

MINUTES
Barth Syndrome Foundation, Inc.
Board of Directors Meeting
April 16, 2025

Members in Attendance:

Jamie Baffa	Board Member, Vice-Chair
Megan Branagh	Board Member
BJ Develle	Board Member
Steven Graessle	Board Member
Miriam Greenberg, PhD	Board Member
Mark Greene	Board Member, Treasurer
Michelle Florez	Board Member
Kate McCurdy	Board Member, Chair
Emily Milligan	Board Member <i>ex officio</i> , Executive Director
Peter van Loo	Board Member
Nina Russell, MD	Board Member
Jonathan Stokes	Board Member

Invited Guests

Bill Belscher	Audit Committee, BSF
Natalie Cohn	Controller, BSF
Melissa Huang, PhD	Staff, BSF
Palmer Lummis	Audit Committee, BSF
Lindsay Marjoram, PhD	Staff, BSF
Emily Madalinski	Staff, BSF

Members in Not Attendance:

Brandi Dague	Board Member, Secretary
Stacey Woodward	Board Member

Affiliate Leaders Attending as Observers

Michaela Damin	Barth Syndrome UK
Elena Bilcu	Barth Syndrome Italia
Susan Hone	Barth Syndrome Canada
Florence Mannes	Barth Syndrome France
Daniela Toniolo	Barth Syndrome Italia

The meeting, held via Zoom, was called to order by Kate McCurdy on Wednesday, Apr 16th at 12:00 p.m. ET.

1. Welcome

Kate McCurdy welcomed all attendees, with a special introduction for new board members Steve Graessle and Stacey Woodward (not present due to prior travel). Kate noted that the annual meeting typically includes board onboarding and review of the financial audit. She reminded directors to complete the annual confidentiality and conflict of interest forms.

2. Meeting Minutes

- a. In the absence of Secretary Brandi Dague, Kate McCurdy presented the February 12, 2025, board meeting minutes for approval.

MOTION to approve the February 12, 2025, minutes as amended was made and **APPROVED** unanimously.

- b. Two offline votes were entered into the record:
 1. On February 4th, the Board approved updated website language to clarify BSF's programs and vision in light of the current political environment.
 2. On March 12th, the Executive Committee approved hiring the firm Elephant (\$9,750 quote) for a second year to assist with annual report design.

3. Follow up to Grant Board Meeting

- a. Lindsay Marjoram presented follow up items from the grant meeting in March to clarify funding sources and levels:
 1. Scenic Biotech: Fully funded by Barth France
 2. Dr. Simon Conway: Fully funded through BSF's SciMed Fund
 3. Dr. Jason Moffat: Supported by Barth Canada (\$15k), Barth Italy (\$25k), and Barth UK (\$10k); the balance will be covered by BSF's Therapy Fund

MOTION to approve the proposed funding distributions was made and **APPROVED** unanimously, with appreciation extended to BSF affiliate organizations.

4. Gene Therapy Update

Lindsay provided an update on gene therapy developments. Key points included:

- a. There are 4 distinct research teams working on gene therapy products with a Barth syndrome target.
- b. Lindsay reviewed findings from 3 gene therapy experts who were recruited to review Pu lab Year 2 research. The reviewers had expertise in commercial, nonclinical, and clinical development of gene therapy products.
- c. The experts also peer reviewed year 3 proposed funding to Dr. Pu (Boston Children's Hospital/Harvard University) for the development of a novel capsid gene therapy for Barth syndrome and did not raise any red flags on proceeding. Experts also opined on grant milestones and endorsed this approach.
- d. Lindsay presented proposed next steps for Year 3 mouse studies, including dose selection and 6-month study to assess effectiveness.
- e. Proposed funding to be split into 3 grants, whereby milestones must be met to trigger the next grant:
 1. Grant 1a: Vector selection from vendor (\$8,479), triggered immediately upon approval
 2. Grant 1b: 1-month mouse study (proposed level: \$30,507; required level \$39,283)
 3. Grant 2: 6-month long-term study (proposed level: \$61,014; required level \$78,566)
 4. Grant 3: last 3-months of long-term study (proposed level: \$30,507; required level \$39,283)
- f. Discussion followed about risks related to current federal scrutiny of Harvard and funding at proposed vs. required levels.

MOTION to approve three milestone-based grants totaling up to \$130,507 (proposed) or \$165,611 (required), contingent on Dr. Pu's ability to complete the work at Harvard and on meeting stated milestones, was made and **APPROVED** unanimously.

5. Biorepository and Registry

- a. Melissa Huang reviewed the evolution of BSF's biorepository and registry (BRR), highlighting needs for the registry: electronic medical record (EMR) connectivity, timely addition of surveys, and multilingual access; and for the repository: unified storage, BSF ownership/oversight, LIMS system for real-time tracking of samples, and timely shipping of samples.
- b. With CZI grant funding available to upgrade BSF's BRR, Melissa reviewed internal business due diligence on platforms.
 1. Matrix identified as the most suitable platform for the registry solution (implementation of \$65k; ongoing fixed \$35k/annually + variable costs for surveys/translations)

2. Van Andel Institute proposed as BSF's biorepository vendor (initial fee of \$2,890 with annual storage fee of \$123 for the first year). Shipping costs, addition of new samples and ancillary work will be variable based on usage.

MOTION to approve the proposed BRR strategy and transition to Matrix and Van Andel Institute was made and **APPROVED** unanimously.

6. **Arrhythmia Project & Cardiac Natural History Registry**

- a. The BSF team reviewed the 2022 conceptual overview of the arrhythmia project when it was originally funded through the Strategic Initiative Program with generous support from Barth France. Under this project structure, NYU would serve as the coordinating center, with University of Pittsburgh and Johns Hopkins University as participating sites. The project was originally supposed to be completed in 2024 but has experienced delays, which BSF has actively been trying to resolve in collaboration with the NYU coordinating center team.
- b. Key challenges were shared including delays in IRB approvals at partner institutions, difficulties in data collection and platform integration, and NIH funding (R01) not yet secured by NYU research team.
- c. Separate but related, BSF secured the award from CZI in 2024 to modernize our own registry, creating potential overlap with EMR data-gathering capabilities as originally proposed for the Cardiac Natural History Registry. BSF is focused on working with the NYU team to ensure the NYU registry creates unique value add.
- d. Given concerns around significant delays and resource depletion, BSF is working with NYU to refine the dataset and ensure synergy with the new BRR.
- e. Emily Milligan stressed the urgency of advancing to proof of concept and analysis within the up-to budget and available human resources.

7. **X4 Clinical Trial (Vote)**

- a. Melissa Huang presented the X4 global clinical trial (Mavorixafor) for neutropenia.
- b. Investigational therapy would be administered (or co-administered with G-CSF for those on it) to improve immune health.
- c. Per BSF's clinical trial policy, Board approval is required to share information on trials not specific to Barth syndrome.

MOTION to authorize BSF to publicize the X4 trial to the community was made and **APPROVED** unanimously.

8. **Treasurer Update**

- a. Natalie Cohn provided a financial update for March 2025 year to date:
 - 1. Contributions are flat compared to the prior year.
 - 2. Expenses are slightly lower, likely due to timing.
 - 3. Operating income is stable at \$26k year-to-date.
 - 4. Investment portfolio is currently down \$100k due to equity market instability.
 - 5. A forecast will be conducted in July.

9. 2024 Audit (Vote)

- a. Mark Greene presented high-level audit financials, followed by Bill Belscher on behalf of the Audit Committee.
- b. BSF maintained a strong financial position with total assets of \$5.6M, some of which include donor restrictions.
- c. 83% of 2024 expenditures were program-related—an increase from previous years, largely due to the International Scientific, Medical & Family Conference.
- d. Auditors concluded a clean audit with no material weaknesses identified and acknowledged satisfactory internal controls and timely reporting.
- e. Natalie Cohn, Mark Greene and the Audit Committee were thanked for their good work overseeing this successful audit.

MOTION to approve the 2024 audit as presented was made and **APPROVED** unanimously.

10. Other Business

- a. Retained Search Update:
 - 1. Emily Milligan reported that the search for a Senior Director of Fundraising is progressing in partnership with search firm DDI.
- b. Strategic Plan:
Jamie Baffa reviewed upcoming strategic planning steps, including:
 - 1. Spring: Survey of board and affiliate leaders
 - 2. May: Deep-dive strategy meetings
 - 3. June: In-person offsite
 - 4. August: Final vote on the strategic plan

Meeting adjourned at 2:02 PM ET.

Respectfully submitted,

Emily Milligan
Executive Director