

**MINUTES**  
**Barth Syndrome Foundation, Inc.**  
**Board of Directors Meeting**  
**July 29, 2025**

**Members in Attendance:**

Kate McCurdy	Board Member, Chair
Brandi Dague	Board Member, Secretary
Jamie Baffa	Board Member, Vice-Chair
Megan Branagh	Board Member
Steven Graessle	Board Member
Mark Greene	Board Member, Treasurer
Michelle Florez	Board Member
Emily Milligan	Board Member <i>ex officio</i> , Executive Director
Peter van Loo	Board Member
Nina Russell, MD	Board Member
Jonathan Stokes	Board Member
Stacey Woodward	Board Member

**Invited Guests**

Natalie Cohn	Controller, BSF
Melissa Huang, PhD	Staff, BSF
Laura Duvelius	Staff, BSF
Shelley Bowen	Staff, BSF
Lindsay Marjoram, PhD	Staff, BSF
Emily Madalinski	Staff, BSF

**Members in Not Attendance:**

BJ Develle	Board Member
Miriam Greenberg, PhD	Board Member

**Affiliate Leaders Attending as Observers**

Susan Hone	Barth Syndrome Canada
Christiane Hope	Barth Syndrome Canada

The meeting, held via Zoom, was called to order by Kate McCurdy on Wednesday, July 29<sup>th</sup> at 12:00 p.m. ET.

## 1. Welcome

Kate McCurdy welcomed all attendees and turned over to Brandi for minutes.

## 2. Meeting Minutes

- a. Secretary Brandi Dague presented the March 29, 2025, Grant Review board meeting minutes and the April 16, 2025 board meeting minutes for approval.

**MOTION** to approve the March 29, 2025, minutes as written was made and **APPROVED** unanimously.

**MOTION** to approve the April 16, 2025, minutes as written was made and **APPROVED** unanimously.

- b. One offline vote was entered into the record:
  - 1. On July 2nd, the Executive Committee approved up to 25 hours at \$325/hour (\$8,125) for a consultant to support Bill Pu's DoD gene therapy grant submission.
  - 2. Lindsay reported that the consultant was retained, the grant was successfully submitted and the consultant came in under budget at \$5,200.

## 3. Financials

- a. Natalie presented a draft of the current financials:
  - 1. Research grant expense reflects new awards made in 2025 and are lower than the full year forecast due to planned but outstanding milestone agreements
  - 2. Salaries to date are higher than 2024 due to new FTE for Development
  - 3. Investment portfolio value has increased \$300k to date in 2025, mostly reflecting market appreciation
  - 4. Current "Restricted" net asset balance of \$4M mostly held for "Therapy" development and a smaller amount for funding specific grants (from affiliates and CZI)
  - 5. Mark Greene: revised forecast will be presented at August 27<sup>th</sup> BoD meeting

## 4. Barth Syndrome Registry and Repository Update

Melissa provided an update on the progress that has been made, using CZI funding, on the migration of registry and repository. Key points included:

- a. Recently completed contract negotiations
- b. Registry migration from Invitae to Matrix is now underway but will be slower than anticipated due to 10+ years of data being transferred and opportunity to clean up the data as it's moved
- c. Following migration, registry will be beta tested with a small group of individuals, followed by refinement and then broad roll out.
- d. Included in the CZI budget are funds to rebrand the registry and create a new storefront home for it

- e. For the repository, BSF is waiting on VAI to provide logistics for sample shipment; Conference samples currently stored at Wayne State will be moved first

## **5. Biorepository and Registry (BRR)**

- a. Melissa Huang reviewed the evolution of BSF's biorepository and registry, highlighting new capabilities of the registry: electronic medical record (EMR) connectivity, timely addition of surveys, and multilingual access; and of the repository: unified storage, BSF ownership/oversight, LIMS system for real-time tracking of samples, and timely shipping of samples.
- b. With CZI grant funding available to upgrade BSF's BRR, Melissa reviewed internal business due diligence on platforms.
  - 1. Matrix identified as the most suitable platform for the registry solution (implementation of \$65k; ongoing fixed \$35k/annually + variable costs for surveys/translations)

## **6. Arrhythmia Project & Cardiac Natural History Registry**

- a. Lindsay Marjoram presented a brief overview of the difference in roles of the BRR versus the NYU registry role (broad versus narrow/clinical, respectively).
- b. Lindsay proposed thinking about BRR and ancillary registries as a hub and spoke model where BRR is in the center and niche/clinical registries are spokes with directional communication
- c. Assessment of NYU's challenges were shared and attributed to them having inadequate project management resources. Current research coordinator is departing this month, providing an opportunity for BSF to influence the profile for a new hire.
- d. Lindsay stated that both Pitt and JHMI are working on their IRBs, with BSF providing oversight, and that the NYU team is actively analyzing their Holter data. As a result, one participant was notified of arrhythmic events of which they were previously unaware.
- e. Emily Milligan mentioned that while BSF has distributed \$120k, NYU has spent \$210k, leaving risk for BSF financial exposure even though this contract was milestone-based and many milestones have not been met. There are risks, however, in terms of important, strategic relationships for BSF if funding were to be canceled

## **7. Elamipretide Update**

- a. Emily Milligan presented a timeline for elamipretide-related events in 2025 starting with 1/29/2025 PDUFA date delay and culminating with when we anticipate a response to Stealth BioTherapeutics' NDA Reconsideration request (first week of August)
- b. Kate McCurdy provided additional details on the Type A meeting itself, stressing that Stealth addressed all concerns present in the complete response letter issued in May and highlighted that she made pleas for broad labeling or, at bare minimum, inclusion of infant case reports on label. FDA was amenable to formal reconsideration request. Kate also highlighted the extraordinary work that Shelley Bowen has undertaken on the advocacy front.
- c. Shelley Bowen presented the following advocacy highlights:

1. 117 congressional meetings held since June 2025, with 91 outstanding meeting requests.
2. Georgia and South Carolina state delegation letters submitted; Florida in progress
3. Advocacy targets: congressional members of the following committees and caucuses: Senate HELP committee, House Energy and Commerce subcommittee on Health, GOP and Democrat Doctors Caucus, Mitochondrial Disease House Caucus
4. Total views on elamipretide news stories that have aired: 285.5k
5. Shelley thanked advocates that were on the call and also mentioned engagement of BSF scientific allies Drs. Vockley, Jefferies and Greenberg

**8. Strategic Planning Update**

- a. Jamie Baffa provided the following updates regarding strategic planning:
  1. Having a strategic plan in place before the end of 2025 is still a priority.
  2. The off-site retreat was rescheduled to October 17-19, 2025 due to the shifting elamipretide timelines and the need to focus on what was most important at the time.
  3. In the coming months, we will revisit the survey results and strategic planning conversations as a board as well as individually to dive in deeper.

Meeting was adjourned at 1:30 PM ET.

Respectfully submitted,

Emily Milligan  
Executive Director