



MINUTES

**Barth Syndrome Foundation, Inc.
Board of Directors Meeting
February 16, 2019**

Members in Attendance:

Susan A. McCormack	Chair, Board Member
David Axelrod, MD	Board Member
Matthew Blumenthal	Board Member
Megan Branagh	Board Member
Nicole Derusha-Mackey	Board Member
Michelle Florez	Board Member
Florence Mannes	Board Member
John Wilkins	Secretary, Board Member
Kevin Woodward	Treasurer, Board Member
Stephen B. McCurdy	Chairman <i>Emeritus</i>
Emily Milligan	Board Member, <i>ex-officio</i>

Members Not in Attendance

B.J. Develle	Board Member
Brandi Dague	Board Member
Catharine Lynne Ritter	Board Member

Invited Guests

Kate McCurdy, *Emerita*, SMAB
Lynda Sedefian, Executive Assistant
Matthew J. Toth, PhD, Science Director
Daniela Toniolo, PhD, Barth Italia Onlus

The Board of Directors invited the affiliate leads to join this meeting to participate in the discussion regarding the 2018 Research Grant Cycle. Daniela Toniolo, PhD, Barth Italia Onlus joined the meeting.

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

Agenda Items:

1. Review & Discussion – 2018 Research Grant Program

The meeting, held via GoToMeeting, was called to order by Susan McCormack, on Saturday, February 16, 2019, at 10:00 am ET.

1. Review & Discussion – 2018 Research Grant Program

Dr. Matthew Toth, BSF Science Director, presented an overview of the 16 grant applications submitted for BSF's 2018 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, the members of the Board reviewed and discussed the proposals that were recommended for funding by the SMAB and:

VOTED: To fund the following seven (7) highly qualified grant applications.

William Pu, MD, Professor

Boston Children's Hospital, Boston, MA

"Discovery of genetic modifiers of Barth syndrome"

Award—US \$50,000 over 1-year period

**Partial funding for this award was provided by the Paula and Woody Varner Fund*

Catherine F. Clarke, PhD, Professor and Chair

University of California at Los Angeles, Los Angeles, CA

"Deuterated polyunsaturated fatty acids as protective therapy in the treatment of Barth syndrome"

Award—US \$100,000 over 2-year period

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

Riekelt Houtkooper, PhD, Professor

Amsterdam Medical Center, Amsterdam, The Netherlands

"Resolution of the underlying basis for the impaired oxidation of fatty acids in Barth syndrome"

Award—US \$50,000 over 1-year period

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

Jan Dudek, PhD, Independent Junior Group Leader

University Medical Center Wurzburg, Wurzburg, Germany

"Impaired fatty acid utilization and alternate substrates in Barth syndrome"

Award—US \$50,000 over 1-year period

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

David Sykes, MD, PhD, Assistant Professor

Massachusetts General Hospital, Boston, MA

“A new model to address the mechanism of neutropenia in patients with Barth syndrome”

Award—US \$50,000 over 1-year period

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

Christopher Y. Park, MD, PhD, Associate Professor

New York University School of Medicine, New York, NY

“Characterization of hematopoietic stem and progenitor cells in Barth syndrome”

Award—US \$50,000 over 1-year period

**Partial funding for this award was provided by Association Syndrome de Barth France and Barth Syndrome Foundation of Canada*

Miriam Greenberg, PhD, Professor

Wayne State University, Detroit, MI

“Supplementation of critical metabolites improves TCA cycle function and viability of tafazzin-deficient cells”

Award—US \$50,000 over 1-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 2018 grant process on behalf of BSF.

2. Other

The Barth Syndrome Foundation (BSF) Research Grant Program continues to encourage basic science and clinical research on the natural history, biochemical basis, and treatment of Barth syndrome. In addition to this fundamental program, the BSF and its international affiliates announced the availability of specific Request For Applications (RFAs) as part of the BSF 2018 Research Grant Cycle. These RFAs were in addition to the usual appeal for research grant applications on Barth syndrome. The goal of BSF’s Research Grant Program is to advance medical knowledge of the basic mechanism of the disease so as to develop more accurate diagnostic methods and improved specific therapies for children and adults affected by Barth syndrome. All applications for the 2018 cycle were evaluated competitively against each other in the same cycle.

Although BSF considered any research proposal related to Barth syndrome, it was particularly interested in supporting research in the following areas:

- sudden death/arrhythmias in Barth syndrome
- mental and behavioral health issues, such as depression, in Barth syndrome
- gastrointestinal pathophysiology in Barth syndrome

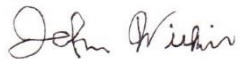
Following an in-depth discussion, the Board:

VOTED: To allow a budget of up to \$100,000 to pursue these research initiatives in 2019.

Adjournment

The meeting was adjourned at 1:30 pm ET. The next Board of Directors meeting is scheduled for Thursday, March 21, 2019 @ 12:00 pm ET. The next Executive Committee meeting is scheduled for Thursday, June 13, 2019 @ 12:00 pm ET.

Respectfully submitted,

A handwritten signature in cursive script that reads "John Wilkins".

John Wilkins
Secretary