

MINUTES
Barth Syndrome Foundation, Inc.
Board of Directors Meeting
February 12, 2025

Members in Attendance:

Kate McCurdy	Board Member, Chair
Jamie Baffa	Board Member, Vice-Chair
Megan Branagh	Board Member
Brandi Dague	Board Member, Secretary
BJ Develle	Board Member
Michelle Florez	Board Member
Mark Greene	Board Member, Treasurer
Emily Milligan	Board Member <i>ex officio</i> , Executive Director
Jonathan Stokes	Board Member
Peter van Loo	Board Member

Invited Guests

Natalie Cohn	Controller, BSF
Melissa Huang, PhD	Staff, BSF
Emily Madalinski	Staff, BSF
Lindsay Marjoram, PhD	Staff, BSF

Members in Not Attendance:

Miriam Greenberg, PhD	Board Member
Nina Russell, MD	Board Member

Affiliate Leaders Attending as Observers

Ralph Easterbrook	Barth Syndrome UK
Susan Hone	Barth Syndrome Foundation of Canada
Christiane Hope	Barth Syndrome Foundation of Canada

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

1. Welcome

- a. Kate McCurdy welcomed all attendees and emphasized the importance of strategic planning and resource allocation, particularly if the first-ever therapy for Barth syndrome receives FDA approval.

2. Meeting Minutes

- a. Brandi Dague presented the December 10, 2024 board minutes for approval.
- b. Minor edits were noted.
 - 1. MOTION to approve the December 10, 2024 minutes as amended was made and APPROVED unanimously.

3. 2024 Unaudited Financials

- a. Natalie Cohn reviewed unaudited financial results for 2024:
 - 1. Revenue exceeded both the budget and forecast, largely due to strong year-end donations.
 - 2. Expenses were in line with forecasts; some were deferred to future periods.
 - 3. Investment portfolio saw a gain of \$232k, resulting in a deficit of only \$70k against the 2024 budget of -\$581k.
 - 4. Mark Greene noted that while some expenses were pushed into future years, the final numbers did not deviate significantly from projections. He highlighted the impact of the Chan Zuckerberg Initiative (CZI) grant and partial recovery of previously lost funds from 2022.

4. FDA and Elamipretide Update

- a. Kate reported that the FDA deemed additional analyses as a major amendment to Stealth BioTherapeutics' new drug application (NDA) for elamipretide in Barth syndrome, resulting in a 3-month delay for the decision now anticipated on or before April 29, 2025.
- b. Despite the delay, optimism remains strong. Advisors to BSF report that delays in the review of new drug applications are viewed favorably for approval based on historical odds.
- c. It had also been reported that FDA officials referenced partnership with BSF as a case study for ultra-rare disease review processes, another promising signal.
- d. If approved, advocacy strategies for 2025 will need to address:
 - 1. Ensuring access and reimbursement for eligible individuals
 - 2. Expanding the drug label to younger patients, if label only references ages 12 and older consistent with the original clinical trial
 - 3. Supporting global approval and access in partnership with BSF affiliates in the UK, France, and Italy
- e. Emily Milligan discussed the complicated U.S. insurance environment, highlighting the importance of the drug sponsor establishing a free drug program for those without adequate coverage.

- f. Emphasis was placed on exploring manufacturer discounts or patient assistance programs to aid patients with partial coverage.
- g. Emily discussed the Foundation's evolving legislative strategy, aligning it with reimbursement and access goals. She stressed the importance of Rare Disease Week 2025 during which BSF is mobilizing approximately 30 volunteers to continue fostering connections within the U.S. Congress in anticipation that deeper ties may be needed to support drug approval or access challenges.

5. Organizational Statements

- a. Lindsay Marjoram presented on the magnifying effect of federal funding by the NIH to advancing Barth syndrome research. More than \$43M since 2006 through 2024 in follow-on funding for BSF grant recipients.
- b. Emily presented 3 proposals for organizational statements in a politically charged environment which will be important for messaging while advocates are in DC for Rare Disease Week.
 - 1. BSF encourages more federal dollars to be directed to the NIH to further R&D for Barth syndrome, not the opposite;
 - 2. The best path toward #BarthPROGRESS is to continue to work closely with the FDA to refine processes for appropriate and timely review of products for ultra-rare indications like Barth;
 - 3. We suggest adopting new words to describe the important work we do to not potentially disqualify BSF from federal grant funding or draw undue scrutiny that detracts from our mission.
- c. Board discussed considerations of the various proposals.
 - 1. Board unanimously approved proposed positions around NIH funding and FDA.
 - 2. Emily to work with communication experts to propose alternative language on BSF's website.

6. 2025 Budget and Expenditures

- a. Mark Greene laid out a summary of the key assumptions built into the 2025 budget proposal:
 - 1. Support for appropriate elamipretide regulatory review & patient access in the U.S. and potentially the U.K./E.U.
 - 2. Evaluate additional therapy areas for investment
 - 3. Conduct strategic planning for organizational priorities over the next 3-5 years
 - 4. Enhance fundraising efforts to support mission pillars
 - 5. Continue work on the biorepository and registry improvements/expansion, supported by the Chan Zuckerberg Initiative (CZI) grant

6. Partner globally to support the Europe Center of Excellence project
7. Planning for the 2026 International Conference, include beginning to secure financial support and sponsorships to reduce funding gap
- b. The proposed 2025 budget was reviewed and discussed.
 1. The 2025 proposed budget was \$1.864M for revenue, \$2.313M for expenses, resulting in a total proposal of -\$469k, not including unrealized gains from BSF's investment portfolio
 - i. APPROVED as presented, with understanding that adjustments may be needed as strategic planning progresses.
- d. Contract Proposals:
 - i. DDI (Development Search): Board approved moving forward with DDI to assist with the Senior Director of Fundraising search.
 - ii. Elephant (Annual Report): Vote postponed; Emily to explore more cost-effective options and discuss with Jamie.
 - iii. Videography (Rare Disease Week): Proposal declined due to high cost and current political environment. Emily to thank the vendor and formally decline.

7. Strategic Planning

- a. Jamie Baffa outlined the anticipated strategic planning process:
 1. A survey will be distributed to board and affiliates in early spring.
 2. May meetings will include deep dives on planning priorities.
 3. Executive Committee and invited guests will host an in-person offsite in June.
 4. A draft plan will be reviewed by the Board in the summer.
 5. Final discussion and potential vote on the plan in August.

Meeting adjourned at 2:00 PM ET.

Respectfully submitted,

Brandi Dague
Secretary