MINUTES
Barth Syndrome Foundation, Inc.
Board of Directors Meeting
October 20, 2021

Members in Attendance:
Kate McCurdy  Board Member, Chair
Emily Milligan  Board Member ex officio, 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 FDAs 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 Insperity 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

d. 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