

MINUTES

Barth Syndrome Foundation, Inc.

Board of Directors Meeting

October 20, 2021

Members in Attendance:

Kate McCurdy	Board Member, Chair
Emily Milligan	Board Member <i>ex officio</i> , Executive Director
Kevin Woodward	Board Member, Treasurer
Jamie Baffa	Board Member, Secretary
Florence Mannes	Board Member
Michelle Florez	Board Member
Nina Russell, MD	Board Member
Brandi Dague	Board Member
B.J. Develle	Board Member
Megan Branagh	Board Member
Maryanne Chrisant, MD	Board Member

Invited Guests

Shelley Bowen	Director of Family Services, BSF
Brett Smith	Operations and Communications, BSF
Erik Lontok	Director of Research, BSF
Bill Belscher	Audit Committee, Chair
Stacey Woodward	Fundraising Committee, Chair
Mark Greene	Finance and Investments Committee
Susan McCormack	Policies and Agreements Committee

Members in Not Attendance:

Andrew Buddemeyer	Board Member
Peter van Loo	Board Member

AGENDA

1. *Discussion on Committee Initiatives*
 - a. *Finance & Investments*
 - b. *Fundraising*
 - c. *SMAB*
2. *Discussion: Treasurer Update on 2021 Financials*
3. *Vote: BSF Retirement Benefit Program*
4. *Focus Topic: BSF Research & Development*

5. *Vote: BSF R&D Strategy*
6. *Vote: Arrhythmia NYU Project*
7. *Discussion: 2022 Conference*
8. *Update: FDA Interaction*

The meeting, held via Zoom, was called to order by Kate McCurdy on Wednesday, October 20 at 12:00 p.m. ET.

1. Welcome
 - a. Kate McCurdy welcomed everyone and called the meeting to order at 12:00 p.m. ET
 - b. Kate then shared that this morning the FDA declined to review (issuing a “refusal to file” letter) Stealth's NDA and the published rationale for FDA's rejection
 - i. FDA reiterated its commitment to working with Stealth on elamipitride as a treatment for Barth syndrome
 - ii. Stealth can request a Type A meeting to get further clarification from FDA
 - iii. Next step could be to "file over protest," which is the first step to an appeal
 - iv. Highly unlikely that elamipitride would be approved through this route, but not impossible
 - v. Stealth is considering this option
 - c. Kate was told by Reenie McCarthy (Stealth CEO) that the seven individuals who are currently on drug will be able to continue to receive it under an expanded use protocol
 - i. Stealth may try to get additional affected individuals on drug in a few months under the expanded use protocol
 - ii. Stealth cannot guarantee the drug will be manufactured in the future
 - d. Kate reiterated the challenge of getting regulatory approval as an ultra-rare disease
 - e. Emily shared that the appeals process is an expensive one for Stealth to pursue, which may affect their decision-making
 - f. Emily also forecasted that BSF will likely release a public statement tomorrow, expressing severe disappointment with the decision
 - g. Also confirmed that all patients on drug have been contacted by Hilary Vernon
 - h. Dr. Chrisant raised the possibility of a coordinated publicity campaign
 - i. Discussion followed
 - i. Next, Kate moved to vote to approve minutes from previous meetings with several minor revisions
 - j. **APPROVED:** Minutes from August 2021 board meeting, pending discussed revisions
 - k. Before shifting to Committee Updates, Kate confirmed that, since the last board meeting, the board had granted approval by email vote to hire a Gene Therapy consultant for \$10,000
2. Committee Updates
 - a. Fundraising Committee (Stacey Woodward)
 - i. Cabo sweepstakes is underperforming at the moment
 1. Reiterated that board and staff can purchase tickets on behalf of others

2. Stacey forecasted a big push this week so we can meet the \$5,000 threshold
3. Purchased some Facebook advertising that backfired, resulting in free entries
- ii. Giving Tuesday
 1. Christy is continuing to support our communications efforts here
 2. Stacey asked for the board to offer a match for Giving Tuesday
 3. Goal is to raise \$100,000 overall
- iii. Swag Shop
 1. Going well so far
 2. Creating an incentive tied into Giving Tuesday for those who create a page and raise at least \$25 dollars
- iv. Hiring of full-time development and stewardship coordinator
 1. With Christy's departure, Stacey reinforced the need for someone connected to staff to serve as a liaison and keep our efforts coordinated
 2. Adding this staff member and one or two more volunteers could give us opportunities to greatly expand our fundraising efforts
- v. Kate reinforced the value of having every single board member participate in the board match for Giving Tuesday
- vi. Proposal made for the board to make a \$10,000 match for Giving Tuesday
- vii. **APPROVED:** Board agreed to offer a \$10,000 match for Giving Tuesday in 2021
- b. Finance & Investments (F&I) Committee (Kevin Woodward)
 - i. Committee has been focused on three areas
 1. Current financials for the organization
 2. Staff programs tied to the financials, most recently Insperty and the forthcoming staff 401k proposal
 3. Analyzing BSF's cash flow and the investment allocations that are appropriate for the foundation
 - ii. Kate called attention to #8 in the F&I committee minutes
 1. Clarified that items only hit our books when the board approves the decision AND the contract is signed
 2. Emily offered to amend this item in the minutes and clarify
- c. Scientific and Medicine Advisory Board (Kate McCurdy)
 - i. Kate proposed that we re-align appointments to the SMAB to begin and end in April – the current timeline is difficult given our grant review cycle
 - ii. This will align terms for the SMAB with the rest of the board and our committees
 - iii. **APPROVED:** We will realign appointments to the SMAB so the terms begin and end in April
3. Treasurer Update (Kevin Woodward)
 - a. Reviewed current performance against budget
 - b. Reinforced that we expect to run a deficit this year

- i. Tracking ahead on donations compared to prior years
 - ii. Expenses are tracking lower than expected
 - iii. In a strong position against our plan
 - c. BSF Retirement Plan Proposal
 - i. Kevin introduced Mark Greene and provided details on his background and role on the Finance & Investment committee
 - ii. Mark shared context regarding why we are looking at offering a retirement plan—it's about attracting and retaining staff who are committed to our mission
 - iii. Moving to Insperity early this year created additional opportunity to explore offering a retirement plan
 - iv. This committee is proposing that we offer a retirement plan through Insperity that includes an employer match of up to 4%
 - v. Goal is to be competitive with other non-profit and for-profit organizations
 - vi. Recommendation summary
 - 1. Would begin in January 2021
 - 2. Annual costs at \$500, plus \$2.50 per month per enrollee with a balance
 - 3. 100% match on first 3% of employee eligible contributions and 50% match on the next 2%
 - 4. Participants pay administrative fee of \$4 per month from the assets within their account
 - vii. Discussion ensued
 - viii. **APPROVED:** BSF to offer a staff retirement benefit as proposed
- 4. Research and Development Pipeline (Erik Lontok)
 - a. Erik reviewed the status of various post-discovery research and development efforts
 - b. Goal of our approach is to increase the number of shots on goal so we can increase our chances of delivering therapies to affected individuals
 - c. Reviewed our dual approach of supporting both Discovery Research and Disease Management Research
 - d. Reviewed the research question and history of the Arrhythmia Project
 - i. Specific program goal is to understand the risk of arrhythmia and prevalence across affected individuals
 - ii. Additional organizational goal is to design a system, contracts and establish a database for collecting clinical data from multiple institutions in a way that will support the creation of a natural history for Barth syndrome
 - e. Reviewed the overall research proposal
 - i. Year One will be focused on a registry and retrospective study
 - 1. Will have a coordinating center at NYU Langone that will primarily be responsible for medical record abstraction
 - 2. Kennedy Krieger Institute in Baltimore (KKI) and University of Pittsburgh Medical Center (UPMC) will supply primary clinical data
 - a. 25-30 participants at KKI
 - b. 8-10 participants at UPMC

3. Will also need to engage local providers
 - ii. BSF Clinical Research Coordinator
 1. Would be tasked with implementing, tracking, and maintaining BSF's Global Unique Identifier (GUID) program which will span across all competitive and noncompetitive research efforts and assets
 2. Goal is to have bring together Barth syndrome datasets from around the world
 - iii. What we are truly building is a natural history of Barth syndrome with arrhythmia as a pilot
 - iv. Emily reinforced the challenge of doing this and how important this effort is for BSF, especially when it comes to bolstering our ability to facilitate clinical trials
 - v. Erik reviewed budget implication, with an overall first year cost of \$170,045 plus an additional \$65,000 for a Clinical Research Coordinator
 - vi. Erik shared the Arrhythmia Prospective Study which reflects our long-term plans
 1. Subgroup aims to access data in already implanted cardiac devices and recruit additional volunteers for remote arrhythmia monitoring
 - f. Kate reinforced that there is no one else in the world who would fund this effort to create the infrastructure for a database that will underpin a natural history for Barth syndrome and BSF is in a unique position to do this
 - g. Emily reinforced that this would require long-term, ongoing investment
 - h. Discussion followed
 - i. **APPROVED:** Initial investment for the Arrhythmia Project and the hiring of a clinical research coordinator
 - j. Florence shared that Barth France is funding this effort at 100,000 Euros (currently about \$120,000) a year for at least five years
5. Development and Stewardship Manager
 - a. Emily reinforced the need and opportunity for someone in this role and that this role needs to include engagement with corporate sponsorship opportunities
 - b. **APPROVED:** BSF to move forward with hiring a Development and Stewardship Coordinator
6. 2022 Conference
 - a. Emily suggested that we would schedule an ad hoc board meeting to discuss plans for the 2022 Conference and shared issues and considerations on her mind
7. Adjournment
 - a. Kate McCurdy adjourned the meeting at 2:06 p.m. ET with the understanding the board would schedule an ad hoc meeting to discuss the 2022 conference

Respectfully submitted,

Jamie Baffa – Secretary