

**MINUTES**  
**Barth Syndrome Foundation, Inc.**  
**Board of Directors Meeting**  
**November 7, 2024**

**Members in Attendance:**

Kate McCurdy	Board Member, Chair
Jamie Baffa	Board Member, Vice-Chair
Megan Branagh	Board Member
Brandi Dague	Board Member, Secretary
Michelle Florez	Board Member
Miriam Greenberg, PhD	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**

Amos Attali	Consultant, BSF
Matt Burns	Consultant, BSF
Natalie Cohn	Controller, BSF
Melissa Huang, PhD	Staff, BSF
Emily Madalinski	Staff, BSF
Lindsay Marjoram, PhD	Staff, BSF

**Members in Not Attendance:**

BJ Develle	Board Member
Nina Russell, MD	Board Member

The meeting, held via Zoom, was called to order by Kate McCurdy on Wednesday, September 11th at 12:02 p.m. ET.

**1. Welcome**

- a. The meeting began with a welcome from Kate McCurdy, the Board Chairperson.

**2. Meeting Minutes**

- a. Brandi Dague asked for approval for the September 11, 2024, board minutes.
  1. **APPROVED** as written

- b. Offline vote: Commit EUR 100k to AMC for the Center of Expertise contingent on AMC satisfying key provisions to be negotiated in a Letter of Commitment. Namely, the timing of BSF's commitment is specifically to trigger the Dutch Sectorplan Grant funds (EUR 204k) by the deadline of October 1, 2024.
  - 1. **APPROVED** on September 26, 2024, by email
- c. Offline vote: Execute the contract with the Hyatt Regency Coconut Point Resort in Bonita Springs, FL, for the 2026 International Conference
  - 1. **APPROVED** on October 24, 2024, by email

### 3. Treasurer Report

- a. Emily Milligan presented the financial forecast for 2024, highlighting a projected operating deficit of \$432k, a significant reduction from the originally budgeted \$581k.
- b. Drivers of the full year forecast for *revenue* include:
  - i. BSF secured **\$800k grant** from the **Chan Zuckerberg Initiative** to support capacity building for BSF's registry and biorepository, of which \$100k will be recognized as revenue in 2024. BSF anticipates additional expenses in 2025 and future years to progress this initiative, likely including 1 additional headcount, and more revenue will be recognized over a total of 4 years as well.
  - ii. **Outperformed Conference revenue budget offsetting incremental expenses** related to larger than anticipated attendance and support services/contracts. Net cost of about \$170k, slightly better than planned.
  - iii. Revenue includes **\$44k of in-kind support** from commercial and non-profit sources that would have otherwise been obtained through grant applications but instead was recorded as in-kind contributions due to change in funding mechanisms.
  - iv. **Affiliate support remains a critical component** of funding BSF's research activities. While affiliate funding appears down, some revenue was budgeted for expenses that BSF did not incur in 2024 so neither the revenue nor the expense were recognized, therefore having **net zero impact to total forecast**.
- c. Drivers of the full year forecast for *expenses* include:
  - i. Approximately **\$80k in unbudgeted costs related to preparation and participation in FDA elamipretide Advisory Committee meeting** in Q3/Q4.

- ii. **Unbudgeted Euro 100k funding approved to secure Dutch Ministry of Education Euro 204k grant for 4-year support of Center of Expertise in Amsterdam.**
  - iii. **“Savings” in Research Agreements** reflects delays in 2 major projects: Gene therapy and Retrospective Arrhythmia/Cardiac Natural History Registry, with expenses pushed out to 2025 and/or future years so this really is simply a matter of the timing of these expenses.
  - iv. **Hire of Senior Fundraiser** delayed until 2025 resulting in moderate cost savings offset slightly by expected cost for recruiter should BSF pursue this method for sourcing.
- d. In summary, BSF continues to be in a moderate to large deficit on an operating level (-\$432k vs -\$581k budgeted) although not as large as budgeted.
  - e. Past the operating line, BSF has benefited from a strong market and lowering interest rates which has recovered (mostly on an unrealized basis) losses in the investment portfolio from prior years.
  - f. If market trends continue, 2024 may reflect a full year break-even result on a net assets basis.

#### **4. Establishment of a Center of Expertise for Barth Syndrome at AMC in the Netherlands**

- a. BSF consultant (Amos Attali), Lindsay Marjoram, and Emily Milligan presented a business case for a Center of Expertise (CoE) for Barth syndrome at the Academic Medical Center (AMC) in The Netherlands, led by a core team including Prof. Dr. Riekelt Houtkooper, Prof. Dr. Taco Kuijpers, Prof. Dr. Clara Karnebeek, and Dr. Annelieke Müller.
  - i. There is a need for centralized care in the EU post-Brexit as well as expanding clinical trial capabilities globally.
  - ii. AMC as “the birthplace of Barth syndrome” discovery is a strategic location with a strong bench of experienced and dedicated researchers and clinicians.
- b. As part of the business diligence process, Amos aggregated information from stakeholders including Dutch Patients, Dutch Patient Organization for Rare Diseases (VKS), AMC experts, BSF France, BSF Italy, BSF-UK.
- c. The initial focus of the CoE will be on serving patients in the Netherlands and Belgium.
- d. Building on the positive momentum from the CoE meeting at the July 2024 International Barth Syndrome Conference, conversations with collaborators in

France, Italy, Germany, and potentially other countries may expand the scope of the center in future phases.

- e. An unanticipated opportunity emerged in August whereby the Dutch Ministry of Education awarded AMC €204,000 over 4 years contingent on AMC securing matching funds from BSF to support the appointment of the CoE lead.
- f. With funding secured, Dr. Priscilla Thakoerdien, MD and MSc in Biomedical Sciences, has since been appointed by the AMC team as CoE Coordinator. Her appointment will commence December 1st as part of her PhD program.
- g. In order for the CoE to be sustainable financially long-term, it will be imperative for the AMC team to apply to the ECZA (equivalent of Dutch Ministry of Health agency) for formal CoE recognition in the Netherlands.
- h. Some challenges that remain to be navigated include a need for Care Pathway consensus, possible ECZA reapplication if unsuccessful in first round in 2025, securing sustainable funding options beyond the initial 4-year investment if ECZA does not support the CoE in full, and ensuring AMC registry's use can be leveraged optimally by coordinating with other existing datasets.
- i. As negotiated by BSF in the Letter of Commitment, BSF must also identify an ambassador to participate in the AMC CoE steering committee meetings to ensure the patient perspective is integrated into the evolution of the CoE.
- j. BSF envisions partnering with BSF's European affiliates and partners will be an important consideration for the long-term sustainability and success of this center.

## **5. FDA Advisory Committee (Ad Comm) Update**

- a. Kate McCurdy provided a brief update on regulatory affairs for access to elamipretide for the Barth syndrome community.
- b. On October 10, 2024, the AdComm voted 10-6 in favor of elamipretide showing efficacy in Barth. While AdComm votes are simply advisory and the FDA retains the final decision-making authority, this was an encouraging outcome.
- c. The favorable vote was in large part due to BSF's patients and clinicians' compelling testimonies.
- d. Dr. Patrizia Cavazzoni, Head of FDA's CDER, attended the full day meeting, which is highly unusual and highlights the engagement by FDA leadership in this case.
- e. Kate reread the statement that Dr. Cavazzoni made at the AdComm meeting, "In a disease such as this one, an ultrarare disease, we accept a greater degree of residual uncertainty compared to a high-prevalence disorder."
- f. Kate emphasized that Dr. Cavazzoni's perspective mirrors BSF's advocacy message that we have don't want FDA to lower its standards, rather to provide a fair, equitable, and appropriate review for a potential new drug in an ultra-rare indication like Barth syndrome.

- g. There is still reasonable concern about whether the FDA will approve the new drug application.
- h. Brandi Dague expressed gratitude for all who provided testimonies and mobilized to make the day a success. She also raised the question about the incoming administration and timing of the FDA's decision.
- i. Emily stated that, in partnership with DC rare disease coalitions and legislators, BSF will continue to seek to understand if and how the incoming administration may influence the trajectory of the new drug application.
- j. Given the proximity of the inauguration to the PDUFA date, at this point BSF has no reason to anticipate implications for the FDA's decision nor that it will be made prior to the PDUFA date of January 29, 2025. Time will tell.

## 6. **BSF Advocacy Strategy & Vote**

- a. Emily Milligan introduced BSF consultant Matt Burns to describe BSF's advocacy strategy leading up to the FDA PDUFA date and through Rare Disease Week 2025.
- b. Matt presented on the campaign objectives:
  - i. **Short-term Objective:** Support FDA approval of elamipretide
  - ii. **Long-term Objective:** Strengthen connections to BSF to support long-term mission fulfillment
- c. The board reviewed four primary campaign goals which included:
  - i. Champion progress achieved through resilience, determination, and community engagement
  - ii. Raise continued awareness regarding the need for appropriate and equitable FDA review to address unmet clinical needs in the Barth community
  - iii. Express gratitude for progress while advocating for FDA approval
  - iv. Maintain positive focus among families and allies during FDA decision period
- d. Matt presented the campaign timeline and activities with the foci being:
  - i. November 2024: "Gratitude for Progress"
  - ii. December 2024: "Gift of Progress"
  - iii. January/February 2025: "Barth Champions of Progress"
- e. Emily reviewed the necessary budget allocations to support the advocacy campaign.
  - i. Total estimated financial impact is approximately \$10.5k plus BSF FTE in 2024, with a majority, approximately \$60k and BSF FTE, anticipated in 2025.
  - ii. **Approved:** Up to \$70k for BSF ambassadors' and staff travel to Rare Disease Week 2025, Quorum Interactive Legislative Platform for effective

engagement of Congressional leaders, Social Media Video Production, Champions of Progress Awards, and other supportive services.

- iii. **Approved:** BSF staff to pursue corporate underwriting possibilities to offset at least some of these expenses.

## 7. **SMAB Nominees, Grant Review Representative, & Votes**

- a. Lindsay Marjoram presented several SMAB-related items for board approval:

- i. Five members renewed for 4-year terms: Todd Cade, Mindong Ren, Bill Pu, Michael Schlame, and Riekelt Houtkooper

- ii. Colin Steward renewed through 2026

- iii. Arnie Strauss, after many years of dedication and service, retired from SMAB; he was thanked for his long and wonderful service to the BSF community

- iv. Michael Schlame stepping down as chair but remaining on SMAB

- v. New appointments included:

- 1. Dr. Stacey Reynolds: New member with expertise in fatigue research and clinical applications

- 2. Dr. Laura Hagerty: New member with industry and drug approval experience

- 3. Dr. Todd Cade: Nominated as new SMAB chair

- 4. Dr. Mindong Ren: Appointed as Grant Review Representative for 2024 cycle

- a. The Scientific Medical Advisory Board Grant Review Representative (SMAB GRR) will serve as a key participant in the BSF grant program board meeting, which typically occurs in February/late March, if and when the SMAB Board Liaison (who usually fills this role) is conflicted in a grant cycle and therefore cannot participate. The GRR will provide expert insights during grant funding discussions to ensure that grant decisions are aligned with the organization's goals and offer a clear, lay-friendly perspective on the relevance and fundability of proposed grants. This position rotates among SMAB members to ensure impartiality and non-conflict when funding is sought.

- vi. **Approved:** The complete slate of SMAB appointments and changes as presented.

**8. Additional Board Business**

- a. **Resolved:** To establish a \$10,000 board match for Giving Tuesday, with all board members encouraged to participate at some level to demonstrate that the board is unanimously behind this effort.
- b. The board discussed inviting affiliate leaders to observe BSF board meetings to improve global coordination.
  - i. **Approved:** To invite affiliate leader (or proxy) and one affiliate Board member as observers to BSF board meetings through April 2025 as a trial period, subject to signing NDAs.
- c. Emily reminded the Board of the November 19th webinar updating the community on AdComm results and encouraged attendance.

9. **Adjournment.** The meeting was adjourned by Kate McCurdy at 2:04 pm ET.

Respectfully submitted,

Brandi Dague

Secretary