



MINUTES

Barth Syndrome Foundation, Inc.
Board of Directors Meeting
February 29th 2020

Members in Attendance:

Susan A. McCormack	Chair, Board Member
David Axelrod, MD	Board Member
Megan Branagh	Board Member
Matthew Blumenthal	Board Member
Brandi Dague	Board Member
B.J. Develle	Board Member
Michelle Florez	Board Member
Florence Mannes	Board Member
Katherine McCurdy	Board Member
Stephen B. McCurdy	Chairman <i>Emeritus</i>
Emily Milligan	Board Member, <i>ex-officio</i>
Peter van Loo	Board Member
John Wilkins	Secretary, Board Member

Members Not in Attendance

Nicole Derusha-Mackey	Board Member
Kevin Woodward	Treasurer, Board Member

Invited Guests

Susan Hone, President, BSF of Canada
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 2019 Research Grant Cycle. Susan Hone, Barth Syndrome Foundation of Canada, and Daniela Toniolo, PhD, Barth Italia Onlus joined the meeting.

Staff in Attendance

Erik Lontok, PhD, Director of Research

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

Agenda Items:

- 1. Research Program Overview**
- 2. Pulse Infotrame Update**
- 3. Review & Discussion – 2019 Research Grant Program**

The meeting, held via GoToMeeting, was called to order by Ms. McCormack, on Saturday, February 29th, at 11am ET.

Agenda Items:

1. Research Program Overview

Ms. McCurdy and Dr. Lontok presented a brief overview of BSF's entire research approach including:

- The existing state of direct BSF research efforts and assets (grant program, various scientific contracts, Human Tafazzin Gene Variants Database, and BRR1.0 and 2.0)
- Research efforts conducted during previous and the upcoming 2020 conference
- Clinical trials, studies, and databases driven by key BTHS stakeholders (NHS Bristol and KKI clinic, TAZPOWER and CARDIOMAN clinical trials, investigator-initiated studies not directly funded by BSF)

2. Pulse Inframe Update

Dr. Lontok provided an update on the work that Pulse Inframe has completed to this date.

3. Review & Discussion – 2019 Research Grant Program

Dr. Erik Lontok, BSF's Director of Research, presented an overview of the 14 grant applications submitted for BSF's 2019 Research Grant Program (now to be called 2020 Research Grant Program so it will be known by the date of award not application) 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 Dr. Lontok'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 projects:

William Pu, MD, Professor

Boston Children's Hospital
Essential activities of Tafazzin that are independent of cardiolipin remodeling
\$50,000 over 1-year period

Robin Duncan, PhD

University of Waterloo
Investigation of a new nutraceutical for treatment of Barth Syndrome
\$41,580 over 2-year period
*Funding for this award will be provided by Barth Syndrome of Canada and Barth Syndrome UK.

Vishal Gohil, PhD

Texas A&M University
Cardiolipin Requirement for Mitochondrial Calcium Import

\$49,999 over 1-year period

Steven Glynn, PhD

Stony Brook University

Structural and Biophysical Studies of Tafazzin

\$50,000 over 2-year period

*Funding for this award will be provided by Association Syndrome de Barth France.

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 external reviewers. Additionally, the Board expressed its appreciation to Dr. Erik Lontok for his oversight of the 2020 grant process on behalf of BSF.

Adjournment

The meeting was adjourned at 1:45 pm ET. The next Board of Directors meeting is scheduled for Tuesday, April 14th at 11 am ET. The next Executive Committee meeting is pending.

Respectfully submitted,

John Wilkins
Secretary