



MINUTES

Barth Syndrome Foundation, Inc.
Board of Directors Meeting
February 28-March 1, 2015

Members in Attendance:

Marcus E. Sernel	Chairman
Lindsay Groff, Executive Director	Board Member, <i>ex-officio</i>
Randy Buddemeyer	Treasurer, Board Member
Stephen Kugelmann	Board Member
Florence Mannes	Board Member
Susan A. McCormack	Secretary, Board Member
Stephen B. McCurdy	Chairman <i>Emeritus</i>
Susan Osnos	Board Member
Catharine Lynne Ritter	Board Member
John Wilkins	Board Member
Kevin Woodward	Board Member

Members Not in Attendance/Partial Attendance

David Axelrod, MD	Board Member
Randy Buddemeyer (Sunday, March 1)	Treasurer, Board Member

Invited Guests:

Shelley Bowen, Director, Family Services & Awareness
Lynda Sedefian, Executive Assistant
Sandra Stevens, Fundraising Project Manager
Matthew J. Toth, PhD, BSF Science Director

The Board of Directors invited the affiliate leads to join a portion of this meeting via teleconference to participate in the discussion regarding the 2014 Research Grant Cycle which was held on Saturday, February 28, from 9:00am - 1:00pm. Christine Hope, Secretary/Treasurer, BSF of Canada; Sonja Schlapak, Board Member, BS Trust; Kate McCurdy, Emeritus, BSF Scientific and Medical Advisory Board; and Michaela Damin, Chair, BS Trust, joined the meeting for a portion of this discussion.

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

Agenda Items:

1. Discussion – 2014 Research Grant Cycle
2. Update – Science/Medicine
3. Discussion – Board Development
4. Discussion – Family Services & Awareness
5. Ratification of Minutes
6. Review – Preliminary 2014 Financials
7. Review – 2015 Year-to-Date Financials
8. Discussion – 2015 Budget
9. Review – Fundraising
10. Review – Strategic Plan
11. Discussion – 2016 Conference

The meeting, held face-to-face in Wellesley, MA, was called to order by the Chairman on Saturday, February 28, 2015 at 9:00am ET. Marc welcomed Florence Mannes who was elected to serve on BSF's Board of Directors commencing January 2015 for a three-year term.

1. **Discussion – 2014 Research Grant Cycle**

Dr. Matthew Toth, BSF Science Director presented an overview of the 21 grant applications submitted for BSF's 2014 Research Grant Program, and informed the Board of the Scientific and Medical Advisory Board's (SMAB) recommendations for funding based upon: (a) importance and impact (the extent to which the research outlined could make a significant contribution to the understanding of Barth syndrome or the treatment of the disorder); (b) feasibility (qualifications of the investigator(s) to carry out the proposed work; expertise, experience, and commitment; availability of all the elements required for the project, such as technology, reagents, and human subjects); and (c) prospect of long-term funding (the likelihood of subsequent NIH or other major funding for any potential follow-on work, if appropriate).

After Matt's presentation, and careful review and discussion of each proposal by the members of the Board, it was:

VOTED: To fund the following 10 highly qualified grant applications totaling US \$327,400 from the 2014 grant application submissions. The Board of BSF of Canada indicated that they intend to fund a portion of one of these grants. Florence Mannes, Chair, Association Barth France, indicated that they intend to fund a portion of one of these grants. Michaela Damin, Chair, BS Trust indicated that they intend to fund a portion of two grants.

Valerian Kagan, PhD, Professor and Vice-Chairman, University of Pittsburgh, Pittsburgh, PA
"Mechanism and role of cardiolipin oxidation and hydrolysis in Barth syndrome"
Award—US \$100,000 over 3-year period

W. Todd Cade, PT, PhD, Associate Professor, Washington University School of Medicine, St. Louis, MO
"Effects of resistance exercise training on cardiac, metabolic, and muscle function and quality of life in Barth syndrome: Part II"
Award—US \$45,313 plus \$13,500 in travel funds over one-year period
Funding for this award was provided by the Will McCurdy Fund for the Advancement of Therapies for Barth Syndrome

Christina Pacak, PhD, Assistant Professor, University of Florida, Gainesville, FL
"Correction of mitochondrial dysfunction in Barth syndrome"
Award—US \$100,000 over 3-year period
**Partial funding for this award was provided by the Will McCurdy Fund for the Advancement of Therapies for Barth Syndrome*
Partial funding for this award was provided by Association Barth France

Cynthia James, PhD, Research Associate, Johns Hopkins University, Baltimore, MD
"How do women adapt to being a Barth syndrome carrier? A mixed methodological study of psychological adjustment and reproductive options"
Award—US \$48,563 over 2-year period

John L. Jefferies, MD, MPH, Director of Advanced Heart Failure, Cincinnati Children's Medical Center, Cincinnati, OH
"Assessment of quality of life, anxiety, and depression in Barth syndrome: Expanding the scope of comprehensive care"
Award—US \$28,749 over 2-year period
Funding for this award was provided by Barth Syndrome Trust

Adam Chicco, PhD, Associate Professor, Colorado State University, Fort Collins, CO
“Translating murine *Taz* deficiency to human Barth syndrome: Focus on impaired lipid oxidation”
Award—US \$49,998 over 1-year period
Partial funding for this award was provided by Barth Syndrome Trust

Colin Phoon, MD, MPhil, Associate Professor, New York University School of Medicine, New York, NY
“Novel antioxidant therapies in a mouse model of Barth syndrome”
Award—US \$50,000 over 2-year period
Funding for this award was provided by the Paula & Woody Varner Fund

Renata Goncalves, PhD, Postdoctoral Research Associate, Buck Institute for Aging, Novato, CA
“Correcting mitochondrial ROS production to decrease Barth syndrome symptoms”
Award—US \$50,000 over 1-year period

The following grants will be conditionally funded, provided the researchers agree to conditions imposed by the Board, as recommended by the Scientific & Medical Advisory Board.

Trudy M. Forte, PhD, Director of Research, Lypro Biosciences, Inc., Berkeley, CA
“Reversal of cardiolipin deficiency in Barth syndrome mouse model”
Award—US \$49,997 over 1-year period
Funding for this award was provided by the Will McCurdy Fund for the Advancement of Therapies for Barth Syndrome

Michael T. Chin, MD, PhD, Associate Professor, University of Washington, Seattle, WA
“Enzyme replacement therapy in heart failure associated with *tafazzin* deficiency”
Award—US \$50,000 over 2-year period
Funding for this award was provided by the Will McCurdy Fund for the Advancement of Therapies for Barth Syndrome

The Board of Directors expressed their appreciation to Dr. Michael Schlame and to all the members of the SMAB who served as grant reviewers, as well as the additional experts who served as outside reviewers. Additionally, the Board expressed its appreciation to Matt Toth for his coordination of the 2014 grant process on behalf of BSF.

2. Update – Science & Medicine

Request for Support to Plan Clinical Gene Therapy Program for Barth Syndrome

Several SMAB members have responded favorably to the idea of gene therapy for Barth syndrome. The overall goal of the project is to refine and translate the current preclinical work with the goal of pushing toward clinical realization.

VOTED: Approve a budget of up to \$39,000 to fund Request for Support to Plan Clinical Gene Therapy Program for Barth Syndrome.

Funding for this award was provided by the Will McCurdy Fund for the Advancement of Therapies for Barth Syndrome

Note: Steve Kugelmann abstained from voting due to a potential conflict of interest.

CRISPR Technology

Dr. William Pu has suggested that it may be valuable to have a mouse line made with a *tafazzin* mutation using the new CRISPR technology. The mouse line could give researchers, especially the preclinical bezafibrate, a better model to work with.

VOTED: Approve a budget of up to \$6,000 to fund the CRISPR Technology.

Funding for this research was provided by the Paula & Woody Varner Fund

3. Discussion – Board Development

Susan Osnos, Chair of the Board Development Committee, encouraged the Board to continue the search for suitable Board members. It is the Board’s goal to add three new board members in 2015 to improve on the quality of resources on the board.*

Board Terms

The Chairman reviewed the term limits of the current Board of Directors. Steve Kugelmann’s term will end as of April 2015 as a member of the Board of Directors. The Board thanked Steve for his dedicated service to BSF.

4. Discussion – Family Services & Awareness

Shelley Bowen, Director, Family Services & Awareness, reported that numerous families joined BSF in the last quarter of 2014. Shelley has hosted several group meetings with these new families which have been well received. To date, there are 164 living BTHS individuals in BSF’s database.

BRR 2.0

Shelley reported that there are 45 Barth syndrome individuals currently enrolled in the BRR 2.0

5. Ratification of Minutes

The board approved the December 16, 2014 Board minutes, as submitted on this date.
The board approved the February 10, 2015 Executive Committee minutes, as submitted on this date.

6. Review - Preliminary 2014 Year-End Financials

Lindsay Groff provided an overview of the preliminary 2014 year-end comparison financial statements, which reflected the following:

Preliminary Statement of Revenue and Expenditures All Funds – 01/01/2014 – 12/31/2014

Total Revenues of \$1,107,713; including unrestricted donations of \$520,564, and the balance of \$587,149 is temporarily restricted (SciMed and Varner).

Total Expenses of \$1,195,629; and a Deficiency of Revenue over Expenditures of \$87,915.

Total Current Assets	\$ 1,883,582
Total Current Liabilities	\$ 150,502
Total Fund Balance	\$ 1,733,080

Review - Preliminary 2015 Year-to-Date Financials

Lindsay provided an overview of the 2015 year-to-date comparison financial statements, which reflected the following:

*Items placed on the May 26, 2015 Board meeting agenda

Preliminary Statement of Revenue and Expenditures All Funds – 01/01/2015 – 1/31/2015

Total Revenues of \$139,531; including unrestricted donations of \$2,801.

Total Expenses of \$59,294; and an Excess of Revenue over Expenditures of \$80,237.

Total Current Assets	\$ 1,962,368
Total Current Liabilities	\$ 149,051
Total Fund Balance	\$ 1,813,317

Lindsay reported that the 2014 audit with Gray, Gray, & Gray is well underway and almost final.

7. Review – 2015 Budget

Lindsay presented a review of the proposed 2015 consolidated budget. The Board engaged in a conversation regarding the 2015 consolidated budget.

Will McCurdy Fund for the Advancement of Therapies for Barth Syndrome

Lindsay Groff and Steve McCurdy provided a description of a newly created fund in honor of Steve’s son, Will McCurdy. Contributions will be used by BSF for the development of therapies designed to prevent, alleviate, or eliminate the symptoms of Barth syndrome.

8. Review - Fundraising

Lindsay provided a review of 2014 year-end fundraising activities, metrics, and the impact on revenue. Our goal for 2014 was \$1million; we exceeded the goal with a total of \$1,107,713. Donations came from a variety of sources, from long-time donors and new donors, as well as from major gifts and grassroots fundraisers.

9. Review – Strategic Plan

Marc reported that we are on track with the strategic plan that was developed in 2013. It was recommended that the Board revisit the strategic plan in 2016 to develop a plan for the next 5-10 years, focusing on how to accelerate progress in the development of therapies for Barth syndrome. It was also recommended that we seek the advice of outside experts in the scientific and medical community to consult us on this strategic plan.

10. Discussion – 2016 Conference

BSF’s 8th International Scientific, Medical & Family Conference is scheduled for July 18-23, 2016 in Clearwater, FL. Staff has begun preliminary discussions in preparation for the 2016 Conference, including what worked well, what can be improved, and the selection of the Steering Committee.

11. Other

Capacity Building Workshop

Shelley Bowen proposed that BSF host a Capacity Building Workshop for the purpose of engaging new (or lapsed) volunteers in organizational activities, buy-in to program plans, and possible commitment to the board. Historical success of volunteer retreats in the early years of BSF has resulted in long-term commitments to organizational and board leadership.

VOTED: To approve a budget of up to \$15,000 to host a Capacity Building Workshop in late 2015.

IT Consultant

Shelley proposed that BSF hire an IT consultant to conduct a review process to assess the system design of our current listserv platform which has a wealth of information. It was recommended that Shelley submit a proposal to the Board after the IT consultant has completed the review process.

VOTED: To approve a budget of up to \$4,000 to hire an IT consultant to conduct a review process to assess the system design of our current listserv platform.

Outreach Program

Shelley proposed that BSF organize, along with support from volunteers, two family outreaches in 2015 to build regional communities within the medical profession and among families to support BSF's overall goals and objectives.

VOTED: To approve a budget of up to \$6,000 to cover staff travel for two outreaches in 2015.

Adjournment

The Board of Directors adjourned the meeting at 12:00pm. The next Board meeting is scheduled for Tuesday, May 26, 2015. The next Executive Committee meeting is scheduled for Tuesday, April 14, 2015.

Respectfully submitted,

A handwritten signature in cursive script, appearing to read "Susan A. McCormack".

Susan A. McCormack
Secretary