

## MINUTES

### Barth Syndrome Foundation, Inc. Board of Directors ANNUAL Meeting

April 30, 2024

#### **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
Miriam Greenberg, PhD	Board Member
Mark Greene	Board Member, Treasurer
Michelle Florez	Board Member
Emily Milligan	Board Member <i>ex officio</i> , Executive Director
Nina Russell, MD	Board Member

#### **Invited Guests**

Shelley Bowen	Staff, BSF
Lindsay Marjoram, PhD	Staff, BSF
Steve McCurdy	Volunteer, BSF
Emily Madalinski	Staff, BSF
Melissa Huang, PhD	Staff, BSF
Natalie Cohn	Controller, BSF
Bill Belscher	Audit Committee, Chair
Palmer Lummis	Audit Committee
Ed Baltazar	Auditor, Dorfman
James Valentine	Regulatory Advisor, HPM

#### **Members in Not Attendance:**

Andrew Buddemeyer	Board Member
Florence Mannes	Board Member
Jonathan Stokes	Board Member
Peter van Loo	Board Member

The meeting, held via Zoom, was called to order by Kate McCurdy on Tuesday, April 30 at 12:03 p.m. ET.

1. Welcome and Gratitude for Service
  - a. The meeting began with a welcome from Kate McCurdy, the Chairperson.
  - b. Kate began by thanking directors who are completing their service: Florence Mannes, President of Association de Barth France, for her 9 years (3-term limit) of tremendous contribution and service on the BSF Board; and Andrew Buddemeyer who is stepping down from the BSF Board after one term (3 years) of service due to competing demands with his professional commitments. We will miss the important and broad perspectives each of them has offered and

are pleased that both will remain integral and engaged members of our community.

- c. Kate also thanked Susan McCormack for her capable and diligent leadership of the Policies and Agreements Committee; she is stepping down from chairing the committee to focus her volunteerism in other meaningful ways for BSF.
- d. Kate also reported that the Board Development Committee decided to not propose any new members until after the conference.

## 2. Meeting Minutes

- a. Jamie Baffa asked for approval for the March 7<sup>th</sup> 2024 and March 30<sup>th</sup> 2024 board minutes
- b. **APPROVED:** Both March minutes as written
- c. In addition, the following offline votes were acknowledged:
  - i. The Board approved the 2024 budget on March 27, 2024, for a total operating budget of about -\$580k (net revenue of \$1,544k, expenses of \$2,125k). The approved budget demonstrates a conservative approach to revenue and dramatically scaled back programmatic investments for a fiscally prudent approach this year. Scaled-back initiatives and infrastructural support eliminated from this budget will only be possible through increased levels of donations, preferably unrestricted.
  - ii. The Board authorized BSF to:
    - 1. Execute agreement with NYU to work with the academic bioinformatics team to benchmark Barth natural history survival rates and generate publication for public domain (\$14,794)
    - 2. Negotiate consulting agreement with Amos Attali to focus business diligence on the legal, administrative, and financial framework necessary for the international Center of Expertise (CoE) to be established at Amsterdam Medical Center (\$20,000)
  - iii. Following the March 30<sup>th</sup> BSF grant call, affiliate grant contributions in 2024 as confirmed by their respective board of directors are:
    - 1. BARTH ITALIA ONLUS confirmed USD \$25,000 in support of David Stokes' grant at New York University.
    - 2. BARTH SYNDROME FOUNDATION OF CANADA agreed to provide \$15,000 to the same grantee (NYU).
    - 3. ASSOCIATION SYNDROME DE BARTH FRANCE confirmed support of \$50,000 to Patrick van der Wel at University of Groningen.

## 3. Treasurer Report

- a. Natalie Cohn reported on the organization's financial status through March 31, 2024, noting that contributions for the first quarter of the year reached \$150,000, with revenue at \$300,000 year-to-date, and total operating expenses at \$540,000. She also mentioned that the results include \$99k in unrealized

gains in the Investment Portfolio as a result of market appreciation.

- b. Natalie also stated that the organization's liquidity position is strong with approximately \$5 million in the investment portfolio of which approximately \$1 million is held in cash, including short-term Treasury investments.
- c. Emily Milligan commented that at this time in the year, we do not project revenue or expense results to be materially different from the approved budget for 2024.

#### 4. 2023 Financials and Audit Report

- a. Bill Belscher, Chairperson of BSF's Audit Committee, provided a high-level overview of the audit process, responsibilities of the audit committee, and the smooth completion of this year's audit.
- b. Kate opened the floor for comments from Palmer Lummis and Michelle Florez, Audit committee members, who both echoed Bill's sentiments on the smooth audit process.
- c. Ed Baltazar was delayed joining the meeting so the Board decided to advance to discussions about conference until he was able to join.

#### 5. 2024 International Conference

- a. Shelley Bowen presented the current status of conference registrations and reservations, highlighting 54 SciMed attendees, 181 family members, and a total of 253 attendees. Anticipated reaching 350 attendees.
- b. Shelley committed to reporting on number of first-time attendees in subsequent reports.
- c. Emily provided an update on the conference revenue, sponsorships, and pending asks. Nearly \$72,000 in confirmed sponsorships, with efforts continuing to meet or exceed the budget of \$165k.
- d. Miriam Greenberg commented on the successful review of the R13 NIH Grant, with acknowledgement to Lindsay Marjoram and Melissa Huang for their contributions. Federal funding from this source is a critical revenue line for financing the conference, and the BSF team is looking forward to hopefully receiving a notice of grant award which will confirm the funding amount.
- e. Emily reiterated that closing the gap between revenue and expenses remains an organizational priority to successfully mitigate the affordability of the conference (budgeted at a planned deficit of \$182k). The long-term ambition for future conferences remains to achieve net zero whereby revenue fully offsets the cost of this cornerstone event.

#### 6. 2023 Financials and Audit Report Continued

- a. Ed Baltazar from Dorfman joined the meeting and presented the 2023 Audited Financial Statements, confirming an unmodified (clean) opinion.
- b. Ed discussed key points including the balance sheet, statement of activities, statement of cash flows, and functional expenses. He noted no significant issues

or findings in the governance report.

- c. Ed agreed to incorporate suggested edits and send to the Board a clean copy, after the receipt of which Kate proposed the Board perform further review before a vote be taken.

#### 7. Family Services Travel Fund

- a. Shelley reported on the success of the travel fund, which has helped 10 families attend the conference. She proposed an additional \$10,000 plus \$1,000 administrative cost to support more families.
- b. Mark Greene clarified the scope of the additional funds and that they should be used exclusively to support the travel fund for families to the Conference.
- c. **APPROVED:** \$11,000 allocated from the Family Services restricted fund to the travel assistance fund.

#### 8. Clinical Trials and Human Subjects Research

- a. Melissa Huang presented 3 studies requiring invasive procedures as defined by BSF's Clinical Trials and Human Subjects Research policy.
  - i. Severe Neutropenia International Registry (Boston Children's Hospital): Registry was transferred from the University of Washington to Boston Children's Hospital. The study team requested BSF's administrative help communicating with patients and to share recruitment materials for re-enrollment of participants due to the study transfer. Study offers an optional skin biopsy or blood sample, hence the invasive definition, albeit minimal risk as defined by the institutional IRB.
  - ii. Profiling of monolysocardiolipins and cardiolipins from blood (Mayo Clinic): Collection of blood samples from adult and pediatric patients at the conference with the goal to develop a US-based diagnostic tool. This study was previously approved by the BSF Board, however the study team now proposes obtaining the blood samples in person by blood draw.
  - iii. Cardiolipin characterization from blood spots using Ion Mobility coupled to Tandem Mass Spectrometry: A validation study (University of Rijeka; Wayne State University): Collection of blood samples for validation of mass spec method for analyzing cardiolipin.
    - 1. Miriam Greenberg commented on the importance of the proposed study.
- b. Melissa noted that for the 2 studies requiring blood draws at the Conference, BSF is contracting a phlebotomist who will perform a single blood draw for the 2 different procedures, avoiding the need for people to undergo the procedure twice.
- c. **APPROVED** all three studies.

9. Therapy Fund Allocation

- a. Lindsay Marjoram recommended Dr. Leo Ferreira’s grant, previously approved by the BSF Board at the April meeting, be funded from the therapy development restricted fund due to its clinical relevance.
- b. **APPROVED** as proposed.

10. Gene Therapy Program Update

- a. Lindsay provided an update on the gene therapy novel capsid program, highlighting progress and funding requirements.
- b. Anticipated need ultimately of \$7-10 million over several years, with potential clinical trials starting late 2027 or early 2028 (best case scenario).

11. FDA Advisory Committee (Ad Comm) Preparation

- a. James Valentine, BSF regulatory counsel from HPM, discussed the FDA’s review process for the elamipretide New Drug Application (NDA), the importance of the upcoming advisory committee meeting anticipated later in 2024, and potential community involvement.
- b. James emphasized the need for a coordinated effort from the BSF community to support the review process.
- c. BSF team will coordinate with the patient community to identify potential patient and caregiver candidates for the upcoming FDA Advisory Committee meeting. The final selection of members will be made by the FDA.
- d. The FDA filed Stealth’s elamipretide NDA with “standard review.” Obtaining priority review status is critical to Stealth and to BSF, however, and both parties feel that it is entirely warranted. The importance of this designation, as well as plans to ask the FDA to reconsider this decision, were explained to the Board.

12. Other Business

- a. The team discussed the need for board members to sign annual disclosures, including the conflict of interest and confidentiality documents.
- b. **APPROVED:** In acknowledgement of Jamie Baffa having been voted in as Vice Chair of the BSF Board of Directors, the Board voted to appoint Brandi Dague to the Officer position of Secretary effective immediately.

13. The meeting was adjourned by Kate McCurdy at 2:02pm ET.

Respectfully submitted,

Brandi Dague

Secretary