

**MINUTES**  
**Barth Syndrome Foundation, Inc.**  
**Board of Directors Meeting**  
**December 10, 2024**

**Members in Attendance:**

Kate McCurdy	Board Member, Chair
Jamie Baffa	Board Member, Vice-Chair
Megan Branagh	Board Member
Brandi Dague	Board Member, Secretary
BJ Develle	Board Member
Miriam Greenberg, PhD	Board Member
Mark Greene	Board Member, Treasurer
Emily Milligan	Board Member <i>ex officio</i> , Executive Director
Nina Russell, MD	Board Member
Jonathan Stokes	Board Member
Peter van Loo	Board Member

**Invited Guests**

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

**Members in Not Attendance:**

Michelle Florez	Board Member
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**Affiliate Leaders Attending as Observers**

Michaela Damin	Barth Syndrome UK
Paola Cazzaniga	Barth Italia Onlus
Ralph Easterbrook	Barth Syndrome UK
Susan Hone	Barth Syndrome Foundation of Canada
Christiane Hope	Barth Syndrome Foundation of Canada
Florence Mannes	Association Syndrome de Barth France
Daniela Toniolo, PhD	Barth Italia Onlus

The meeting, held via Zoom, was called to order by Kate McCurdy on Tuesday, December 10th at 12:00 p.m. ET.

## 1. Welcome

- a. Kate McCurdy welcomed attendees and noted this meeting marked the beginning of a trial period through April 2025 where affiliate representatives would be invited to observe board meetings to improve global coordination.
- b. The Chair acknowledged the signing of confidentiality agreements by affiliate representatives and explained the need for confidentiality during board deliberations.

## 2. Meeting Minutes

- a. Brandi Dague presented the November 7, 2024 board minutes for approval.
  1. **APPROVED** as written

## 3. Treasurer Report

- a. Emily Milligan presented a revised financial format and forecast for 2024, highlighting:
  1. Current projected operating deficit of \$326,000, an improvement from the originally budgeted \$581,000 deficit
  2. Revenue improvements including:
    - \$100,000 recognized in 2024 from \$800,000 Chan Zuckerberg Initiative 5-year grant
    - Conference revenue exceeding budget by \$45,000
    - Additional major donor contributions
  3. Expense variations including:
    - Delay in hiring senior fundraising position to 2025
    - Unbudgeted advocacy expenses for FDA Advisory Committee meeting
    - Unbudgeted EUR 100,000 for Amsterdam Center of Excellence project
    - Delays in gene therapy and arrhythmia projects pushing expenses to 2025
- b. Mark Greene noted strong market performance and lowering interest rates have helped recover previous portfolio losses. These are not included in the figures above, as they are below the “operating income/loss” line.

## 4. 2024 Year in Review

- a. Emily Milligan and staff presented a comprehensive overview of achievements, all of which are aligned with BSF's mission of saving lives through education, advances in treatments, and finding a cure for Barth syndrome.
- b. Family Services Highlights
  1. Supporting 306 living affected individuals globally

2. Family Support Programs:
  - Family Services Fund helped 10 families attend conference (~\$30,000 in support from BSF)
  - Emergency Relief Program assisted 7 families facing extreme adversity
  - Expanded library of educational resources including feeding/nutrition guidance
  - Hosted educational roundtables on topics including infant care and research updates
- c. Advocacy Achievements Highlights
  1. FDA Advisory Committee Outcomes:
    - Secured 10-6 favorable vote on October 10 for elamipretide's efficacy in treating Barth syndrome
    - Mobilized 40 Barth advocates to attend
    - Helped train and support 15 speakers as presenters for the Open Public hearing
    - Generated 80 public docket comments
    - Achieved over 5,300 views of proceedings
    - Notable participation of FDA leadership including Dr. Patrizia Cavazzoni, Director of CDER; Dr. Peter Stein, CDER's Office of New Drugs; and Dr. Hylton Joffe, Office of Cardiology, Hematology, Endocrinology, and Nephrology
  2. Legislative and Regulatory Progress:
    - Secured H.Res 1025 designating April 5th as "Barth Syndrome Awareness Day"
    - Conducted 109 Congressional meetings during Rare Disease Week
    - Participated in White House Office of Science and Technology Policy event
    - Helped achieve FDA Priority Review designation for elamipretide
    - Maintained media presence in outlets including ABC, CBS, BioCentury, and STAT news, among others
- d. Research and Development Highlights
  1. Conference Achievements:
    - 89 Scientific & Medical attendees, with 33% first-time participants
    - 38 poster presentations
    - 8 different scientific sessions (with multiple speakers in each)
    - New initiatives including poster blitz, mentoring lunch, and CME sessions
    - 92% of attendees planning future collaborations
  2. Research Progress:

- Awarded \$800,000 5-year Rare As One grant from Chan Zuckerberg Initiative to help us build organizational capacity
- Secured \$40,000 NIH conference grant
- 29 peer-reviewed papers published on Barth syndrome, with 24% from SMAB members
- Conducted 7 IRB-approved research studies at our international conference, with 200+ appointments
- Advanced Amsterdam Center of Excellence project
- Published first registry journal article with pending future publication on survival curve analysis with New York University

e. Communications and Engagement Highlights

1. Digital Outreach:

- Sent 128 coordinated emails with 180,824 opens and 65,400 clicks
- Achieved significant social media growth:
  - \* Facebook: 55.6k views, 25.7k reach
  - \* Instagram: 22.2k views, 6.9k reach
  - \* LinkedIn: 40,225 impressions

2. Enhanced Communication Tools:

- Implemented editorial calendar across all programs
- Developed specialized plans for conference and FDA Advisory Committee
- Created new annual report and conference formats and materials
- Launched regular "First Thursday" monthly on-line updates

f. Fundraising Achievements Highlights

1. Campaign Results:

- Giving Tuesday raised over \$105,000
- Conference sponsorships/activities generated \$207,000
- Happy Heart Week surpassed \$140,000 and reached \$1M lifetime
- Hockey with a Heart partnership raised over \$100,000

2. Volunteer-Led Initiatives:

- Multiple successful individual fundraisers including:
  - \* Cocodona 250 (\$6,000+)
  - \* Steven W's birthday celebration (\$9,000+)
  - \* Maryland Ironman (\$7,000+)

**5. #BarthPROGRESS Campaign**

- a. Emily Milligan presented communications strategy leading up to and surrounding January 29, 2025, FDA PDUFA date (by which FDA decision on elamipretide will be rendered):

1. Campaign phase

- November: "Gratitude for Progress" - reflecting on community resilience
  - December: "Gift of Progress" - recognizing donor support
  - January/February: "Champions of Progress" - honoring key contributors
2. Core objectives:
    - Reaffirm BSF's dedication regardless of FDA decision
    - Ensure transparent stakeholder communications
    - Maintain balanced messaging celebrating progress while acknowledging challenges
  3. Decision preparation:
    - Developed response strategies for approval and non-approval scenarios
    - Created communications plan for various approval conditions including access to drug and insurance implications
    - Established timeline for stakeholder updates including families, affiliates, and partners
  4. The Board reviewed timing sequence from pre-decision preparations through post-decision community engagement
  5. Staff introduced financial implications for both 2024 and 2025, which were \$10.5k and \$60k plus staff time, respectively

## **6. Giving Tuesday Report**

- a. Emily Madalinski reported approximately \$105,000 raised
- b. Highlights included:
  1. Over 40 individual fundraisers
  2. New SMS communication channel implemented
  3. Board match of \$10,000; with 100% board giving
  4. 100% staff giving
  5. Personalized acknowledgments for gifts over \$200 and all Barth families irrespective of amounts

## **7. Executive Session**

- a. The meeting moved to executive session at 1:25 PM ET for discussion of staff compensation.
- b. Staff and affiliates exited the meeting.

Respectfully submitted,

Brandi Dague  
Secretary