

MINUTES
Barth Syndrome Foundation, Inc.
Board of Directors Meeting
June 26, 2024

Members in Attendance:

Kate McCurdy	Board Member, Chair
Megan Branagh	Board Member
Brandi Dague	Board Member, Secretary
BJ Develle	Board Member
Mark Greene	Board Member, Treasurer
Michelle Florez	Board Member
Emily Milligan	Board Member <i>ex officio</i> , Executive Director
Nina Russell, MD	Board Member

Invited Guests

Shelley Bowen	Staff, BSF
Lindsay Marjoram, PhD	Staff, BSF
Steve McCurdy	Volunteer, BSF
Emily Madalinski	Staff, BSF
Melissa Huang, PhD	Staff, BSF
Natalie Cohn	Controller, BSF

Members in Not Attendance:

Jamie Baffa	Board Member, Vice-Chair
Miriam Greenberg, PhD	Board Member
Jonathan Stokes	Board Member
Peter van Loo	Board Member

The meeting, held via Zoom, was called to order by Kate McCurdy on Wednesday, June 26 at 12:03 p.m. ET.

1. Welcome
 - a. The meeting began with a welcome from Kate McCurdy, the Chairperson.

2. BSF Gene Therapy Programs(s) Update
 - a. Lindsay Marjoram provided a succinct and comprehensive update on BSF's gene therapy program:
 - b. Lindsay summarized the funding that BSF has invested in gene therapy. These include 3 grants (2011, 2015, and 2017) using the standard AAV capsid as well as a 2 year investment (2023-2024) that will investigate a novel capsid that could get better expression but at a lower dose.

- i. The novel capsid program has experienced some experimental and staffing delays, so the current clinical trial date estimate is in the 2027-2028 timeframe.
- ii. The standard AAV program researchers are also looking at novel capsids, but are satisfied with their promoter. They also have an active research program related to another condition that is reviewing immunosuppressive regimens and functional endpoint evaluation along with the effect of exercise on AAV uptake, both of which could be very important for a gene therapy approach with Barth syndrome. Currently, there is no firm timeline for a clinical trial in Barth syndrome.
- iii. BSF has engaged with a new researcher out of UPenn who has developed a novel capsid with high expression in the heart and has demonstrated proof of concept in primates. His program is unique in that they do not require external funding. Currently, they have not demonstrated good expression in skeletal muscle, however, this was not on their radar. They will be presenting a poster at the BSF conference, so we look forward to learning more.

c. Lindsay pointed out our very exciting progress on gene therapy. Back in 2018, BSF had 1 gene therapy program and now in 2024, we have 3 programs. However, due to financial and patient population constraints with the single dose administration, BSF can't afford to progress 3 different programs in parallel. From a financial standpoint, Lindsay estimated the cost of a single gene therapy trial at \$7-10m for 4-6 participants. However, BSF continues to explore an array of funding vehicles.

d. Lindsay mentioned that BSF will host a small dinner at the upcoming conference with our gene therapy constituents in attendance in hopes of uniting to progress the most promising candidate.

3. Meeting Minutes

- a. Brandi Dague asked for approval for the April 30, 2024 board minutes
- b. **APPROVED** as written

4. Strategic Planning Update

- a. Kate McCurdy proposed an extension of the current Strategic Plan through 2025 and that we delay the Board's strategic planning session for 1 year.
- b. **APPROVED** as proposed

5. NDA Review and Ad Comm Update

- a. Kate McCurdy confirmed that Stealth was granted Priority Review status by the FDA after initially receiving a Standard Review status. This is a HUGE win towards our efforts to get Elamipretide approved.
- b. Kate also confirmed that Stealth has completed their Mid Cycle Review with the FDA regarding the approval.

- c. Emily Milligan provided an update on the Ad Comm meeting with the FDA. While no official date has been set, we believe it will be held in the Fall. It is expected to be a full day meeting with 60-90 (probably 60) minutes dedicated to the Open Public Hearing (OPH) where the BSF community could share its key patient testimonies. It has not yet been decided if the event will be in-person or a hybrid event.
- d. Emily expressed that given the importance of our preparation for the OPH, we are exploring the engagement of an external consultant to assist. She estimates this would cost approximately \$20-50k. Also, should this be an in-person event, the travel expenses for our patient community in attendance are estimated at \$40-60k based on experience during Rare Disease Week. Neither of these expenses are in the current BSF budget.
- e. Emily also mentioned the importance of the principle of BSF maintaining both the reality and the optics of independence from Stealth during this review period until the date at which a final decision about elamipretide has been made. This way it is clear that our patient voice is our own and that we are not unduly influenced by the company or their business priorities.
- f. Emily also noted that should Elamipretide be approved in the US, Stealth had announced a licensing agreement with Pharmanovia in the UK. We have learned that Pharmanovia has filed a submission to potentially move into a clinical trial with the MHRA (the UK's equivalent to our FDA) and potentially beyond. This could potentially augment access to our international population.

6. Advocacy Update

- a. Emily Milligan informed the board that both senators in the state of Arizona (Kyrsten Sinema and Mark Kelly) wrote to the FDA, independently of BSF and Stealth, seeking an Ad Comm with appropriate representation.
- b. Emily mentioned that Senator Mike Braun has been very supportive of the BSF community. He was trying to garner bipartisan support for a letter to the FDA that highlighted the inequities our community was being subjected to in terms of a fair and equitable review. While he was not able to get a bipartisan Senate letter, he did submit the letter to the FDA with bipartisan House support. He has also been pushing a legislative effort called the Promising Pathways Act (PPA). This legislation has received bipartisan support and numerous other sponsors. His office has recently been critical of one of our rare disease partners, NORD and vice versa. We have encouraged the organizations to work together to find common ground to advance the efforts of the collective rare disease community.

7. 2024 Conference Update

- a. Melissa Huang reported on the number of attendees. 345 registered so far: 230 family members, of which 60 are affected individuals and 95 are first time attendees. Nearly 1/3 first time attendees. 22 new families. 40 transplant families. BSF Travel Assistance (administered by NORD with BSF funds) helped 14 families to attend. 82 Sci-Med attendees.

- b. Melissa noted that on the Sci-Med track there will be 60 speakers, 38 posters (highest ever), and 7 on-site studies.
- c. Emily mentioned that we began capping the reimbursement to our Sci-Med speakers, similar to other organizations, in order to help offset the costs of our Family track speakers.
- d. Emily provided an update on the conference revenue, sponsorships, and pending asks. The goal was to offset 50% cost (or \$191k); we budgeted \$165k in revenues. We have confirmed 83% of our goal and are very confident that we will meet \$165k budget, assuming we receive a pending NIH Grant award. If we reach our goal, that will result in shrinking our planned budget deficit by \$26k.
- e. Emily reiterated that closing the gap between revenue and expenses remains an organizational priority to successfully mitigate the affordability of the conference (budgeted at a planned deficit of \$182k). The long-term ambition for future conferences remains to achieve net zero whereby revenue fully offsets the cost of this cornerstone event.
- f. Kate instructed the board to act as ambassadors during the conference by engaging affected members, new families, old families, Sci-Med attendees, etc. She also charged us to keep our eyes open for new board candidates.
- g. BJ Develle spoke on how our community has evolved over the years. He discussed some of the increased challenges in engaging our ever diverse community and how we can offer guidance especially to volunteers who work with our families on how best to be respectful of each other and everyone's individual situations, needs and boundaries. We will offer training for our volunteers and members on this prior to the conference. Kate encouraged the Board to attend.

8. 2023 Audited Financial Statements

- a. Kate McCurdy mentioned that all of the requested changes have been made in the financial statements. Mark Greene proposed that we vote to accept.
- b. **APPROVED** as written
- c. Mark notified the Board that the lead partner on our account, Ed Balthazar, has left Dorfman, but we have already been in contact with another partner, Patricia Diaz, who leads their Not-for-Profit practice.

9. Treasurer Report

- a. Natalie Cohn reported on the organization's financial status through May 31, 2024, noting that contributions are \$383,000 year-to-date, with revenue at \$676,000, and total operating expenses at \$910,000. She also mentioned that the results include \$53k in unrealized gains in the Investment Portfolio as a result of market appreciation. We are generating an operating loss at this time and expect to generate a loss for the full year, which was budgeted due to the conference.

- b. Emily Milligan commented that we should see a slight revenue uptick for conference, but don't expect expenses or revenue results to be materially different from the approved budget for 2024.
 - c. Mark Greene also commented that with the increased costs related to the upcoming Ad Comm meeting, we might expect a small shortfall to budget. Emily concurred but also added that there could also be some savings related to FTE expenses actual vs. budget due to delays in hiring a development director.
- 10. Senior Communications Consultant
 - a. Emily Milligan proposed to extend the contract of our current resource for a total budget of \$15,000.
 - b. **APPROVED** as proposed
- 11. Other Business
 - a. Emily Milligan mentioned that Stealth gave us ownership of the NotTooRareTo Care website so we have control of it. This means, however, that BSF may need to fund the ongoing maintenance of the this website as well as the external media relations provided by Anna Stallman. She is working through what is necessary as compared to what we might like to have and the budget implications thereof.
- 12. The meeting was adjourned by Kate McCurdy at 2:02pm ET.

Respectfully submitted,

Brandi Dague

Secretary