



MINUTES

Barth Syndrome Foundation, Inc.
Board of Directors Meeting
February 20, 2016

Members in Attendance:

Marcus E. Sernel	Chairman
David Axelrod, MD	Board Member
Matthew Blumenthal	Board Member
Randy Buddemeyer	Treasurer, Board Member
Florence Mannes	Board Member
Catharine Lynne Ritter	Board Member
John Wilkins	Board Member
Kevin Woodward	Board Member
Stephen B. McCurdy	Chairman <i>Emeritus</i>
Lindsay Groff, Executive Director	Board Member, <i>ex-officio</i>

Members Not in Attendance/Partial Attendance

Susan A. McCormack	Secretary, Board Member
Susan Osnos	Board Member

Invited Guests:

Kate McCurdy, Scientific and Medical Advisory Board, *Emerita*
Lynda Sedefian, Executive Assistant
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 2015 Research Grant Cycle. Susan Hone, President, BSF of Canada; Christine Hope, Secretary/Treasurer, BSF of Canada; Paola Cazzaniga, President, Association Barth Italy; and Daniela Toniolo, PhD, Association Barth Italy, 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 – 2015 Research Grant Cycle
2. Discussion – 2016 DRAFT Budget
3. Review – January 2016 Financials
4. Other

The meeting, held via teleconference, was called to order by the Chairman on Saturday, February 20, 2016 at 9:00am ET. Marc welcomed all to the meeting and provided a summary of the grant process.

1. Discussion – 2015 Research Grant Cycle

Dr. Matthew Toth, BSF Science Director presented an overview of the 16 grant applications submitted for BSF's 2015 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 six (6) highly qualified grant applications totaling a maximum of US \$350,000 from the 2015 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.

George Schweitzer, PhD, Postdoctoral Research Associate
Washington University School of Medicine, St. Louis, MO
"Metabolic adaptation in Barth syndrome"
Award—US \$50,000 over 1-year period

Yuguang (Roger) Shi, PhD, Professor
University of Texas Health Sciences Center in San Antonio, San Antonio, TX
"Molecular mechanisms underlying a causative role ALCAT1 in the pathogenesis of Barth syndrome"
Award—US \$100,000 over 3-year period

Miriam Greenberg, PhD, Professor
Wayne State University, Detroit, MI
"Cardiolipin is required for mitochondrial protein processing"
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.

Eric Ortlund, PhD, Associate Professor
Emory University, Atlanta, GA
"Structural studies of human *tafazzin*"
Award— a maximum of US \$50,000 over 1-year period

Robin Duncan, Assistant Professor
University of Waterloo, Waterloo, Ontario, Canada
"A new enzyme and pathway in cardiolipin synthesis"
Award— a maximum of US \$50,000 over 2-year period

Doron Rapaport, PhD, Professor
University of Tuebingen, Tuebingen, Bavaria, Germany
"Use of high throughput screens in yeast to investigate the pathomechanism of Barth syndrome"
Award— a maximum of US \$50,000 over 2-year period

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 2015 grant process on behalf of BSF.

At this time, all affiliate leads left the meeting. The BSF Board of Directors thanked them for their involvement in and support of BSF's 2015 Research Grant Program.

2. **Discussion – 2016 DRAFT Budget**

Lindsay presented a review of the preliminary proposed 2016 consolidated budget in light of the funding decisions made. The Board engaged in a discussion and gave Lindsay to proceed according to her proposed budget.

3. **Review – January 2016 Financials**

Lindsay Groff provided an overview of the January 2016 comparison financial statements, which reflected the following:

Preliminary Statement of Revenue and Expenditures All Funds – 01/01/2016 – 1/31/2016

Total Revenues of \$13,273 including unrestricted donations of \$10,219; the balance of \$3,054 is temporarily restricted (SciMed, Varner, and Will McCurdy Funds).

Total Expenses of \$48,165; and a Deficiency of Revenue over Expenditures of \$34,892.

Total Current Assets	\$ 3,873,134
Total Current Liabilities	\$ 257,984
Total Fund Balance	\$ 3,615,150

4. **Other**

Bezafibrate Clinical Trial

Matt provided an update on how to best proceed with a US bezafibrate clinical trial. Matt will continue his efforts and will keep the board informed.*

Predictive Drug Repurposing for Barth Syndrome

Matt reported that he has received a proposal from Healx entitled, “Predictive Drug Repurposing for Barth Syndrome.” The objective of this work is to (a) analyze existing transcriptomic data and generate further data in Barth syndrome; and (b) identify the top 12 drug repurposing candidates for the treatment of Barth syndrome. Following a discussion, the board authorized Matt to pursue final negotiations with Healx, with oversight from Lindsay and the Executive Committee.

VOTED: Approve a budget of up to \$65,000 to fund Predictive Drug Repurposing for Barth Syndrome.

At this time all BSF staff members left the meeting at which time the BSF Board of Directors met to discuss 2015 staff evaluations and vote on compensation [confidential session].

Adjournment

The Board of Directors adjourned the meeting at 1230 PM. The next Board meeting is scheduled for Thursday, April 14, 2016. The next Executive Committee meeting is scheduled for Tuesday, March 22, 2016.

Respectfully submitted,



Marcus E. Sernel
Chairman

*Items placed on the March 22, 2016 Executive Committee meeting agenda