

MINUTES

Barth Syndrome Foundation, Inc.

Board of Directors Meeting

June 27, 2023

Members in Attendance:

Andrew Buddemeyer	Board Member
BJ Develle	Board Member
Brandi Dague	Board Member
Emily Milligan, MPH	Board Member <i>ex officio</i> , Executive Director
Florence Mannes	Board Member
Jamie Baffa	Board Member, Secretary
Jonathan Stokes	Board Member
Kate McCurdy	Board Member, Chair
Mark Greene	Board Member, Treasurer
Michelle Florez	Board Member
Nina Russell, MD	Board Member
Peter van Loo	Board Member

Invited Guests

Brett Smith	Operations and Communications, BSF
Clive Spiegler	Gene Therapy Consultant
Emily Madalinski	Development and Stewardship, BSF
Erik Lontok, PhD	Director of Research, BSF
Melissa Huang, PhD	Clinical Research Coordinator, BSF
Natalie Cohn	Controller
Shelley Bowen	Director of Family Services, BSF
Steve McCurdy	Finance & Investments Committee
Susan McCormack	Policy & Agreements Committee Chair

Members in Not Attendance:

Megan Branagh	Board Member
Miriam Greenberg, PhD	Board Member

The meeting, held via Zoom, was called to order by Kate McCurdy on Tuesday, June 27th at 12:01 p.m. ET.

1. Welcome
 - a. Kate McCurdy welcomed Jon Stokes to his first board meeting

- b. Notified the board that Maryanne Chrisant, MD has resigned from the board due to ongoing scheduling conflicts but has reinforced her commitment to supporting and advising BSF in other ways
2. Update: Gene Therapy Novel Capsid Program
- a. Clive Spiegler reviewed goals for this discussion and BSF's parallel path approach
 - b. Achievements with Novel Capsid program
 - i. Biodistribution study in mice completed and NOVEL capsid variant selected
 - ii. Manufacturing strategy assessment complete
 - iii. GTRP funding for the manufacture of research grade vector for Barth syndrome at Penn Vector Corp has been approved by NIH; this award is worth about \$150K
 - iv. Mice studies initiated to select optimal promotor and codon optimized DNA sequence for final candidate
 - c. Expect to select final candidate by beginning of September and send to Penn Vector Corp in October
 - d. Noted that there is an ongoing movement in the industry toward novel capsids that can be used at lower doses to improve safety
 - e. Two options for manufacturing a novel capsid (NOVEL)
 - i. Option A: Start with Penn Vector Corp for proof-of-concept efficacy studies in mice and switch to Andelyn for IND toxicology (tox) studies and clinical grade vector
 - ii. Option B: Manufacture vector for all studies at Andelyn
 - f. Option A utilizes NIH GTRP grant through Penn Vector Corp that we now have been awarded
 - i. No cost to BSF for research grade vector
 - ii. Short timeline (seven to 10 weeks)
 - iii. Funding program ends December 18, so would need to complete our work with them by that date; some small amount of risk associated with this timeline
 - iv. Will still need to change manufacturers for tox and clinical vector manufacturing
 - g. Option B
 - i. Allows to use a similar process for all vectors
 - ii. May result in six-month delay
 - h. Recommendation is to go with Option A

- i. Noted that we would need to start to work with Andelyn in January 2024 on manufacturing material for tox studies if we decide to fund this work all the way to a pre-IND meeting
 - j. There will be a choice later this year on whether to continue on tot his engagement with the FDA, which carries a significant financial investment
 - i. However, it would clarify the path to the clinic and make the work more appealing to partners
 - k. Kate reiterated that this is not the only path that remains open for gene therapy
 - l. Steve McCurdy noted that upfront funding for this work helps to de-risk the program as it continues
 - m. **APPROVED:** Option A to start with Penn Vector Corp for manufacturing research grade NOVEL vector
3. Update: TCT BSF R33 Commitment
- a. NIH has eliminated the requirement for third party funding associated with the third year of the R33 grant that Michael Chin has for his Barth syndrome enzyme replacement work
 - b. Thus, BSF is no longer committed to funding TCT and so has freed up \$87,500
 - c. TCT has successfully purified Tafazzin protein and expects to publish his findings soon
 - d. Michael Chin has been named the interim Dean of Graduate School for Biomedical Sciences at Tufts University
 - e. Emily Milligan noted that we would revise the SciMed budget forecast based on this development
4. Update: Elamipretide Next Steps
- a. Stealth is working with the FDA to show them new elamipretide data to try to "unstick" the regulatory process; Kate, Todd Cade, and Colin Steward also are involved in this effort
 - b. Melissa Huang reviewed the planning for a potential Phase Four trial by Stealth, which will involve sites outside of the United States (in the event that elamipretide were to be granted accelerated approval)
 - c. Stealth has entered an exclusive licensing agreement with Pharmanovia to do the regulatory work and commercialize elamipretide for Barth syndrome in Europe and parts of the Middle East and North Africa
 - d. BSF has received comments from the FDA on a joint white paper outline tentatively entitled "Executable Regulatory Pathway for Rare Diseases: The Barth Syndrome Story" that we had submitted to them in March

5. Update: Policy on Clinical Trials and Clinical Studies
 - a. This policy is current under revision to better align with BSF's current role as an active partner in clinical trials and human subject studies
 - b. Goal is to approve this before receiving proposals for onsite research during 2024 Conference
 - c. Melissa reviewed background on the policy and what the revised policy will need to accomplish
6. Vote: The Woodward BSF-Family Engagement Fund
 - a. The Woodward family wishes to establish a restricted fund at BSF to allow families to participate fully in BSF programs, in honor of Steven Woodward
 - b. Kate reviewed principles that the Finance & Investments Committee and the Fundraising Committee have been working on that BSF should follow in establishing restricted funds, highlighting that the goal is to avoid having money sitting somewhere that the foundation cannot use
 - c. The Board agreed with these principles as guides for our policy
 - d. Emily forecasted that the Policy and Agreements Committee would work to translate these principles into a formal policy
 - e. **APPROVED:** BSF to establish The Woodward BSF-Family Engagement Fund
 - f. **APPROVED:** Moving funds given to date in honor of Steven Woodward from the general fund to The Woodward BSF-Family Engagement Fund to “seed” the fund
7. Update: Treasurer Report
 - a. Natalie reviewed the report with only one notable change to the forecast related to cancellation of STWT
 - b. Balance sheet is strong
8. Other Business
 - a. Kate shared that Erik has just submitted his resignation from BSF to take a position in another organization and that his last day will be July 7; she acknowledged his many contributions with appreciation and reinforced that all of the great work in the pipeline will continue; we will miss him and wish him well in his new endeavor
 - b. Board retreat will likely be in Chicago, beginning Friday October 13 and concluding after lunch on Sunday, October 15
 - c. Kate said that April minutes are not yet available, so we will conduct an offline vote for their approval
 - d. Reviewed Board Resource Handbook

- e. Acknowledged cancellation of all STWT events after the one in Gurnee, IL on June 23rd and 24th resulting in a reduction of expected expenses from \$75k to ~\$28k
9. Adjournment
- a. Kate adjourned the meeting at 2:00 p.m. ET

Respectfully submitted,

Jamie Baffa – Secretary