BTHS helped us to understand what Bryn was going through. The Foundation and the Conference gave us the hope we needed—that Bryn would not have to follow in his brother’s footsteps—that he could grow up.

2012 Family Services Spotlight

- Provided support for 162 affected individuals and their families throughout 2012
- Increased the number of affected individuals and families who BSF and/or BSF affiliates serve by 9.3%
- Monitored the family listserv with a total of 3,092 posts
- Conference provided education and support to 187 family members, 44 of whom were affected males
BSF’s 2012 Varner Award for Pioneers in Science and Medicine was awarded to Colin G. Steward, PhD, FRCP, FRCPCH, for his contributions to the science and medicine of Barth syndrome (BTHS). This biennial award is given to a scientist or physician whose dedication to work in his or her field has made a positive and lasting impact on Barth syndrome.

Dr. Steward stands firm in his conviction that Barth syndrome is under-diagnosed, leading him to work tirelessly to raise global awareness about the disorder. In addition, his tenacious leadership has helped expand and improve services for Barth syndrome families in the UK, and he heads the trail-blazing NHS-supported Barth syndrome clinic at the Bristol Royal Hospital for Children that he was instrumental in creating. His dedication and genuine compassion continue to improve the lives of many affected individuals and their families around the world, so it is no surprise that people have described Dr. Steward as determined, honest, approachable, and respectful. His efforts have made a significant and lasting impact within the Barth syndrome community and beyond.
"I am so happy for your progress and look forward to seeing your registry up and running. I am sure that your registry will be one of the models for success. Interacting with organizations like BSF makes our work much more pleasant and gives us a sense of the collaborative effort to fulfill our goals." ~ Yaffa Rubinstein, PhD, Director of Patient Resources for Clinical and Translational Research, Office of Rare Diseases Research, National Center for Advancing Translational Sciences, National Institutes of Health

SCIENCE & MEDICINE

BSF Accepted into NIH-Sponsored Pilot Program

The Barth Syndrome Foundation (BSF) was selected to participate in a two-year pilot project of the National Institutes of Health (NIH) called the Global Rare Disease Registry and Data Repository (GRDR). As a pilot participant, BSF will work in collaboration with leaders in rare disease research at the Office of Rare Diseases Research, National Center for Advancing Translational Sciences, National Institutes of Health, PatientCrossroads, Children’s Hospital of Philadelphia, and WebMD. The GRDR program will collect de-identified patient health information from participating registries established by the individual rare disease organizations in order to allow analyses of data across many rare diseases as well as to facilitate clinical trials and other studies.

This GRDR program builds on our existing registry. To add to the excitement, a new publication entitled, The Barth Syndrome Registry: Distinguishing disease characteristics and growth data from a longitudinal study. (Am J Med Genet A. 2012 Oct 8. doi: 10.1002/ajmg.a.35609 [Epub 2012 Oct 8]) has been released. This paper is an important addition to the field of Barth syndrome clinical knowledge. As the title states, the data collected by the Barth Syndrome Registry & Repository (BRR) are presented for all to reference. Publications like this allow researchers to use the information collected in the BRR to advance our understanding and to help find specific treatments for Barth syndrome. The BSF community has benefited from the research that has emerged in the last decade, and we expect the GRDR effort to attract even more efforts to test scientific or clinical theories.

Expansion of Scientific and Medical Advisory Board

The Barth Syndrome Foundation’s (BSF) international Scientific and Medical Advisory Board is a dedicated team of researchers and physicians who generously donate their time and expertise to our mission. Without them, we would not be able to review grant applications with multi-dimensional expertise or write medically-approved educational materials about Barth syndrome. These eminent scientists and physicians are central to our goals and our operation. BSF is incredibly pleased to introduce our new members, each of whom has agreed to serve for a four-year term. These additions include more sub-specialties, perspectives, and experiences to broaden our horizons and the breadth of our collaborative team.

- W. Todd Cade, PT, PhD, Assistant Professor of Physical Therapy and Internal Medicine, Washington University School of Medicine, St. Louis, MO, USA
- William T. Pu, MD, Associate Professor, Harvard Medical School; Department of Cardiology, Boston Children’s Hospital; Principal Faculty, Harvard Stem Cell Institute, Boston, MA, USA
- Mindong Ren, PhD, Associate Professor, Department of Cell Biology, New York University School of Medicine, New York, NY, USA
- Arnold W. Strauss, MD, BK Rachford Professor and Chair, Department of Pediatrics, University of Cincinnati College of Medicine; Director, Cincinnati Children’s Research Foundation; Chief Medical Officer, Cincinnati Children’s Hospital Medical Center; Professor, UC Department of Pediatrics, Cincinnati Children’s Hospital, Cincinnati, OH, USA

Barth Syndrome Researcher Obtains NIH Award

W. Todd Cade, PT, PhD, Assistant Professor of Physical Therapy and Internal Medicine, Washington University School of Medicine, St. Louis, MO, has received an award from the National Heart, Lung and Blood Institute of the National Institutes of Health for his R01 grant entitled, “Heart and Skeletal Muscle Metabolism, Energetics and Function in Barth Syndrome.” This award is distributed over five years and is in the amount of $1.6 M. This is a significant achievement for which BSF is very proud and excited. Dr. Cade began recruitment for patient participation for this research project at BSF’s 2012 Conference!

BSF Accepted into SMARTT Program

BSF has been accepted into the Science Moving towards Research Translation and Therapy (SMARTT) Program, a part of the National Heart, Lung and Blood Institute of the National Institutes of Health, which supports the translation of novel discoveries into successful new therapies for heart, lung, and blood diseases by providing free, confidential, and rapid preclinical development services to investigators. BSF is working with SMARTT to develop a clinical trial using the drug bezafibrate.
With the completion of the 2012 Barth Syndrome Foundation (BSF) Research Grant Cycle, 11 annual award cycles have committed a total of over $2.7 million to this important effort through 72 research grants to 43 principal investigators. As with all BSF grant cycles, the 2012 cycle grants were awarded the following year, thus being included in 2013 fiscal year expenses. BSF, with the advice of its international Scientific Medical & Advisory Board, and with support from international affiliates, awarded nine research projects. This competitive grant program has resulted in many publications which further describe scientific and medical components of this multi-faceted disease and are leading towards new ideas for treatment. A complete list of all grant awardees can be found on BSF’s website at www.barthsyndrome.org, and those awarded in the 2012 cycle are:

<table>
<thead>
<tr>
<th>Name</th>
<th>Affiliation</th>
<th>Project Title</th>
<th>Award</th>
<th>Funding Source</th>
</tr>
</thead>
<tbody>
<tr>
<td>William T. Pu, MD, PhD, Associate Professor, Boston Children’s Hospital, Boston, MA, USA</td>
<td>Maturation of Barth syndrome models for clinical translation</td>
<td>Award — $40,000 over 1-year period*</td>
<td></td>
<td>Funding for this award was provided by Barth Syndrome Trust (UK &amp; Europe)</td>
</tr>
<tr>
<td>Richard Epand, PhD, Professor, McMaster University, Hamilton, Ontario, Canada</td>
<td>Relationship between membrane physical properties and the action of tafazzin</td>
<td>Award — $37,950 over 2-year period****</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Colin Phoon, MD, MPhil, Associate Professor, New York University Medical Center, New York, NY, USA</td>
<td>Role of mitochondria during myocardial morphogenesis in Barth syndrome</td>
<td>Award — $40,000 over 1-year period**</td>
<td></td>
<td>Funding for this award was provided by the Paula &amp; Woody Varner Fund</td>
</tr>
<tr>
<td>Matthew P. Gillum, PhD, Research Assistant Professor, University of Iowa, Iowa City, IA, USA</td>
<td>Implications of phosphatidylserine deficiency in skeletal muscle and heart of ROSA26-taz shRNA Tet-on mouse model of Barth syndrome</td>
<td>Award — $40,000 over 1-year period</td>
<td></td>
<td>Funding for this award was provided by BSF and BSF of Canada</td>
</tr>
<tr>
<td>Colin Phoon, MD, MPhil, Associate Professor, New York University Medical Center, New York, NY, USA</td>
<td>Role of mitochondria during myocardial morphogenesis in Barth syndrome</td>
<td>Award — $40,000 over 1-year period**</td>
<td></td>
<td>Funding for this award was provided by the Paula &amp; Woody Varner Fund</td>
</tr>
<tr>
<td>Yuguang (Roger) Shi, PhD, Professor, Pennsylvania State University School of Medicine, Hershey, PA, USA</td>
<td>Regulation of cardiomyopathy by ALCAT1 in Barth syndrome</td>
<td>Award — $40,000 over 1-year period</td>
<td></td>
<td>Funding for this award was provided by Association Barth France</td>
</tr>
<tr>
<td>Robert Ryan, PhD, Senior Scientist, Children’s Hospital and Research Center at Oakland, Oakland, CA, USA</td>
<td>Cardiolipin replacement therapy for Barth syndrome</td>
<td>Award — $40,000 over 1-year period</td>
<td></td>
<td>Funding for this award was provided by BSF and BSF of Canada</td>
</tr>
<tr>
<td>Adam Chicco, PhD, Assistant Professor, Colorado State University, Fort Collins, CO, USA</td>
<td>Mechanisms of substrate-specific impairment of oxidative phosphorylation in taz-deficient cardiac mitochondria</td>
<td>Award — $40,000 over 1-year period</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Michael T. Chin, MD, PhD, Associate Professor University of Washington, Seattle, WA, USA</td>
<td>Tafazzin enzyme replacement therapy for heart muscle in Barth syndrome</td>
<td>Award — $40,000 over 1-year period</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Angela Corcelli, PhD, Associate Professor University of Bari, Aldo Moro, Bari, Italy</td>
<td>Determination of the monolysocardiolipin/ cardiolipin (MLCL/CL) ratio in intact nucleated cells: A new tool for the screening of Barth syndrome</td>
<td>Award — $36,300 over 1-year period***</td>
<td></td>
<td>Funding for this award was provided by the Paula &amp; Woody Varner Fund</td>
</tr>
</tbody>
</table>

BSF’s smaller research grants are meant to attract talented researchers whose initial work then successfully receives greater funding from larger institutions. Our strategy continues to produce great results!
FINANCES

Charity watchdogs have always used financial statements as simplistic measures of charities’ worthiness and expressions of responsible management. By all measures, the Barth Syndrome Foundation’s (BSF) recent audited financial statements once again show very strong performance. 2012 produced a slight deficit of $75,864 despite investing $341,002 in nine research grants (the 2011 cycle awarded in early 2012) and bearing the costs of our biennial conference. BSF also continues to maintain a sizeable reserve of $1.8 million in net assets which enables us to take measured risks and confidently invest in our future. We continued to spend less than five cents per dollar raised in fundraising expenses, and more than 81% of total expenses on our programs—better than most charities. We again earned the highest ratings from the Better Business Bureau and met all 43 measures of good governance required by the National Health Council.

More importantly, we remain intensely focused on our mission to make sure that our programs (described in earlier pages of this report) are the best that they can be. The BSF Board and staff are constantly learning from other groups’ best practices, finding new and inventive ways to accomplish our goals with limited resources. Our greatest thanks go to our donors whose continued financial support enables everything. You are truly our “angels!”

**Statement of Financial Position**
December 31, 2012 (with comparative totals for year ended December 31, 2011)

<table>
<thead>
<tr>
<th>Assets</th>
<th>12/31/2012</th>
<th>12/31/2011</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash &amp; cash equivalents</td>
<td>$565,387</td>
<td>$329,443</td>
</tr>
<tr>
<td>Investments</td>
<td>1,303,620</td>
<td>1,499,571</td>
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<tr>
<td>Accounts receivable</td>
<td>37,186</td>
<td>163,351</td>
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<tr>
<td>Prepaid expenses</td>
<td>3,592</td>
<td>2,148</td>
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<tr>
<td><strong>Total assets</strong></td>
<td><strong>$1,909,785</strong></td>
<td><strong>$1,994,513</strong></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Liabilities and Net Assets</th>
<th>12/31/2012</th>
<th>12/31/2011</th>
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</thead>
<tbody>
<tr>
<td>Accounts payable &amp; accrued expenses</td>
<td>$29,938</td>
<td>$19,302</td>
</tr>
<tr>
<td>Grants payable</td>
<td>60,500</td>
<td>80,000</td>
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<tr>
<td><strong>Total liabilities</strong></td>
<td><strong>90,438</strong></td>
<td><strong>99,302</strong></td>
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**NET ASSETS:**

<table>
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<tr>
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<th>12/31/2012</th>
<th>12/31/2011</th>
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<tr>
<td>Unrestricted</td>
<td>849,083</td>
<td>937,779</td>
</tr>
<tr>
<td>Temporarily restricted</td>
<td>970,264</td>
<td>957,432</td>
</tr>
<tr>
<td><strong>Total net assets</strong></td>
<td><strong>1,819,347</strong></td>
<td><strong>1,895,211</strong></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th></th>
<th>12/31/2012</th>
<th>12/31/2011</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Total liabilities &amp; net assets</strong></td>
<td><strong>$1,909,785</strong></td>
<td><strong>$1,994,513</strong></td>
</tr>
</tbody>
</table>

*See annual audit for notes and additional information*
### Statement of Activities

For the Year Ended December 31, 2012 (with comparative totals for the year ended December 31, 2011)

<table>
<thead>
<tr>
<th></th>
<th>Year Ended 12/31/12</th>
<th>Year Ended 12/31/11</th>
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<tr>
<td><strong>PUBLIC SUPPORT AND OTHER REVENUES:</strong></td>
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<tr>
<td>Public Support:</td>
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<tr>
<td>Contributions</td>
<td>$871,480</td>
<td>$706,090</td>
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<tr>
<td>Investment Income</td>
<td>7,975</td>
<td>11,094</td>
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<tr>
<td>Unrealized Gain (Loss) on Investments</td>
<td>990</td>
<td>(1,766)</td>
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<tr>
<td><strong>Total Public Support &amp; Other Revenues</strong></td>
<td><strong>880,445</strong></td>
<td><strong>715,418</strong></td>
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</tbody>
</table>

| **EXPENSES AND LOSSES:** |                      |                      |
| Program Services:         |                      |                      |
| Communications & Other    | 100,710              | 67,997               |
| BSF Conference            | 123,515              | 7,833                |
| Family Services           | 78,148               | 69,098               |
| Barth Syndrome Registry & Repository | 4,800       | 88,901               |
| Research Grants           | 340,823              | 262,115              |
| Research Grants Funded Directly by BSF of CA | (40,000) | (38,350) |
| Science & Medicine        | 171,408              | 164,629              |
| **Total Expense & Losses** | **779,404** | **622,223** |

| Supporting Services:       |                      |                      |
| Management & General       | 137,384              | 119,616              |
| Development & Fundraising  | 39,521               | 10,827               |
| **Total Expense & Losses** | **956,309** | **752,666** |

**CHANGE IN NET ASSETS**

|                                |                      |
|                                 | (75,864)             | (37,248)             |

**NET ASSETS, beginning of year**

|                                | 1,895,211             | 1,932,459             |

**NET ASSETS, end of year**

|                                | $1,819,347            | $1,895,211            |

Note: BSF’s full 2012 audited financials are available on our website at www.barthsyndrome.org.
LEADING THE WAY

The Barth Syndrome Foundation’s Board of Directors provides oversight of governance, fundraising efforts, and the overall guidance of BSF—while BSF’s international Scientific & Medical Advisory Board offers expertise that is invaluable to the mission and future of our organization. Finally, BSF is privileged indeed to have the support of key partners from the public and private community that provide the bulk of the funding for our programs. BSF wishes to thank and recognize all of the individuals for their hard work and dedication.

### Barth Syndrome Foundation

**PO Box 618**  
**Larchmont, NY 10538**

#### BARTH SYNDROME FOUNDATION BOARD OF DIRECTORS

<table>
<thead>
<tr>
<th>Name</th>
<th>Title</th>
</tr>
</thead>
<tbody>
<tr>
<td>Stephen B. McCurdy</td>
<td>Chairman</td>
</tr>
<tr>
<td>Michaela Damin</td>
<td>Board Member</td>
</tr>
<tr>
<td>Susan S. Osnos</td>
<td>Board Member</td>
</tr>
<tr>
<td>Lindsay B. Groff</td>
<td>ex-officio, Executive Director</td>
</tr>
<tr>
<td>Stephen Kugelmann</td>
<td>Board Member</td>
</tr>
<tr>
<td>Marcus E. Sernel</td>
<td>Vice-Chairman</td>
</tr>
<tr>
<td>David Axelrod</td>
<td>MD, Board Member</td>
</tr>
<tr>
<td>Susan A. McCormack</td>
<td>Secretary</td>
</tr>
<tr>
<td>John Wilkins</td>
<td>Board Member</td>
</tr>
<tr>
<td>Randy Buddemeyer</td>
<td>Treasurer</td>
</tr>
<tr>
<td>Katherine R. McCurdy</td>
<td>Board Member</td>
</tr>
<tr>
<td>Susan V. Wilkins</td>
<td>Board Member</td>
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</table>

#### BARTH SYNDROME FOUNDATION EXECUTIVE STAFF

<table>
<thead>
<tr>
<th>Name</th>
<th>Title</th>
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</thead>
<tbody>
<tr>
<td>Lindsay B. Groff</td>
<td>Executive Director</td>
</tr>
<tr>
<td>Shelley Bowen</td>
<td>Director</td>
</tr>
<tr>
<td>Matthew J. Toth</td>
<td>PhD, Science Director</td>
</tr>
<tr>
<td>Lynda M. Sedefian</td>
<td>Executive Assistant</td>
</tr>
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#### INTERNATIONAL AFFILIATES

<table>
<thead>
<tr>
<th>Name</th>
<th>Location</th>
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<tbody>
<tr>
<td>Barth Syndrome Trust (UK &amp; Europe)</td>
<td></td>
</tr>
<tr>
<td>Michaela Damin, Chair</td>
<td></td>
</tr>
<tr>
<td>1 The Vikings</td>
<td>Romsey, Hampshire S051 5RG</td>
</tr>
<tr>
<td>United Kingdom</td>
<td></td>
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<tr>
<td>Barth Trust of South Africa</td>
<td></td>
</tr>
<tr>
<td>Jeannette Thorpe, Chair</td>
<td></td>
</tr>
<tr>
<td>49 Abelia Road</td>
<td>Kloof, Pinetown</td>
</tr>
<tr>
<td>3610 Natal</td>
<td>South Africa</td>
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<tr>
<td>Barth Syndrome Foundation of Canada</td>
<td></td>
</tr>
<tr>
<td>Lynn Elwood, President</td>
<td></td>
</tr>
<tr>
<td>162 Guelph Street, Suite 115</td>
<td>Georgetown, ON L7G 5X7</td>
</tr>
<tr>
<td>Canada</td>
<td></td>
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<tr>
<td>Association Barth France</td>
<td></td>
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<tr>
<td>Florence Mannes, Chair</td>
<td></td>
</tr>
<tr>
<td>12, rue Lalo</td>
<td>75116 Paris</td>
</tr>
<tr>
<td>France</td>
<td></td>
</tr>
</tbody>
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#### SCIENTIFIC & MEDICAL ADVISORY BOARD

<table>
<thead>
<tr>
<th>Name</th>
<th>Institution/Title</th>
</tr>
</thead>
<tbody>
<tr>
<td>Michael Schlame, MD – Chairman</td>
<td>Cell Biology &amp; Anesthesiology, NYU School of Medicine, New York, NY, USA</td>
</tr>
<tr>
<td>Peter G. Barth, MD, PhD – Emeritus</td>
<td>Pediatric Neurology (retired), Emma Children’s Hospital/AMC, Amsterdam, The Netherlands</td>
</tr>
<tr>
<td>W. Todd Cade, PT, PhD</td>
<td>Physical Therapy &amp; Internal Medicine, Washington University School of Medicine, St. Louis, MO, USA</td>
</tr>
<tr>
<td>Gerald F. Cox, MD, PhD</td>
<td>Clinical Genetics, Children’s Hospital Boston, Boston, MA; Clinical Research, Genzyme Corporation, Cambridge, MA, USA</td>
</tr>
<tr>
<td>Iris L. Gonzalez, PhD</td>
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Raphaël, age 4

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