

MINUTES

Barth Syndrome Foundation, Inc. Board of Directors ANNUAL Meeting September 11, 2024

Members in Attendance:

Jamie Baffa	Board Member, Vice-Chair
Megan Branagh	Board Member
Brandi Dague	Board Member, Secretary
BJ Develle	Board Member
Michelle Florez	Board Member
Miriam Greenberg, PhD	Board Member
Jonathan Stokes	Board Member
Peter van Loo	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:

Kate McCurdy	Board Member, Chair
Emily Milligan	Board Member <i>ex officio</i> , Executive Director
Mark Greene	Board Member, Treasurer
Nina Russell, MD	Board Member

The meeting, held via Zoom, was called to order by Jamie Baffa on Wednesday, September 11th at 12:02 p.m. ET.

1. Welcome
 - a. The meeting began with a welcome from Jamie Baffa, the Vice-Chairperson.
2. Meeting Minutes
 - a. Brandi Dague asked for approval for the June 26, 2024 and July 31, 2024 board minutes
 - b. **APPROVED** as written
3. Treasurer Report
 - a. Natalie Cohn reported on the organization's financial status through July 31, 2024, noting that revenue is at \$800,000, which is twice as much as last year. The NIH Grant is not included yet but will be booked in the August financials. Total operating expenses are at just over \$1,000,000, which is what was

expected and comparable to FYTD23. Operating income sits at a deficit of \$257,000 which is expected given that the bulk of our donations come at year end. She also mentioned that the results include \$200,000 in unrealized gains in the Investment Portfolio as a result of market appreciation.

- b. Natalie noted that we will have comparisons to forecast after the August close. From a balance sheet perspective, Natalie commented that we are in a strong position and moved \$250,000 from investments into August to cover conference expenses as previously anticipated.
- c. Natalie also stated that the organization has approximately \$5 million in the investment portfolio of which approximately \$500,000 is held in cash, which is strong.
- d. Jamie Baffa commented that we should expect to see a larger deficit with the updated forecast based upon 2024 conference expenses and also the Board's direction to utilize independent AdComm prep resources.

4. Science and Research Updates

- a. Lindsay Marjoram notified the Board of the exciting news that BSF has been awarded the Rare as One grant by the Chan Zuckerberg Institute. The main goal of these funds will be to form a central repository external to academic institutions of readily available de-identified biospecimens, which is harmonized with our registry, for further investigation into Barth syndrome. These restricted funds will be received as follows: \$100k in Year 1; \$200k in Years 2-4, and \$100k in Year 5 for a total of \$800k over 5 years. Melissa Huang will manage the centralization efforts with two main goals:
 - i. Identify a storage solution under BSF's purview
 - ii. Establish a robust patient entry system capturing genetic variant, disease severity, etc. Melissa mentioned that we have identified a new multi-lingual platform called Matrix, for which a more detailed proposal will be presented at a later date.
- b. Melissa Huang also shared the exciting news that a special edition article based upon data from the BSF Registry 2.0 and in collaboration with the Southeast Regional Genetic Network has been accepted for publication with Dr. Hilary Vernon as the editor. Once published, a lay summary will be provided. Lindsay acknowledged Melissa's role in shepherding the efforts to get this accomplished. Jamie recognized our own team's efforts in getting BSF's research work published.

5. Gene Therapy Update

- a. Lindsay Marjoram recapped gene therapy and how it can cure Barth syndrome as well as FDA's engagement during the process. She discussed the delay in Dr. Pu's efforts with the alternate vector and explained that it is very common in this type of research.

- b. Lindsay explained the role of the Board and how it will inform BSF's direction with respect to the path forward. 1) Making informed decisions with possible input from outside experts and 2) Ensuring financial feasibility. In making an informed decision on therapy programs, the Board must determine if gene therapy is "ready" for Barth syndrome as well as is the Barth community "ready" for gene therapy? Additional considerations include looking for an established roadmap from peer organizations, understanding who will shepherd our efforts through clinic in the event we don't have industry support, and how/when it will be financed.
 - c. Jamie Baffa concurred that these considerations were spot-on in terms of the decisions the Board would need to make surrounding gene therapy. He mentioned that funding will be a key question as well as BSF's comfort with a) being at the forefront of trying to advance a novel capsid vs. b) being a close follower of others' hopefully successful novel capsid efforts so that the pathways have already been established. The former would be more risky and costly, but the latter would likely yield a possible Barth therapy at a slower pace. He strongly noted that patients' ultimate ability to access any treatment would be another key factor to consider.
 - d. Jamie mentioned that we should ensure we are looking at therapies overall in a strategic way, and not just solely at gene therapy. Lindsay concurred that BSF should be thinking about that philosophically and be open/prepared to support other types of therapies that could present at any time. Jonathan Stokes recommended, based on his experience in developing gene therapy trials, that BSF delve into the BSF community's understanding and expectations about gene therapy. BJ Develle concurred that we need to ensure that we are supporting all groups within our community. Lindsay mentioned that we have two small biotechs that have applied for BSF grants for the very first time.
 - e. Several attendees commented on the progress that has been in having multiple opportunities for gene therapy, a place that frankly could not have been imagined a few years ago. Jamie stressed the importance of ensuring that each board member is as informed as they can be in order to make the very important upcoming decisions regarding the best path to be selected for our community.
6. 2024 Conference Debrief
- a. Melissa Huang stated that the 2024 conference was our largest in terms of attendees and programming. It included 111 sessions across 3 tracks for 345 attendees from 11 countries. The largest audience was affected individuals and their families, but over a quarter of the attendees were physicians and researchers, also with our largest industry presence to date. Melissa acknowledged Shelley Bowen and Emily Madalinski's efforts in creating such a successful event. The general feedback was very positive, with a 4.4 out of 5 (highest) rating in the post-event survey. Attendees seemed to enjoy the various tracks, the intermixing of families with physician/researchers, and the feeling of being all together with very high engagement all around. Jamie thanked everyone on the BSF staff, many of which had never attended a conference, for their efforts and

engagement in making this such a successful event. Miriam Greenberg expressed special recognition for Lindsay and Melissa who came into the planning very late in the game but put together such an impactful science track which was the best she had ever seen.

7. 2026 Conference Planning

- a. Michael Bowen shared that we requested and received a proposal from the Hyatt Regency in Bonita Springs for the 2026 conference given the positive outcome and good feedback from this year's conference. The room rate offered was very reasonable and the food and beverage minimum was below what was actually spent for 2024. Concessions from 2024 were also essentially matched for 2026. The hotel's proposal is valid until the end of October and the board's proposal was to allow Michael to continue negotiating on behalf of BSF. Jamie concluded by thanking Michael for his efforts.
- b. **APPROVED** as proposed

5. Ad Comm Prep

a. External Media Consultant

1. Jamie Baffa reminded the Board that they voted in July at the conference to hire an external media consultant to advise on all media and public relations related to the AdComm. He had recommended someone from his professional network, Matt Burns, who had specific experience in shepherding another organization through their AdComm. Both Kate and Emily met with him, along with several other candidates, and also recommend hiring him. His proposal meets BSF's needs and is in the expected price range. A motion was made to approve hiring Matt for a five-month period for a total expense of \$50,000.
2. **APPROVED** as proposed

b. Community Engagement

1. Shelley Bowen explained that it's been a very busy time after the conference preparing for the upcoming Ad Comm meeting. BSF wants to ensure that every member of the community has an opportunity to engage, and communication efforts will be starting shortly across multiple platforms. The final list of participants will be selected by the FDA, but BSF feels it is very important to have those affected individuals who have been on the drug try to speak in-person (if at all possible) at the actual Ad Comm meeting. We also have engaged several key opinion leaders who will either apply to participate in-person or will write letters. If chosen, these individuals will participate in Open Public Hearing (OPH) training given by an experienced external consultant whom Emily was able to engage at a discounted fee. BSF will be reaching out to the above affected individuals, caregivers and clinicians and ask that they write testimonials and submit them to the Ad Comm docket, and BSF also will schedule training sessions to help everyone prepare. Jamie thanked Shelley and the team for their efforts to make this OPH successful for BSF.

10. 2025 Grant Program Changes and Improvements

- a. Lindsay Marjoram discussed changes to the 2025 grants. These include consolidating the Idea and Development grants so there is no distinction between the two as well as setting the grant amount as up to \$100k. However, the Board can choose to award less as we have done in prior years. Previously, there was a \$50k limit for Idea grants and a \$100k ceiling for Development grants, but the Idea Grant level is thought to be becoming less enticing as most components of science have gotten more expensive. Also, we will migrate from an email process to a grant portal with end-to-end management. It will be much more user friendly for participants and also will give BSF better visibility as to the status of applications, which Melissa Huang demonstrated. Currently, we have 3 applications in progress with another 4 Intents to Apply. Half are new to BSF and also half are from biotech. Melissa commented that this indicates that Barth syndrome is an emerging interest; to see the science grow is great. Miriam Greenberg recommended asking the SMAB members and previous awardees to share BSF's Request for Grant Proposals with their networks. She recognized that the changes being made will be really great improvements to the overall process.

12. The meeting was adjourned by Jamie Baffa at 1:58 pm ET.

Respectfully submitted,

Brandi Dague

Secretary