



MINUTES

Barth Syndrome Foundation, Inc.
Board of Directors Meeting
February 13, 2018

Members in Attendance:

Marcus E. Sernel	Chairman
David Axelrod, MD	Board Member
Matthew Blumenthal	Board Member
Megan Branagh	Board Member
Nicole Derusha-Mackey	Board Member
B.J. Develle	Board Member
Florence Mannes	Board Member
Susan A. McCormack	Secretary, Board Member
Susan Osnos	Board Member
John Wilkins	Board Member
Stephen B. McCurdy	Chairman <i>Emeritus</i>

Members Not in Attendance/Partial Attendance

Catharine Lynne Ritter	Board Member
Kevin Woodward	Treasurer, Board Member

Invited Guests:

Shelley Bowen, Director, Family Services & Awareness
Kate McCurdy, *Emerita*, SMAB
Lynda Sedefian, Executive Assistant
Matt Toth, PhD, Science Director
Michaela Damin, Chairperson, Barth Syndrome Trust
Susan Hone, President, Barth Syndrome Foundation of Canada
Christiane Hope, Secretary/Treasurer, Barth Syndrome Foundation of Canada
Paola Cazzaniga, President, Barth Italia Onlus
Daniela Toniolo, PhD, Barth Italia Onlus

The Board of Directors invited the affiliate leads to join a portion of this meeting via teleconference to participate in the discussion regarding the 2017 Research Grant Cycle: Susan Hone, President, BSF of Canada; Christiane Hope, Secretary/Treasurer, BSF of Canada; Paola Cazzaniga, President, Barth Italia Onlus; Daniela Toniolo, PhD, Barth Italia Onlus; and Michaela Damin, Chairperson, Barth Syndrome Trust.

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

Agenda Items:

1. Update & Discussion – 2017 Research Grant Program
2. Other

The meeting, held via GoToMeeting, was called to order by the Chairman on Tuesday, February 13, 2018, at 12:00 pm ET. Marc Sernel welcomed all to the meeting.

1. **Update & Discussion – 2017 Research Grant Program**

Dr. Matthew Toth, BSF Science Director, presented an overview of the 13 grant applications submitted for BSF's 2017 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 five (5) highly qualified grant applications.

Borko Amulic, PhD, Lecturer (Assistant Professor)

University of Bristol, Bristol, UK

"Neutrophil dysfunction in Barth syndrome"

Award — US \$49,967 over a 2-year period

**Partial funding for this award was provided by Barth Syndrome Trust*

Martin van der Laan, PhD, Professor

Saarland University, Homburg, Germany

"Mutual connections of mitochondrial membrane architecture and cardiolipin homeostasis in Barth syndrome"

Award — US \$48,906 over a 1.5-year period

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

Miriam Greenberg, PhD, Professor

Wayne State University, Detroit, MI

"Cardiolipin activates pyruvate dehydrogenase (PDH)—a potential new target for treatment of Barth syndrome"

Award — US \$50,000 over a 1-year period

**Funding for this award was provided by Barth Syndrome Foundation*

Richard Eband, PhD, Professor

McMaster University, Hamilton, Ontario, Canada

"The cause and consequences of plasmalogen depletion in Barth syndrome"

Award — US \$50,000 over a 1-year period

**Partial funding for this award was provided by Barth Syndrome Foundation of Canada*

Colin Phoon, MPhil, MD, Associate Professor

New York University School of Medicine, New York, NY

"Prenatal cardiac phenotype as a platform for testing Barth syndrome therapies"

Award — US \$70,000 over a 2-year period

**Partial funding for this award was provided by the Paula and Woody Varner 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 2017 grant process on behalf of BSF.

2. Other

In follow-up to discussions held during the BSF Board of Directors' meeting held on January 26, 2018, it has been proposed to fund the following initiatives:

VOTED: To allow a budget of up to \$6,500 to fund the proposal for adding two sets of questions (QOL and heart transplant) to the existing database (BRR2.0). This addition is in conjunction with the work of Drs. Feingold and Taylor who are pursuing a survey of the experiences of heart transplantation in the BSF community.

VOTED: To allow a budget of up to \$34,000 to fund the Healx-Pu proposal to pursue further testing of the two compounds identified by Healx on BTHS iPS cells. (Pending these results the next phase of work could be testing on BTHS KO or KD mice.)

VOTED: To allow a budget of up to \$37,000 to fund the Adelphi proposal to study the health of a sub-population (older individuals affected with BTHS) within BSF's community.

VOTED: To allow a budget of up to \$40,000 to support a Patient-Focused Drug Development (PFDD) meeting at BSF's 2018 International Scientific, Medical & Family Conference which is scheduled for July 16-21, 2018 in Clearwater Beach, FL.

Adjournment

The Board of Directors adjourned the meeting at 2:45 pm ET. The next Board meeting is scheduled for Monday, April 9, 2018 @ 12:00 pm ET. The next Executive Committee meeting is scheduled for Tuesday, March 13, 2018 @ 12:00 pm ET.

Respectfully submitted,



Susan A. McCormack
Secretary