

MINUTES

Barth Syndrome Foundation, Inc.

Board of Directors Meeting

November 2, 2023

Members in Attendance:

Andrew Buddemeyer	Board Member
Brandi Dague	Board Member
Emily Milligan, MPH	Board Member <i>ex officio</i> , Executive Director
Jamie Baffa	Board Member, Secretary
Kate McCurdy	Board Member, Chair
Mark Greene	Board Member, Treasurer
Peter van Loo	Board Member
Megan Branagh	Board Member
Michelle Florez	Board Member

Invited Guests

Edith Wolff	Consultant
Clive Spiegler	Consultant
Emily Madalinski	Development and Stewardship, BSF
Lindsay Marjoram, PhD	Incoming Director of Research, BSF
Melissa Huang, PhD	Clinical Research Coordinator, BSF
Natalie Cohn	Controller
Shelley Bowen	Director of Family Services, BSF
Steve McCurdy	Finance & Investments Committee

Members in Not Attendance:

BJ Develle	Board Member
Florence Mannes	Board Member
Jonathan Stokes	Board Member
Miriam Greenberg, PhD	Board Member
Nina Russell, MD	Board Member

The meeting, held via Zoom, was called to order by Kate McCurdy on Thursday, November 2nd at 12:02 p.m. ET.

1. Welcome
2. Meeting Minutes
 - a. Board agreed to postpone voting on the August minutes due to limited time to review

- b. Jamie Baffa noted the offline approval for Jess Lee’s scope of work to provide strategic communication support for the foundation from September through December 2023 for a total fee of \$20,000.
- 3. Treasurer Report
 - a. Natalie Cohn reviewed minor changes in the expense forecasts for the year
 - i. Salary expenses expected to decrease due to staff changes
 - ii. Savings are offset in part by spending in other areas, including consultants and other vendors to backfill staff departures
 - b. Emily Milligan reviewed progress against revenue forecasts (as of September 26th) for the year
 - i. Significant gap currently with major donors, though there is confidence that BSF will close this gap with year-end donations
 - ii. Difficult fundraising year for grassroots contributions. Staff cautioned that goal of \$130,000 for Giving Tuesday will be a stretch.
 - iii. BSF awarded \$70,000 for education and advocacy grant from Stealth, with funds received in Q4.
 - c. Mark Greene summarized that while we are \$50,000 behind forecast on revenue, BSF is \$160,000 ahead of forecast on a net operating basis compared to last forecast due to lower expenditures than expected
 - d. BSF’s balance sheet continues to be strong. Natalie and Mark noted that while BSF is monitoring cash flow, the foundation has liquidity in reserve and feels comfortable about its current position
 - e. Kate McCurdy reinforced the sound financial position of BSF while acknowledging that how the foundation spends money has evolved
- 4. Director of Research Welcome
 - a. Emily introduced Lindsay Marjoram as BSF’s new director of research and shared her perspective that Lindsay will be an excellent fit to lead BSF’s research and development portfolio and priorities
 - b. Kate reinforced enthusiasm for Lindsay’s hiring
- 5. Gene Therapy Update and Vote
 - a. Kate introduced Edith Wolff as BSF's Novel Capsid Gene Therapy project management consultant moving forward while thanking Clive Spiegler for the work he has done to date with BSF
 - b. Clive expressed his appreciation for the Foundation's support before sharing an update on Gene Therapy progress

- i. Recognized several key achievements in the novel capsid path since the last board meeting:
 - 1. Completed an external technical review with Dr. Gerry Cox and numerous expert consultants on September 22
 - 2. Facilitated a business development panel with industry discussion to gather feedback on commercial viability
 - 3. Onboarded a new project manager, Edith, to continue to lead this work for BSF
- ii. Dr. Bill Pu continues to work to optimize the novel capsid candidate
 - 1. A small technical hurdle was encountered by the Pu lab but Clive is confident it will be solved by year end; however, the delay means it is unlikely BSF can leverage GTRP funding for production of research grade vector
 - 2. Clive and Edith vetted vendors for research grade vector production and made recommendations for two vendors
- c. Two votes up for discussion during today's board meeting related to gene therapy
 - i. Funds to manufacture research grade vector to minimize the delay in starting proof-of-concept efficacy study once technical issue resolved and final candidate selected (up to \$27,500 –a 2024 expense)
 - ii. Assist in funding for a second year work to be conducted at Boston Children's Hospital through BSF's Strategic Initiatives Program to complete remaining candidate optimization work and conduct the pilot efficacy and transgene expression studies in mice (\$69,300)
- d. Steve McCurdy clarified that BSF's share of funding does not fully fund the project; Bill Pu has funds from other sources that will make up the balance
- e. Edith reinforced that projected costs for pre-IND work to develop manufacturing processes/vector for IND tox studies (\$415,000 +/- 15%) are an estimate and would be subject to negotiation; this large investment decision may well come before the BSF board for a vote in early 2024
 - i. All projected costs do not factor in large animal safety/tox work
- f. Steve reinforced that BSF does not have the money needed to fund all expenses on our own and emphasized the importance of finding partners to cost share
- g. Clive shared key learnings from the Business Development Panel Discussion
 - i. Low prevalence of Barth syndrome makes commercial viability unlikely (<2,000 patients is challenging for industry partners)

- ii. Will likely need to pursue non-commercial options
- iii. Panelists urged pushing for successful clinical activity for two to three Phase 1 participants to build momentum
- iv. Advised to follow others using the novel capsid to allow for regulatory review of the approach before presenting the FDA with a Barth syndrome candidate; also advised to consult with a regulatory specialist, especially if seeking expanded access / compassionate use program
- h. **APPROVED:** Funding to manufacture research grade material (up to \$27,500 out of the Therapy Fund) for proof-of-concept efficacy study
- i. **APPROVED:** Co-fund a second year of research and development work at Boston Children's Hospital (\$69,300 out of the Therapy Fund)

6. Elamipretide and Ultra-Rare Disease Advocacy

- a. The foundation's goal for its advocacy work is to convince the FDA to exercise its regulatory flexibility to give elamipretide a full, appropriate, fair, and equitable review
- b. Based on key opinion leaders' input, BSF's belief is that if there is a full and fair review, elamipretide will likely be approved
- c. Emily summarized BSF's advocacy approach with Congress, FDA, and the media, reinforcing how involved and intense of an effort this has been for staff and for the families involved over the past several months
- d. Kate conveyed the value of this work extends beyond the intended outcome of an FDA product review. BSF has built strong relationships with influential regulatory and congressional leaders, and institutional knowledge has expanded across staff and volunteers through the experience gained doing this type of work that is expected of patient advocacy groups in life science drug development
- e. Emily noted that we, in conjunction with affiliates, are also beginning conversations in Europe with Pharmanovia regarding elamipretide

7. Votes: BSF Policies

- a. **APPROVED:** Expenditures and Commitments Authorization Policy, as written
- b. **APPROVED:** Executive Committee Policy, as written
- c. Emily noted that recent staff transitions highlighted inconsistencies between the employment policy and employee handbook
- d. At the advice of BSF's HR counsel, staff recommends that BSF remove its employment policy and maintain the employee handbook to set expectations for staff
- e. **APPROVED:** Removal of the Employment Policy
- f. **APPROVED:** Clinical Trials and Human Subjects Research Policy, as written

8. Update: Amsterdam Medical Center (Center of Expertise)
 - a. Emily reviewed the current state, with two existing BSF centers of expertise at Kennedy Krieger Institute, Maryland, and Bristol, England, but no European Union center
 - b. Shelley Bowen traveled to Amsterdam to meet with the team in May 2023 and understand complexities to set up a center of expertise at the Amsterdam Medical Center
 - c. BSF must first have full line of into the costs and benefits before making a long-term commitment to launching a new Center of Expertise (COE)
 - d. Amos Attali, who applied for the director of research role, has emerged as a potential and well-suited, local resource for undertaking diligence. BSF is in discussions with Amos to contract him to lead a planning process for the COE
 - e. Emily forecasted, that while a focused scope of work, hiring of a consultant will not be an inexpensive endeavor. It is a necessary step, though, pending negotiations and advisement from legal for Dutch-specific hiring, to facilitate a BSF decision about downstream, necessary support for a successful COE. Anticipated vote in December for the board to contract with Amos to lead the planning for the COE
9. Giving Tuesday
 - a. Kate reminded the board the importance of Giving Tuesday to our overall fundraising efforts
 - b. Set a goal for the board collectively to commit to a \$10,000 goal for Giving Tuesday and requested each board member to inform Emily of their planned donation amount
 - c. Reinforced the real importance of 100% board participation to rally additional donations
10. Update: Research Grants
 - a. BSF's grant application window closed on October 31, and Melissa Huang provided a brief update on grant applications
 - b. 12 applications for the 2024 Grant program
 - i. Six are international
 - ii. Six are young investigators
 - iii. Three are first time applications
 - c. Two applications for the Iris L. Gonzalez Prize
11. Adjournment
 - a. Kate adjourned the meeting at 2:00 p.m. ET

Respectfully submitted,

Jamie Baffa – Secretary