

## MINUTES

Barth Syndrome Foundation, Inc.

Board of Directors Meeting

September 12, 2022

### **Members in Attendance:**

Andrew Buddemeyer	Board Member
B.J. Develle	Board Member
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
Mark Greene	Board Member
Maryanne Chrisant, MD	Board Member
Megan Branagh	Board Member
Michelle Florez	Board Member
Miriam Greenberg	Board Member
Nina Russell, MD	Board Member

### **Invited Guests**

Bill Belscher	Audit Committee, Chair
Brett Smith	Operations and Communications, BSF
Erik Lontok	Director of Research, BSF
Melissa Huang	Clinical Research Coordinator, BSF
Steve McCurdy	Finance & Investments Committee

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

Florence Mannes	Board Member
Peter van Loo	Board Member

### **AGENDA**

1. *Welcome*
2. *Update: Gene Therapy*
3. *Update: Arrhythmia Retrospective Study and BTHS Natural History Project*
4. *Vote: North Star Review Board IRB*
5. *Update: Regulatory Affairs*
6. *Update: #Stronger Together World Tour*
7. *Update: Treasurer Report*
8. *Update: Human Resources*
9. *Vote: Board Minutes*
10. *Other Business*

The meeting, held via Zoom, was called to order by Kate McCurdy on Monday, September 12 at 12:04 p.m. ET.

1. Welcome
  - a. Kate McCurdy welcomed board members and invited guests
  - b. Kate formally recognized Melissa Huang's successful dissertation defense and the awarding of her doctorate
2. Update: Gene Therapy
  - a. Erik Lontok reviewed the conversation the board had in February regarding gene therapy and the two paths available to BSF
  - b. In July, Barth syndrome was one of 14 diseases invited to apply to the Bespoke Gene Therapy Consortium (BGTC)
  - c. However, BSF was told that Barth syndrome was the only disease for which two independent applications (one from the University of Florida and one from Boston Children's Hospital) were submitted, which we were instructed is not ideal
  - d. BSF convened a meeting with both research teams to reconcile the applications, and all parties are now collaborating on how to put forth the best, single BGTC application supported by BSF in advance of the 10/31 deadline
  - e. Emily Milligan reviewed various scenarios of funding gene therapy programs currently in play and would denote a substantial financial commitment by BSF. The landscape remains highly dynamic, and BSF continues to try to define partnering opportunities that would both co-fund the initiative and in parallel de-risk technical hurdles, such as the immune response to a gene therapy.
  - f. One possible industry partner is willing to fund a significant amount of a Ph I/II trial if BSF doesn't receive BTGC funding, however, their preference is to start in Q4 2022 and would ideally require > \$500k co-funding commitment from BSF. There are also questions as to whether the proposed budget will cover all expenses associated with the trial, which poses a risk to the program.
  - g. If we receive the BTGC funding, BSF may be asked to fund some supplemental travel costs for patients and families or to support the costs of having additional patients participate in the program
  - h. After speaking with Dr. Barry Byrne, BSF expects to commit \$300k to cover these costs
  - i. Kate then forecasted that while there is no vote today, we will likely need to take a vote in October regarding financial investments
  - j. Jamie Baffa asked when BSF would expect a BGTC decision, and Emily shared that she expects it sometime in Q2 2023
  - k. Additional discussion followed
3. Update: Arrhythmia Retrospective Study and BTHS Natural History Project
  - a. Erik reviewed the background on the Arrhythmia Project

- b. BSF is nearing the execution of contracts, with the goal of having both coordinating and participating site contracts finalized by the October board meeting
  - c. Erik reviewed the importance of getting the Global Unique Identifier (GUID) correct with this work
4. Vote: North Star Review Board IRB
- a. Melissa then reviewed the board's previous vote to agree to terms with a new IRB vendor
  - b. That vendor has since been acquired, moving BSF to revisit this decision and is now recommending that BSF contract with North Star Review Board
  - c. Melissa shared North Star Review Board's qualifications and costs:
    - i. North Star seems well-qualified and suited to handle the IRB work that will be needed by BSF
    - ii. \$2,000 in top line costs to contract with North Star, along with a \$750 annual fee
    - iii. There is a consulting fee of \$250, if needed
    - iv. The costs are significantly lower than the original provider BSF was to contract with
  - d. Discussion followed
  - e. **APPROVED:** BSF to move forward with contracting with North Star Review Board
5. Update: Regulatory Affairs
- a. Kate shared that there have been two FDA meetings since the last board meeting
    - i. BSF held a workshop with the FDA on 7/29 to further discuss Barth syndrome as an ultra-rare disease and to brainstorm about possible clinical endpoints, trial design features and regulatory pathways forward for Barth syndrome therapy development
    - ii. Stealth had a meeting with the FDA on 8/4
  - b. Stealth is currently working on their response to the meeting
  - c. There is a public meeting on 9/15 hosted by STAT about unique challenges of regulatory affairs for rare disease drug development, sponsored by Alexion, focused almost exclusively on Barth
  - d. There is a public FDA meeting on 9/20 during which Emily will speak about the need for appropriate regulatory flexibility for Barth syndrome products.
  - e. Discussion followed
6. Update: #Stronger Together World Tour
- a. Melissa shared a recap of data and insights from #STWT, including participants, average ages, and affiliation to BSF.
    - i. STWT has offered 12 families their first face-to-face

- ii. Breakdown by location: 46 Gurnee, 38 LaGrange, 35 Scottsdale, and 33 Fitchburg
    - iii. ~60% family members, ~20% affected individuals, ~15% advocates, with staff rounding out attendance
    - iv. Overall, feedback has been exceptionally positive
  - b. Emily reviewed the budget impact of #STWT
  - c. Overall, BSF is under budget for these events, however, fundraising off of them has been a challenge
  - d. Affiliates have not asked BSF to help offset their costs, though we expect to provide the Netherlands with some assistance
  - e. BSF has helped families find close to \$7,000 in travel funding through NORD
  - f. Discussion followed
- 7. Vote: Board Minutes
  - a. Jamie indicated that the June minutes would need to be amended to include the offline Mayo Clinic vote
  - b. No other changes suggested
  - c. **APPROVED:** June minutes, pending the addition of the offline Mayo Clinic vote
- 8. Update: Treasurer's Report
  - a. Natalie reviewed the financials in detail and shared that BSF is in a stronger position compared to a year ago
  - b. Emily noted that we removed the expected funding from Barth France (\$75,000) from the forecast as it is coupled with the launching of the Arrhythmia Project and so likely will not happen this year
    - i. If this is not realized, there will also be no cost associated with it
  - c. Emily noted that NIH Conference Funds for our 2020 conference are being pulled through to cover expenses for this year's SciMed symposium, adding \$30,000 to our revenue forecast
- 9. Update: Human Resources
  - a. Emily shared an update on the search for a Development and Stewardship Manager
  - b. Received over 45 CVs, conducted nine interviews, and BSF has an offer out to a candidate
  - c. The goal is to bring someone onboard in early October so they are able to assist with Giving Tuesday on 11/29
  - d. Emily also noted that BSF is looking for ways to support grant writing as well
  - e. Kate then reiterated two significant changes in the budget and finances
    - i. Postponements to strategic initiatives, such as the Arrhythmia Project, affect the budget

ii. BSF's investment portfolio is down this year, however, it is down less than the market overall given the relatively conservative portfolio

f. Discussion followed

g. Emily shared that BSF secured a significant donation from a family foundation due to publicity from Happy Heart Week and expressed thanks for Megan Branagh's efforts

h. BSF also received a \$40,000 grant from Stealth to fund regulatory education

10. Other Business

a. Kate dismissed those non-board members in the meeting and called an executive session.

11. Adjournment

a. Kate McCurdy adjourned the meeting at 2:02 p.m. ET

Respectfully submitted,

Jamie Baffa – Secretary