

MINUTES
Barth Syndrome Foundation, Inc.
Board of Directors Meeting
March 5, 2022

Members in Attendance:

Kate McCurdy	Board Member, Chair
Emily Milligan	Board Member <i>ex officio</i> , Executive Director
Kevin Woodward	Board Member, Treasurer
Jamie Baffa	Board Member, Secretary
Andrew Buddemeyer	Board Member
Florence Mannes	Board Member
Megan Branagh	Board Member
Brandi Dague	Board Member
BJ Develle	Board Member

Invited Guests

Erik Lontok	Director of Research, BSF
Melissa Huang	Clinical Research Coordinator, BSF
Daniela Toniolo, PhD	Barth Italia
Michaela Damin	Barth Syndrome UK
Lynn Elwood	Barth Syndrome Foundation of Canada
Susan Hone	Barth Syndrome Foundation of Canada
Chris Hope	Barth Syndrome Foundation of Canada

Members in Not Attendance:

Maryanne Chrisant, MD	Board Member
Nina Russell, MD	Board Member
Peter van Loo	Board Member
Michelle Florez	Board Member

The meeting, held via Zoom, was called to order by Kate McCurdy on Saturday, March 5 at 12:06 p.m. ET.

1. Welcome
 - a. Kate McCurdy welcomed those to the meeting and reminded the board that the foundation has budgeted \$200,000 to fund research grants this cycle
 - b. The board does not need to use the full amount and can vote to exceed based on the opportunities available

2. Review of Grant Proposals

- a. Erik Lontok shared an overview of the basic biology of BSF, what we know today, and the areas of research that the foundation is interested in funding
- b. BSF received 10 grant applications for this cycle and each application has been evaluated by the SMAB based on 1) the importance and impact to Barth syndrome, 2) feasibility of the proposed work, and 3) prospects for long-term funding
- c. Erik then reviewed the following grant proposals:
 - i. *Investigating the basis for neutropenia in Barth syndrome* (Borko Amulic, PhD, University of Bristol / \$49,999.29)
 - ii. *ALCAT1 as a novel target for the treatment of cardiomyopathy in Barth syndrome* (Jun Zhang, PhD, UTHSC San Antonio / \$100,000)
 - iii. *Role of FGF21 in regulating cardiac function and systemic and cardiac metabolism in Barth syndrome* (Jan Dudek, PhD, University Medical Center Würzburg / \$100,000)
 - iv. *Feeding the starving heart in Barth syndrome* (Adam Chicco, PhD, Colorado State University / \$82,400)
 - v. *Expanding the genotype-phenotype spectrum of Barth syndrome through the Genomics England 100,000 Genomes Project* (Richard Pitceathly, MD, PhD, UCL Queen Square Institute of Neurology / \$49,994.99)
 - vi. *Aberrant NLRP3 inflammasome activation contributes the pathogenesis of Barth syndrome* (Jun Zhang, PhD, UTHSC San Antonio / \$50,000)
 - vii. *Restoring balance in cellular oxidative metabolism with alternative energy substrates* (Yana Sandlers, PhD, Cleveland State University / \$49,993)
 - viii. *Cardiolipin metabolism regulates MyoD1 expression and muscle development* (Miriam Greenberg, PhD, Wayne State University / \$50,000)
 - ix. *Effects of in vivo treatment with elamipretide on cardiolipin dynamics and membrane ultrastructure of tafazzin knockdown mitochondria* (Simona Lobasso, PhD, University of Bari Aldo Moro / \$49,500)
 - x. *MLCL/CL: A potential biomarker of disease severity and progression in Barth syndrome* (Robert Pitceathly, MD, PhD, UCL Queen Square Institute of Neurology / \$99,996.84)
- d. Kate then moved the board to vote on the grant proposals, incorporating the recommendations of the SMAB and Director of Research
 - i. **APPROVED:** *Investigating the basis for neutropenia in Barth syndrome* (Borko Amulic, PhD, University of Bristol / \$49,999.26)
 - ii. **APPROVED:** *ALCAT1 as a novel target for the treatment of cardiomyopathy in Barth syndrome* (Jun Zhang, PhD, UTHSC San Antonio / \$100,000)
 - iii. **APPROVED:** *Feeding the starving heart in Barth syndrome* (Adam Chicco, PhD, Colorado State University / \$82,400)
- e. Barth Syndrome UK agreed to fund half of Borko Amulic's proposal (\$25,000)

- f. Barth Syndrome Foundation of Canada agreed to fund the second half of Borko's proposal (\$25,000)
 - g. Kate then dismissed invited guests (excluding the Director of Research) from the meeting
 - h. Kate proposed that the approved proposals not already being funded by Barth Syndrome UK and Barth Syndrome Foundation of Canada be funded through the Will McCurdy Fund for Advancement in Therapies for Barth Syndrome
 - i. **APPROVED:** Jun Zhang's and Adam Chicco's proposals to be funded through the Will McCurdy Fund for Advancement in Therapies for Barth Syndrome
 - j. Kate then recommended that the foundation invite Borko to the SMAB and Erik strongly endorsed this recommendation
 - k. Given that Colin Steward is expected to retire, Kate reinforced her perspective that it's important to have a representative from the Bristol clinic on the SMAB
 - l. **APPROVED:** Borko to be invited to join the SMAB
3. Adjournment
- a. Kate McCurdy adjourned the meeting at 2:06 p.m. ET

Respectfully submitted,

Jamie Baffa – Secretary