

MINUTES

Barth Syndrome Foundation, Inc.

Board of Directors Meeting

December 13, 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
Megan Branagh	Board Member
Michelle Florez	Board Member
Miriam Greenberg	Board Member
Nina Russell, MD	Board Member

Invited Guests

Brett Smith	Operations and Communications, BSF
Emily Madalinski	Development and Stewardship, BSF
Erik Lontok	Director of Research, BSF
Melissa Huang	Clinical Research Coordinator, BSF
Shelley Bowen	Director of Family Services, BSF
Steve McCurdy	Finance & Investments Committee
Susan McCormack	Policy & Agreements Committee

Members in Not Attendance:

Florence Mannes	Board Member
Maryanne Chrisant, MD	Board Member
Peter van Loo	Board Member

AGENDA

1. *Welcome*
2. *Vote: Email Votes, October BOD Minutes, November BOD Minutes*
3. *Vote: Organizational Policies*
4. *Update: Treasurer Report*
5. *Vote: TransCellular Therapeutics (TCT) Grant*
6. *Vote: Novel Capsids Gene Therapy Consultants*
7. *Vote: NYU Natural History of Cardiac Pathologies Registry Agreement*
8. *Discussion: 2022 Organizational Priorities Annual Update*

9. *Discussion: 2023 Organizational Priorities*
10. *Executive Session*

The meeting, held via Zoom, was called to order by Kate McCurdy on Tuesday, December 13, 2022, at 12:02 p.m. ET.

1. Welcome
 - a. Kate McCurdy welcomed board members and invited guests
2. Vote: Email Votes, October BOD Minutes, November BOD Minutes
 - a. Jamie Baffa asked for final input and approval on BOD minutes from October and November
 - b. **APPROVED:** October BOD minutes
 - c. **APPROVED:** November BOD minutes
 - d. Kate reviewed offline votes taken between BOD meetings, including:
 - i. **APPROVED:** \$93,750 total investment in additional work by Dr. Bill Pu's lab to identify a novel capsid (\$68,750) and ongoing advisory support from Clive Spiegler (\$25,000)
 - ii. **APPROVED:** Funding up to \$250,000 over five years to cover supplemental BGTC trial participant and caregiver travel and per diem expenses should Barth syndrome be selected for BTGC funding. BSF funding is further contingent on study team completing paperwork required by BSF research governance.
 - iii. **APPROVED:** September BOD minutes
 - iv. **APPROVED:** Holding additional Board meeting in November 2022 to cover agenda items left unaddressed after 10/17/22 Board meeting call
3. Vote: Organizational Policies
 - a. Susan McCormack reviewed proposed changes to policies and procedures, including:
 - i. Updating BSF's record retention and document destruction policy in partnership with external legal counsel from Kirkland to align with best practices for similar organizations
 1. **APPROVED:** New "Records Management Policy" and "Records Retention Schedule"
 2. **APPROVED:** The "Records Management Policy" and the "Records Retention Schedule" replace the previous "Record Retention and Document Destruction Policy"
 - ii. Updating the Code of Ethics

1. Kate asked to add “inclusion” as a BSF value to match the website
 2. **APPROVED:** With suggested change
 - iii. Updating the Conflict of Interest Policy
 1. No edits, but the board discussed whether to include reviews or decisions made by the board related to this policy in meeting minutes
 2. **APPROVED**
 - iv. Creating a new policy for the Research and Development Committee
 1. Kate highlighted the importance of having a range of expertise on this committee
 2. **APPROVED**
 - v. Making small revisions to:
 1. Confidentiality Policy (previously Confidentiality Acknowledgement)
 2. Whistleblower Policy
 3. Operating Reserve Policy
 4. Executive Committee Policy
 5. **APPROVED:** All updates to the above policies
4. Update: Treasurer Report
- a. Mark Greene reviewed the current financial state of BSF and year-end projections
 - i. Fundraising is on track, with a few more large donations to pursue
 - ii. Affiliate contributions lower than expected due to timing associated with delays in launch of Arrhythmia Project until 2023
 - iii. No revenue from the 2022 Stronger Together World Tour (STWT)
 - iv. Noted that after updating the year-to-go forecast in the investment portfolio, BSF’s operating income will be a deficit closer to \$116,000
 - b. Emily Milligan summarized the year-end financial picture against the forecast
 - c. Kate reviewed strategic investments and the status of BSF’s investment account
 - i. Overall, investments are down 12% which is less than expected due to BSF’s conservative investment position
 - ii. Mark noted the foundation did not lose any cash, we simply moved it
 - d. Kate reinforced that the foundation is in a strong financial position
5. Vote: TransCellular Therapeutics (TCT) Grant

- a. Erik Lontok reviewed the transition from an NIH R61 grant to and an NIH R33 grant
- b. Steve McCurdy reinforced that BSF would be responsible for funding \$87,500, if Michael Chin passes all the NIH scientific hurdles to transition to an R33 grant
- c. The foundation proposes that the board authorize a letter that would confirm that BSF would fund its portion of the R33 conditioned on NIH full approval and funding, in line with the terms of our standard research grant
- d. Separately, BSF will require that TCT agree to certain conditions, including:
 - i. Should TCT or its successor cease development of enzyme replacement therapy for Barth syndrome, TCT or its successor will extend its patent license and access to its work to that time to BSF and its chosen partner, at no cost to BSF, so that development of enzyme replacement therapy may continue
 - ii. If TCT or its successor continue to develop enzyme replacement therapy for any other disease or disorder, BSF will be entitled a share of revenues in accordance with the terms of our research grant proposal
 - iii. TCT will include a representative of BSF in its meetings with potential third-party venture capital investors
 - iv. TCT will agree to work jointly with BSF to name an experienced, independent advisory team to guide its preparation and submission of IND and NDA documents; and
 - v. TCT will provide a map of the scientific milestones required for IND and NDA submissions to the FDA.
- e. Emily reinforced that these terms put the foundation in a strong position, regardless of the outcome
- f. Kate noted that if BSF doesn't fund a portion of the R33 grant, this pathway to a treatment will likely disappear
- g. Discussion followed
- h. **APPROVED:** BSF to sign a letter indicating that BSF would fund its portion of the R33 grant conditioned on NIH full approval and funding, in line with the terms of our standard research grant and issue a separate letter to TCT with the conditions outlined above

6. Vote: Novel Capsids Gene Therapy Consultants

- a. Erik noted that to pursue a novel capsid, we need to pursue four parallel paths
 - i. Nonclinical
 - ii. Technical
 - iii. Clinical

iv. Regulatory

- b. Diana Blumenthal and Janice Phillips have been providing technical advice on a pro-bono basis
 - c. Erik proposing up to \$12,000 for Diana and Janice and \$8,050 for Andra Miller for additional advice
 - d. Emily noted that this work is a hedge against AAV9 (which may be funded through BGTC) and that the foundation only has the funds to support one of these paths to its conclusion
 - e. This vote would keep this path advancing while we await a decision for BGTC funding
 - f. **APPROVED:** BSF to contract Diana Blumenthal and Janice Phillips (up to \$12,000), and Andra Miller (\$8,050) as consultants
7. Vote: NYU Natural History of BTHS Cardiac Pathologies Registry Agreement
- a. Emily provided background on this effort, including the intersection of the Arrhythmia Study and the Natural History of BTHS Cardiac Pathologies
 - b. The board had approved \$350k in the previous meeting pending final agreements
 - c. BSF is waiting on coordinating and participating site agreements, which are expected in time for February 2023 board meeting
 - d. Emily highlighted that the goal is for other researchers to be able to input additional cardiac clinical data into the natural history registries
 - e. With the fully executed registry agreement, BSF will pay NYU \$56,606 in December 2023 and \$21,227 once the registry has been executed, expected in June 2023
 - f. Emily flagged that this is milestone-based funding, so we will only acknowledge revenue and expenses once work has been completed and invoiced
 - g. **APPROVED:** Execute Natural History of BTHS Cardiac Pathologies Registry Agreement with total milestones valued at \$56,606
8. Discussion: 2022 Organizational Priorities Annual Update
- a. Emily reviewed high level achievements
 - b. Melissa Huang shared how BSF has invested in research engagement and recruitment
 - i. Recruitment efforts for Mayo Clinic study
 - ii. SciMed virtual symposium
 - c. Erik discussed highlights regarding research and development
 - i. Seven new investigators and nine outside the United States among 14 grant applications

- ii. Submission of the BGTC clinical trial proposal
 - d. Shelley shared highlights from Family Services
 - i. Rare disease week on Capitol Hill
 - ii. Sibling webinar
 - iii. Collaboration with National Neutropenia Network
 - iv. Empower Award during 2022 Nicer Symposium
 - v. Barth syndrome emergency relief program
 - vi. Year-end celebration
 - vii. Collaboration with Project Sunshine
 - e. Emily and Melissa discussed Stronger Together World Tour
 - f. Emily shared advocacy highlights
 - g. Brett Smith shared communications highlights
 - h. Emily Madalinski shared fundraising highlights
 - i. Most successful Giving Tuesday ever
 - ii. 72% fundraiser retention
 - iii. 10 new fundraisers
 - iv. \$124,250 raised (\$109,001 in 2021)
- 9. Discussion: 2023 Organizational Priorities
 - a. Emily briefly reviewed a slide that included priorities for 2023 and asked the board to please review and offer feedback and suggestions once distributed
- 10. Executive Session
 - a. Staff and invited guests asked to leave
- 11. Adjournment
 - a. Kate adjourned the meeting at 2:09 p.m. ET

Respectfully submitted,
Jamie Baffa – Secretary