

MINUTES
Barth Syndrome Foundation, Inc.
Board of Directors Meeting
February 22, 2022

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
Andrew Buddemeyer	Board Member
Peter van Loo	Board Member

Invited Guests

Brett Smith	Operations and Communications, BSF
Erik Lontok	Director of Research, BSF
Melissa Huang	Clinical Research Coordinator, BSF
Loree Tillman	Development and Stewardship Manager, BSF
Natalie Cohn	Controller
Clive Spiegler	Gene Therapy Consultant
Steve McCurdy	Finance & Investments Committee
Bill Belscher	Audit Committee, Chair

Members in Not Attendance:

Maryanne Chrisant, MD	Board Member
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AGENDA

1. *Welcome*
2. *Update on 2021 Full-Year Financials*
3. *Discussion and Vote: 2022 Possible Strategic Investments*
4. *Discussion and Vote: Full 2022 Budget*
5. *Discussion: Board Self-Assessment*
6. *Update: Drug Development Advocacy*
7. *Discussion and Vote: SMAB Members for Additional Terms*

8. *Discussion and Vote: Board of Directors Candidates*
9. *Discussion and Vote: Additional Terms for Current Board Members*

The meeting, held via Zoom, was called to order by Kate McCurdy on Monday, February 22, 2022 at 12:04 p.m. ET.

1. Welcome
 - a. Kate McCurdy welcomed Loree Tillman to BSF as a consultant serving as the foundation's Development and Stewardship manager, helping especially with several specific projects
 - b. Kate then explained changes in how the board will operate regarding certain administrative votes and tasks
 - i. Recognition that board meetings are quite full and there is an opportunity to shift some more procedural agenda items to email vote
2. Update on 2021 Full-Year Financials
 - a. Kevin Woodward shared a summary of the DRAFT 2021 financials
 - i. BSF ended the year with a surplus of \$433k
 - ii. Because we had budgeted a nearly \$200k deficit, we actually came in \$627k over budget, which is better than had been projected
 - iii. From a revenue standpoint, some of this was driven by select major donors giving at a higher amount than usual, however, we do not expect these increases to repeat themselves in the future
 - iv. Our investment portfolio also did quite well, even though we were heavily invested in fixed income / bonds
 - v. We had projected \$1.1 million in revenue and finished with closer to \$1.5 million
 - vi. We also spent less last year than expected, however, this is largely driven by deferring arrhythmia project expenses to 2022
 - vii. Natalie Cohn then shared that these numbers are not final and we can expect small changes as we finalize 2021 expenses and revenues
 - viii. Kevin explained that we will not review year to date numbers in the interest of time and because we don't yet have an approved budget
3. Discussion and Vote: Possible 2022 Strategic Investments
 - a. Emily Milligan reviewed BSF's strategic plan and how it should guide our decisions regarding where and how to invest, and then encouraged board members to weigh in with questions and feedback
 - b. Emily reviewed the overall list of strategic investments
 - c. These investments total \$572.5k above operating expenses, however we do expect some offsets and some of this will be carried over from last years' budget

- d. Kate clarified that the \$113k revenue potential for the arrhythmia project reflects the pledge from Barth France
- e. Discussion then shifted to a detailed review of strategic investments as aligned to BSF's strategic plan
 - i. Invest in R&D
 - 1. Launching the natural history study and retrospective multi-site Arrhythmia Project
 - a. Contracting has been a challenge, though Emily said she'd like to have a contract ready for the board to approve soon
 - b. In addition to \$270k this year, would require an investment of more than \$300k in 2023
 - 2. Continue with our seed grant program
 - a. \$200k for 2022 that is already captured in our operating budget
 - b. Flat compared to last year
 - 3. Progress at least one gene therapy research and development program (\$75k)
 - 4. Position BSF for a go / no-go decision on enzyme replacement therapy (\$25k)
 - 5. Define next steps for BSF's patient registry (FTE only)
 - 6. Implement variants database 2.0 as a public resource (FTE only)
 - 7. Create a data use agreement to magnify effect of all BSF-funded human subjects research (\$7.5k)
 - 8. Invest in BSF biorepository solution (\$15k)
 - a. Emily forecasted that this would likely be a commercial, not academic, solution
 - 9. Publish consolidated recommendations for future BTHS clinical trials from what we have learned in the US and UK (FTE only)
 - ii. Support our Barth Families
 - 1. Global meetings/gatherings called Stronger Together World Tour - STWT (\$100k)
 - a. Aiming for four meetings in the US, as well as some funding for international gatherings
 - 2. Advance our clinical guidance around feeding, nutrition, and digestion in BTHS
 - 3. Expand BSF Cares program (\$20k)
 - 4. Improving the BSF website (\$50k, plus more in 2023)
 - iii. Advocate for Our Community

1. Exhaust all ethical means of creating access to elamipretide as a therapy for Barth syndrome
 2. Create marketable sponsorships for BSF activities (offset of \$35k)
 3. Make a decision regarding Blackbaud and potentially select a new CRM / fundraising platform (\$10k)
- iv. Never, Ever Give Up
 1. Develop an organizational fundraising strategy
 2. Grow our volunteer leadership by identifying 20 new volunteers
- f. Discussion followed
- g. Erik Lontok introduced Clive Spiegler who shared the gene therapy roadmap
- i. Goal is to align on the dual path strategy for gene therapy and approve the plan and funding for the next phase of activity
 - ii. Reviewed the key components of and the science behind gene therapy
 - iii. Mentioned the high quality of the animal models as an asset for BSF
 - iv. There are currently two Barth AAV9 gene therapy programs that are fairly similar—they use the same capsid but different promoters – but are at different stages of development
 - v. Decision at hand is to continue with the AAV9 capsid, fund a novel capsid, or go down both paths
 - vi. Discussion followed
 - vii. Goal is to come back in June with a more detailed point of view on the paths forward and BSF's role in them
 - viii. At this time, Clive recommends pursuing a dual path strategy until we need to make a choice
 - ix. Nina Russell encouraged BSF to pursue an additional novel capsid, if possible, to further de-risk our position in gene therapy
- h. Steve McCurdy also has helped BSF on the business development side of our therapy development efforts – both with gene therapy and enzyme replacement therapy
- i. Erik turned the discussion to Enzyme Replacement Therapy and reviewed the current state of Dr. Michael Chin's research
- i. Steve shared that the NIH funding for Dr. Chin's research goes for two years and that the third year is contingent on finding a partner to co-find a portion of the cost; BSF would be a logical partner for that
 - ii. Because the University of Washington owns the patents he is using, Dr. Chin has asked BSF to fund the \$25k cost of these for the next year through his company, TransCellular Therapeutics (TCT)
 - iii. Steve reiterated that gene therapy has great potential but, even if it works, likely will not be feasible for or available to everyone and that we should

continue to pursue enzyme replacement therapy as an alternative; he voiced his support of TCT at this juncture in order to help de-risk any future possible investment

- iv. We need to recognize that the probability of a meaningful return from our possible investment would be relatively low
 - j. Initial discussion followed but Kate assured the board that we will have more time to discuss both gene therapy and enzyme replacement therapy in the future before any investment decisions are finalized
4. Discussion and Vote: Full 2022 Budget
- a. In reviewing the budget, Kate reinforced that BSF has had significant surpluses over the past two years and that while the proposed investments are large, they are only a fraction of our cumulative surpluses
 - b. Discussion followed
 - c. Kate then challenged Emily and the BSF staff to increase BSF's revenue goal by \$11k to match our revenue from 2021
 - d. Emily agreed and Kate moved to a vote
 - e. **APPROVED:** 2022 budget as proposed, with an increase in the revenue target by \$11k to the 2021 revenue level
5. Discussion: Board Self-Assessment
- a. Jamie Baffa shared background on the benefit of the Board self-assessment task and the request that board members please respond well before the April meeting
6. Update: Drug Development Advocacy
- a. Kate shared a brief update on interactions with Stealth and also with the FDA
 - b. This included sharing a letter from BSF to the FDA regarding the feasibility challenges with ultra-rare diseases
7. Discussion and Vote: SMAB Members for Additional Terms
- a. Kate suggested that SMAB re-nominations be handled by email in the interest of time
8. Discussion and Vote: Board of Directors Candidates
- a. Two individuals were proposed as new board members
 - i. Miriam Greenberg, PhD
 1. She is a long-time Barth researcher, which is a gap on our board, and also has served since 2005 as an SMAB member (which she will continue to do)
 2. Miriam is interested in a one-year term as a trial
 3. She is dedicated to BSF and would like to serve if voted in, but joining the board would be a sacrifice as she then would be ineligible to apply for a BSF research grant (since the BSF board is the body that awards grants and so that would be a conflict of interest)

ii. Mark Greene

1. He is currently a much valued member of our Finance and Investments committee
2. Brings an impressive resume and a great deal of experience
3. He is very interested in further supporting BSF by serving on the board

- b. **APPROVED:** Both Miriam and Mark will join the board in April
- c. Kate indicated that she will be stepping down as the SMAB liaison as Miriam Greenberg will fill that role moving forward
- d. Kate will also be stepping down from the Finance and Investments committee but still can be invited to meetings when it would be appropriate to have the Board Chair participate

9. Discussion and Vote: Additional Terms for Current Board Members

- a. Brandi Dague, Michelle Florez, Kate McCurdy, and Peter van Loo left the call
- b. Jamie opened the floor for discussion and then proceeded to lead the vote
- c. **APPROVED:** Brandi Dague for an additional three-year term
- d. **APPROVED:** Kate McCurdy for an additional three-year term
- e. **APPROVED:** Michelle Florez for an additional three-year term
- f. **APPROVED:** Peter van Loo for an additional three-year term

10. Adjournment

- a. Jamie Baffa adjourned the meeting at 2:07 p.m. ET

Respectfully submitted,

Jamie Baffa – Secretary