

MINUTES

Barth Syndrome Foundation, Inc.

Board of Directors Meeting

February 17, 2023

Members in Attendance:

Brandi Dague	Board Member
Emily Milligan	Board Member <i>ex officio</i> , Executive Director
Jamie Baffa	Board Member, Secretary
Kate McCurdy	Board Member, Chair
Kevin Woodward	Board Member, Treasurer
Megan Branagh	Board Member
Michelle Florez	Board Member
Miriam Greenberg, PhD	Board Member
Nina Russell, MD	Board Member
Peter van Loo	Board Member

Invited Guests

Brett Smith	Operations and Communications, BSF
Emily Madalinski	Development and Stewardship, BSF
Erik Lontok, PhD	Director of Research, BSF
Melissa Huang, PhD	Clinical Research Coordinator, BSF
Michael Bowen	Policies & Agreements Committee
Natalie Cohn	Controller
Shelley Bowen	Director of Family Services, BSF
Steve McCurdy	Finance & Investments Committee

Members in Not Attendance:

Florence Mannes	Board Member
Maryanne Chrisant, MD	Board Member
Andrew Buddemeyer	Board Member
B.J. Develle	Board Member
Mark Greene	Board Member

AGENDA

1. *Welcome*
2. *Vote: December Minutes*
3. *Update: Treasurer Report (2022 Preliminary Financials)*
4. *Vote: 2023 Strategic Priorities*
5. *Vote: Arrhythmia Retrospective Study*
6. *Vote: Stronger Together 2023 World Tour (Great Wolf Lodge Contract)*

7. *Vote: Emergency Relief Program (Proposed changes to eligibility requirements)*
8. *Vote: Communication Consultants (Advocacy / Therapy Development Communications)*
9. *Vote: AMC Center of Expertise*
10. *Update: TransCellular Therapeutics (TCT)*
11. *Vote: 2023 Budget*
12. *Vote: SMAB and Board Renewals*

The meeting, held via Zoom, was called to order by Kate McCurdy on Friday, February 17, 2023, at 12:04 p.m. ET.

1. Welcome
2. Vote: December Minutes
 - a. Jamie Baffa asked for final input and approval on BOD minutes from December
 - b. **APPROVED:** December BOD minutes
3. Update: Treasurer Report (2022 Preliminary Financials)
 - a. Natalie Cohn shared a summary of the 2022 preliminary financials
 - i. \$1,358,241 in operating revenue
 - ii. \$ 1,367,327 in operating expenses
 - iii. Does not reflect approximately the \$700,000 unrealized decrease in value of BSF's investment portfolio due to overall market downturn
 - b. Kate McCurdy highlighted that BSF's operations were close to breaking even, despite nearly \$300,000 invested in strategic initiatives
 - c. Natalie noted that revenue patterns and operating expenses were largely consistent with past years and expectations
 - d. Emily Milligan thanked Megan Branagh for her efforts on Happy Heart Week
 - i. Noted that Happy Heart Week will not have an in-person event in 2023, meaning revenue from this event will likely be lower this year
 - e. Giving Tuesday exceeded expectations and several major donors came through with larger gifts than usual
 - f. Affiliate contributions included \$100,000 from Barth France that is earmarked for the cardiac natural history registry and arrhythmia study
 - g. \$280,417 was spent on strategic initiatives against an expectation of \$572,500, with much of the difference rolling over to 2023

- h. Natalie noted that BSF is in the midst of its 2022 audit, with no adjustments or issues at this time
 - i. Final financials will be available following the audit, however, few changes are expected
 - j. Emily noted that due to the carry over of certain strategic investments into 2023, BSF expects to run a deficit of \$325,000 for 2023
4. Vote: 2023 Strategic Priorities
- a. Emily reviewed 2023 strategic priorities (some of which are new and some of which are carryovers or enhancements on 2022 goals)
 - i. Research and Development
 1. Launch retrospective arrhythmia project with initial datasets captured for the cardiac natural history registry
 2. Decide on advancement of gene therapies
 3. Deploy research GUIDs, with 40% of US-based population enrolled
 4. Support the development of a US-based diagnostic and/or access to current diagnostic testing
 5. Support the development of a new Center of Expertise in Europe to expand patients' access to comprehensive clinical care and offer additional site for future clinical studies
 6. Maintain our discovery research and tool efforts (including BSF seed grant program)
 7. Merge the gene variant database with a sustainable solution, and identify intermediate and long-term solutions for other BSF assets such as biorepository and/or registry
 8. Broaden BSF's network of experts to address unmet clinical and research challenges
 9. Launch R&D group to advise BSF R&D strategy, inform portfolio management decisions, and ensure a robust and diverse pipeline
 - ii. Advocate for Our Community

1. Exhaust all ethical means to guarantee Barth families, in consultation with their treating physician, can access elamipretide and future potential therapies if desired
 - a. Highlighted the importance of having access to therapies “if desired” and the goal for individuals and families to have the ability to make decisions for themselves to access treatments
 2. In collaboration with FDA and BTHS key opinion leaders, inform guidance on regulatory pathways for potential therapies targeting cardiac outcomes in Barth syndrome
 3. Ensure that the value BSF creates through various non-scientific programs (eg, website, listserv) is translated into marketable sponsorships
 4. Improve quality and frequency of donor touchpoints with goal of increasing retention and growth
- iii. Support Barth Families
1. Increase Barth families’ access to BSF’s Emergency Relief Program
 2. Advance the field and/or clinical guidance around feeding, nutritional, and/or digestive aspects of BTHS
 3. Identify activities to engage Barth teens in program development and delivery
 4. Offer in-person engagement at STWT events to strengthen and support the BTHS community in an off-conference year
 5. Restructure website content to improve visitor experience
 6. Obtain and implement a communications expert’s input to better reach our constituency, especially to keep them informed of mission-critical initiatives
 7. Implement a secure solution that will provide a broadly accepted way to connect the global Barth community
 8. Provide encouragement and support during the most trying of times and celebrate milestones and accomplishments with the help of the Barth Cares committee
- iv. Never, Ever Give Up!

1. In concert with peer organizations, opportunistically identify and implement > 3 novel educational and/or research initiatives
2. Increase the number of volunteer leaders who commit to lending their time and talents to furthering BSF's mission
3. Position BSF for a successful 2024 international scientific, medical, and family conference
4. We are resilient and will evolve our strategic plan for a 3-year horizon
 - a. Noted that BSF's strategic plan expires in 2023 – will need to revisit

5. Vote: Arrhythmia Retrospective Study

- a. Emily shared a brief primer on the objective and history of the arrhythmia project
- b. Board had previously taken a contingent vote to approve, pending the finalization of the agreement between BSF and NYU
- c. Emily reviewed highlights of the agreement
 - i. Creates a steering committee consisting of BSF representation and representatives from each institution involved in the study
 - ii. Requires data to be de-identified, using the GUID
 - iii. Determined that BSF has only non-exclusive, non-commercial, sub-licensable license to use data for research purposes connected to Barth syndrome
 - iv. Milestone agreement, distinct from BSF's grant awards
 1. Total value of contract is \$272,783, but expenses will only be realized upon sites achieving contracted milestones
- d. Expected expense for 2023 is \$114,599, but seeking approval for the full contract amount according to the proposed milestone schedule
- e. **APPROVED:** Commit BSF up to \$272,783 for the full value of the agreements associated with the arrhythmia project

6. Vote: Stronger Together 2023 World Tour (Great Wolf Lodge Contract)

- a. Emily reviewed survey results from the STWT feedback survey

- b. Proposal is to host STWT stops at the same four locations (Scottsdale, AZ, LaGrange, GA, Fitchburg, MA, and Gurnee, IL) in 2023 based on a combination of participant feedback and costs
 - c. Michael Bowen reviewed the budget for 2023 in comparison to the final costs for 2022
 - d. Contract highlights and considerations
 - i. "No attrition" room block, same as 2022
 - ii. Potentially committing BSF to a higher food and beverage minimum (\$12,000 in 2022 and \$20,000 in 2023) based on actual spending (\$24,000) in 2023
 - iii. Contract cancellation charges to only come into effect if the cancellation is 30 days or less prior to the first event
 - e. Salem Oaks consulting fees (\$5k) to triple, driven by a more comprehensive program that will require two people from their team to facilitate
 - f. Michael noted that 2023 estimates are based on the number of families that attended in 2022, and that they could change based on higher or lower participation numbers across each site
 - g. Emily noted that BSF expects the NORD travel grants may be more restrictive than in 2022
 - h. **APPROVED:** BSF to spend up to \$75,000 to support 2023 STWT
7. Vote: Emergency Relief Program (Proposed changes to eligibility requirements)
- a. Shelley reviewed the background on the Emergency Relief Program and how it works
 - b. Goal is to offset some duress families are experiencing due to economic strife as it relates to caring for or living with Barth syndrome
 - c. Emily acknowledged that care management can include a multitude of services, including mental health, vision, dental and time away from work for healthcare visits
 - d. Proposal is to remove "hospitalization and / or qualifying event" as an eligibility requirement
 - e. **APPROVED:** "Hospitalization and / or qualifying event" is no longer required to apply for the emergency relief program

8. Vote: Communication Consultants (Advocacy / Therapy Development Communications)

- a. Kate reviewed the proposal from On Time Medical Communications to write a white paper in partnership with the FDA on the path forward for ultra rare diseases (\$12,000 to \$15,000 in fees)
- b. Kate expressed hope that BSF would secure grant funding to offset these fees and associated expenses to the foundation
- c. Emily clarified that this consulting firm comes recommended from Stealth. To expedite the procurement process, BSF did not bid out this work. However, there is no concern about conflicts of interests, as the contracting relationship is directly between BSF and the firm.
- d. **APPROVED:** Proposal from On Time Medical Communications up to \$15,000
- e. Emily reviewed a proposal from Nancy Intrator, a communications consultant with prior experience at the Muscular Dystrophy Association, to advise on the communication related to BSF's transition from research grant support toward broader therapy development (\$3,000 in fees)
- f. Highlighted her specific experience in communicating about Gene Therapy
- g. Forecasted that this work will influence a potential capital campaign
- h. **APPROVED:** Scope of Work for Nancy Intrator (\$3,000)

9. Vote: AMC Center of Expertise

- a. Erik Lontok reviewed the background and goal for establishing the Center of Expertise at Amsterdam University Medical Center
- b. This center will make it easier for BSF to conduct clinical trials outside of the U.S. and U.K.
- c. Proposal is to fund \$8,000 in travel expenses for BSF staff to help establish the Center of Expertise in line with local needs and context
- d. **APPROVED:** \$8,000 in travel expenses for BSF staff

10. Update: TransCellular Therapeutics (TCT)

- a. Emily reminded the Board that BSF tentatively approved funds in December 2022 to support further work on enzyme replacement therapy development from Barth syndrome if NIH approves transition to an R33 award for TCT
- b. Erik shared that Dr. Chin is beginning the final steps needed before testing enzyme replacement therapy in BSF mice

- c. Steve McCurdy noted that BSF, with pro-bono advisement from Kirkland Ellis, is negotiating an agreement between BSF, Dr. Chin, and the University of Washington to preserve BSF's interests downstream and in the event that TCT is acquired

11. Vote: 2023 Budget

- a. Emily reviewed the detailed 2023 budget
- b. Highlighted that the budget includes funds to hire a grant writing firm to assist BSF
- c. Net deficit would be projected at \$330,000 due to an additional \$10,000 approved for STWT during this BOD meeting
- d. Reviewed revenue forecasts
- e. Revised Giving Tuesday budget from \$125,000 to \$130,000 to reflect anticipated consistent performance year-over-year, 2022 vs 2023
- f. **APPROVED:** 2023 budget with discussed amendments

12. Vote: SMAB and Board Renewals

- a. SMAB Members (four members whose four-year terms are up in April)
 - i. Grant Hatch is retiring, and declined an additional term
 - ii. John Lynn Jefferies is committed to serving another 4-year term if approved
 - iii. Colin Steward is committed to serving another 3-year term if approved
 - iv. Hilary Vernon is committed to serving another 4-year term if approved
- b. **APPROVED:** John Lynn Jefferies, Colin Steward, and Hilary Vernon, approved to extend their membership on the SMAB
- c. Board Members
 - i. Due to timing, the BOD lacked the quorum necessary to make board elections

13. Adjournment

- a. Kate adjourned the meeting at 2:09 p.m. ET

Respectfully submitted,

Jamie Baffa – Secretary