



Barth Syndrome
Foundation

Barth Syndrome Foundation, Inc. Board of Directors Meeting

December 12, 2020

Members in Attendance:

Kate McCurdy	Chair, Board Member
David Axelrod, MD	Board Member
Jamie Baffa	Board Member
Matthew Blumenthal	Board Member
Megan Branagh	Board Member
Brandi Dague	Board Member
B.J. Develle	Board Member
Michelle Florez	Board Member
Florence Mannes	Board Member
Emily Milligan	Board Member, <i>ex-officio</i>
Nina Russell MD	Board Member
Peter van Loo	Board Member
Kevin Woodward	Treasurer, Board Member

Invited Guests

Shelley Bowen	Director of Family Services, BSF
Julia Greenstein	Consultant
Erik Lontok	Director of Research, BSF
Susan McCormack	Former Chairperson
Steve McCurdy	Former Chairperson
Marc Sernel	Former Chairperson
Brett Smith	Operations & Communications Lead, BSF

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

Agenda Items:

1. **Welcome**
2. **Process and Goals**
3. **Therapeutic Development Challenges**
4. **Research Roadmap Recommendations**
5. **Discussion**
6. **Vote: 3-Year Research Strategic Plan**
7. **Other Business**
8. **Adjournment**

The meeting, held via Zoom, was called to order by Kate McCurdy, on Saturday December 12, 2020 at 12pm ET.

1. Welcome

- a. This is a really important meeting and culmination of a lot of work with input from staff, consultants, our SMAB, and BSF advisors to equip the Board with information to set priorities in SciMed program over next 3 years.
- b. Former Board chairs have been invited to participate in this process.
- c. At the end of the meeting, all Board members will take a poll about BSF research strategic priorities.
- d. The staff will then apply the Board's input to develop an overall BSF operating plan for 2021 that includes our organizational priorities and corresponding outlay of dollars to be presented for a Board vote in February.

2. Process and Goals

- a. Questions are positioned across 4 different areas: Discovery Research, Therapy Development, Research Mechanisms & Tools, and Strategic Partnerships
- b. Team provided overview of historical funding, with discovery research denoting the primary outlay of funding to create a pipeline of opportunities to reach the therapy development stage
- c. Goal of today's meeting is to build off of Board virtual retreat in October and answer how BSF is going to spend its funds and human resources across these different areas to continue to grow our mission impact.
- d. Staff to present the framework and background on which the proposed plan was generated, followed by Board discussion and vote on priorities

3. Therapeutic Development Challenges

- a. Julia Greenstein provided short recap of research and development environment as well as strategies to save time and money for patients to access treatments

4. Research Roadmap Recommendations and Board Discussion

- a. Erik presented recommendations as drafted in white paper circulated to Board

5. Vote: 3-Year Research Strategic Plan

- a. Having weighed the input from experts and advisors, the Board voted on the following priorities:
 - i. Continue convening of our entire field via the BSF International Conference, as well as communication and fundraising efforts
 - ii. Redirect a portion of grant funds towards specific areas such as drug repurposing or understudied disease management challenges at an estimated annual outlay of \$125k.
 - iii. Launch a dedicated and collaborative natural history study at an estimated annual cost of \$125k per year.
 - iv. Maintain our discovery research efforts with an estimated annual outlay of about \$200k per year.
 - v. Deploy staff/consultant resources to identify, de-risk, and develop partnerships to advance gene/enzyme replacement therapies
 - vi. Convert our registry efforts into a patient engagement/contact database which would require identification of an alternate and equally secure platform to house personal health information

6. Other Business

- a. Discussed, proposed, and **approved** for Jamie Baffa to serve as the Acting Secretary of the Board and member on the Executive Committee until April 2021.
- b. Discussed, proposed and **approved** for BJ Develle to serve as the Chairperson of the Inclusion Committee.

7. Adjournment

With no further business, Kate McCurdy adjourned the meeting at 2:26pm ET. The next Board of Directors' meeting will be scheduled early in 2021.

Respectfully submitted,
Emily Milligan, Notetaker