



MINUTES

Barth Syndrome Foundation, Inc.
Board of Directors Meeting
February 15, 2017

Members in Attendance:

Marcus E. Sernel	Chairman
Matthew Blumenthal	Board Member
Randy Buddemeyer	Treasurer, 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>

Members Not in Attendance/Partial Attendance

David Axelrod, MD	Board Member
Catharine Lynne Ritter	Board Member
Stephen B. McCurdy	Chairman <i>Emeritus</i>

Invited Guests:

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 2016 Research Grant Cycle: Susan Hone, President, BSF of Canada; Christine Hope, Secretary/Treasurer, BSF of Canada; 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. Update & Discussion – 2016 Research Grant Cycle
2. Discussion – 2017 Budget
3. Review – Staff Performances

The meeting, held via teleconference, was called to order by the Chairman on Wednesday, February 15, 2017 at 12:00pm ET. Marc welcomed all to the meeting and provided a summary of the grant process.

1. Discussion – 2016 Research Grant Cycle

Dr. Matthew Toth, BSF Science Director presented an overview of the 14 grant applications submitted for BSF's 2016 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 four (4) highly qualified grant applications. 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.

Christina Pacak, PhD, Assistant Professor

University of Florida, Gainesville, Florida

"Optimization of AAV-mediated gene therapy for Barth syndrome"

Award — US \$100,000 over 2-year period

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

Deborah Tribouillard-Tanvier, PhD, Permanent Researcher

CNRS, University of Bordeaux, Bordeaux, France

"Discovery of drug candidates for the Barth syndrome using a yeast-based screening approach and higher eukaryotic models of this disease"

Award — US \$50,000 over 2-year period

**Funding for this award was provided by Association Barth France*

After discussion, the Board noted that the project needs to be re-budgeted due to some prohibited expenses.

W. Todd Cade, PT, PhD, Professor

Washington University, St. Louis, Missouri

"Characterization of the 'metabolic phenotype' in Barth syndrome with cardiac transplantation"

Award — US \$49,820 over 1-year period with \$21,000 in travel expenses

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

The Board agreed with the SMAB recommendation to increase travel funding to \$21,000 to include the 10th volunteer.

Shanta Dhar, PhD, Associate Professor

University of Miami, Miami, Florida

"Cardiolipin enhancing biodegradable nanoparticle for Barth syndrome"

Award — US \$50,000 over 2-year period

**Funding for this award was provided by the Science & Medicine Fund*

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 2016 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 2016 Research Grant Program.

2. Discussion – 2017 Budget

Lindsay presented a brief review of the preliminary proposed 2017 consolidated budget. The Board engaged in a discussion. The Board will continue this discussion at the next meeting.*

3. Staff Performance Reviews

At this time, all BSF staff members left the meeting while the Board met to discuss 2016 staff evaluations and vote on compensation [Executive Session].

Adjournment

The Board of Directors adjourned the meeting at 2:15 pm ET. The next Board meeting will be face-to-face and is scheduled for April 7-9, 2017, in Wellesley, MA.

Respectfully submitted,



Susan A. McCormack
Secretary