



# MINUTES

Barth Syndrome Foundation, Inc.  
Board of Directors Meeting  
April 8-9, 2017

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**Members in Attendance:**

Marcus E. Sernel	Chairman
Matthew Blumenthal	Board Member
B.J. Develle	Board Member
Nicole Derusha-Mackey	Board Member
Florence Mannes	Board Member
Susan A. McCormack	Secretary, Board Member
Susan Osnos	Board Member
John Wilkins	Board Member
Kevin Woodward	Board Member
Lindsay Groff, Executive Director	Board Member, <i>ex-officio</i>
Stephen B. McCurdy	Chairman <i>Emeritus</i>

**Members Not in Attendance/Partial Attendance**

David Axelrod, MD	Board Member
Randy Buddemeyer	Treasurer, Board Member
Catharine Lynne Ritter	Board Member

**Invited Guests:**

Shelley Bowen, Director, Family Services & Awareness  
Lynda Sedefian, Executive Assistant  
Matthew J. Toth, PhD, BSF Science Director  
Anthony Aiudi, Program Lead, Stealth BioTherapeutics  
Mark Korson, MD, Senior Medical Director, Genetic Metabolic Center for Education (GMCE)

The meeting, held face-to-face in Wellesley, MA, was called to order by the Chairman on Saturday, April 8, 2017, at 9:00 am ET.

The Board of Directors invited Anthony Aiudi, Program Lead, Stealth BioTherapeutics, to attend a portion of the meeting on Saturday, April 8<sup>th</sup>, to provide an update on the upcoming clinical trial on Barth syndrome.

The Board of Directors invited Dr. Mark Korson, Senior Medical Director, Genetic Metabolic Center for Education, to attend a portion of the meeting on Saturday, April 8<sup>th</sup>, to provide an overview of his project entitled, "Patient as A Teacher Project" video series.

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

**Agenda Items:**

1. Ratification of Minutes
2. Update - Finances
3. Update – Fundraising
4. Update & Discussion – 2018 Conference
5. Update & Discussion – Science/Medicine
6. Update & Discussion – Leadership Development
7. Update & Discussion – Family Services & Awareness

\*Items needing follow-up

8. Update & Discussion – Strategic Plan
9. Other

**1. Ratification of Minutes**

The Board approved the January 25, 2017 Board minutes, as amended on this date.  
 The Board approved the February 15, 2017 Board minutes, as amended on this date.

**2. Update - Finances**

Lindsay Groff provided an overview of the February 2017 financial statements, which reflected the following:

*Preliminary Statement of Revenue and Expenditures All Funds – 01/01/2017 – 2/28/2017*

Total Revenues of \$115,844, including unrestricted donations of \$104,881; the balance of \$10,963 is temporarily restricted (Science/Medicine, Varner, and Will McCurdy Funds).

Total Expenses of \$312,977; and a Deficiency of Revenue over Expenditures of \$197,132.

Total Current Assets	\$3,801,542
Total Current Liabilities	\$ 444,236
<b>Total Fund Balance</b>	<b>\$3,357,306</b>

2017 Budget

Lindsay Groff provided an overview of the revised 2017 consolidated budget. The Board engaged in a discussion.

**3. Update – Fundraising**

Lindsay provided a review of 2017 fundraising activities. She discussed the plan for direct mail appeals, e-appeals, grassroots events, and early planning for the 2018 conference. Lindsay also reported that Felicia Ganote will return to her role as a paid contractor with BSF to help with fundraising.

**4. Update & Discussion – 2018 Conference**

Lindsay provided an updated summary of site visits conducted for BSF’s 2018 Conference. After a discussion, the Board agreed that the 2018 Conference will be held at the Hilton Clearwater Beach.

**5. Update & Discussion – Science/Medicine**

Matt Toth provided an update on science and medicine initiatives, including an update on the Research Grant Program, Stealth and elamipretide, the potential Bezafibrate trial, gene therapy, and enzyme replacement therapy.

The Board also heard a detailed presentation from Stealth representative Anthony Aiudi on the status of Stealth’s efforts in preparing for the planned clinical with Stealth’s lead compound (elamipretide) for treatment of Barth Syndrome. The Board thanked Anthony for attending the meeting in person to provide the update and in providing answers to the Board’s questions.

*Barth Registry & Repository 2.0 (BRR2.0)*

Matt and Shelley Bowen met with Drs. Carolyn Taylor and Brian Feingold to discuss coordinating data of the BRR2.0 with the Pediatric Heart Transplant Study (PHTS). These physicians are interested in describing the experience of heart transplantation in the BTHS population and comparing it with the larger PHTS database. In brief, by adding certain questions (5-10) to the existing BRR2.0 format, this new heart transplantation data can be recorded and compared with the PHTS data.

After an in-depth discussion, the Board authorized the Executive Committee to contact Drs. Taylor and Feingold to discuss coordinating data of the BRR2.0 with the Pediatric Heart Transplant Study (PHTS).\*

**VOTED:** The Board approved up to \$7,000, pending Executive Committee approval of the final proposal, for this project.

\*Items needing follow-up

Scientific & Medical Advisory Board (SMAB)

Travel plans are being made for the off-year meeting coinciding with the June 28-July 1, 2017 UMDF (United Mitochondrial Disease Foundation) conference (Alexandria, Virginia). On Thursday, June 29th, the afternoon session will focus on BTHS as a mitochondrial disease with three SMAB members presenting (Drs. Schlame, Vernon, and Pu). BSF has donated to support this afternoon session. The SMAB meeting will be a working dinner on Thursday evening. A few of the SMAB members will attend the entire UMDF conference at a reduced rate. Eleven SMAB members have indicated they would attend.

Matt reported that he has received notification from three of our SMAB members who wish to resign their position as SMAB members and to officially retire from the board: (1) Dr. Peter Barth; (2) Dr. Richard Kelley; and (3) Dr. Iris Gonzalez. Drs. Barth, Kelley, and Gonzalez have each received the Varner award as pioneers in the science and medicine of Barth syndrome.

**VOTED:** The Board accepted the resignation of the above-mentioned SMAB members and expressed their sincere gratitude for their immense contributions.

Matt also reported that the following SMAB members' terms will be renewed for a three-year term: (1) Dr. Michael Schlame; (2) Dr. Todd Cade; (3) Dr. William Pu; (4) Dr. Mindong Ren; and (5) Dr. Arnold Strauss.

**VOTED:** The Board voted to re-elect the above-mentioned SMAB members for a three-year term.

Matt reported that he will seek new members of the SMAB and will keep the Board informed.

The Board also agreed that it would seek the assistance of Kate McCurdy to fill the role of liaison to the SMAB -- a role that Kate filled when previously a member of the Board -- while Cathy Ritter continues to attend to a serious medical emergency in her family.

Lipid meeting

Drs. Angela Corcelli, Peter Buetikofer, and Michael Schlame are planning the third workshop dedicated to cardiopipin, which will be held in Martina Franca (Italy), October 1-2, 2017. This is a follow-up to last year's meeting, and BSF has been asked to offer financial support. Last time BSF contributed \$2,000, and the workshop attendees met with representatives of Association Barth Italy. BSF may wish to contribute to this workshop in 2017.

**6. Update & Discussion – Leadership Development**

Susan Osnos reviewed the term limits of the current Board of Directors. Randy Buddemeyer's term has ended, as of April 2017, as a member of the Board of Directors. Randy served as Treasurer on the Board. The Board thanked Randy for his dedicated service to BSF.

Susan reported that the following Board members' terms are up for renewal: (1) Susan McCormack; (2) John Wilkins; and (3) Kevin Woodward.

**VOTED:** The Board voted to re-elect the above-named Board members for an additional three-year term, commencing on this date. Said term will run through 2020.

Susan reported that Kevin Woodward has accepted the position of Treasurer.

**VOTED:** The Board voted to elect Kevin Woodward as Treasurer of the Board of Directors.

Susan provided an update on proposed candidates for Board membership. After a discussion, Board members were assigned the task of engaging in a preliminary conversation with the proposed candidates to assess their interest in BSF and its mission.

**7. Update & Discussion – Family Services & Awareness**

Shelley Bowen provided an update on initiatives of Family Services & Awareness, as follows:

Collaborative Initiative with Genetic Metabolic Center for Education

Dr. Mark Korson, Senior Medical Director, Genetic Metabolic Center for Education (GMCE), attended a portion of this meeting on Saturday, April 8<sup>th</sup>, to provide an overview of the “Patient as A Teacher Project” video series. The mission of the GMCE is to provide resources and training to clinicians, so they are better equipped to diagnose and care for individuals with genetic metabolic diseases such as BTHS. Shelley proposed that the Board fund the filming of two videos with the following desired outcomes: (1) to promote awareness about BTHS among healthcare providers; and (2) to be used by BSF for awareness/education.

After discussion, the Board agreed to send Dr. Korson videos that BSF has of family interviews for his review, and to ask Dr. Korson to submit a proposal to BSF for editing.\*

Clinical Trials Action Plan

Shelley proposed that BSF host a “Clinical Trials Action Plan” workshop whose ultimate objective is to engage the Barth syndrome community in active participation with clinical trials and ongoing clinical research to enhance understanding and development of more effective treatments for this extremely rare genetic condition. After discussion, the Board recommended that Shelley and Steve McCurdy work together to provide a more detailed recommendation to educate the BSF community on clinical trials and clinical research.\*

**8. Update & Discussion – Strategic Plan**

Lindsay Groff provided a review of BSF’s 2013-2015 strategic plan. After an in-depth discussion, the Board is in the process of finalizing the strategic plan for 2017-2020.\*

**9. Other**

Regional Family Gatherings

The Board engaged in a discussion about regional family gatherings for 2017. To date, there is one outreach scheduled for the Indianapolis area. After discussion, the Board assigned the role of regional family gathering coordinator to John Wilkins.

**VOTED:** The Board voted to elect John Wilkins as regional family gathering coordinator and to create a budget of up to \$1,000 to offset costs of regional gatherings in 2017.

**Adjournment**

The Board of Directors adjourned the meeting on Sunday, April 9, 2017 at 12:00 pm ET. The next Board meeting will be held via teleconference and is scheduled for Wednesday, June 28, 2017. The next Executive Committee meeting is scheduled for Tuesday, May 23, 2017.

Respectfully submitted,



Susan A. McCormack  
Secretary