MINUTES

Barth Syndrome Foundation, Inc.

Board of Directors Meeting

October 17, 2022

Members in Attendance:

Andrew Buddemeyer	Board Member
Brandi Dague	Board Member
Emily Milligan	Board Member *ex officio*, Executive Director
Jamie Baffa	Board Member, Secretary
Kate McCurdy	Board Member, Chair
Kevin Woodward	Board Member, Treasurer
Mark Greene	Board Member
Maryanne Chrisant, MD	Board Member
Megan Branagh	Board Member
Michelle Florez	Board Member
Florence Mannes	Board Member
Miriam Greenberg, PhD	Board Member
Peter van Loo	Board Member

Invited Guests

Bill Belscher	Audit Committee, Chair
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	Family Services, BSF
Steve McCurdy	Finance & Investments Committee

Members in Not Attendance:

B.J. Develle	Board Member
Nina Russell, MD	Board Member

AGENDA

1. *Welcome*
2. *Update: Research and Development 2022 Organizational Priorities*
3. *Vote: Gene Therapy Novel Capsid Program*
4. *Update: Gene Therapy AAV9 BTGC Initiative*
5. *Update: Enzyme Replacement Program*
6. *Vote: Arrhythmia Retrospective Study*
7. *Update: Regulatory Affairs*

8. *Update: Treasurer Report*
9. *Vote: Board Minutes*
10. *Other Business*

The meeting, held via Zoom, was called to order by Kate McCurdy on Monday, October 17 at 11:35 a.m. ET.

1. Welcome
    a. Kate McCurdy welcomed board members and invited guests
2. Update: Research and Development 2022 Organizational Priorities
    a. Erik Lontok reviewed the programs and initiatives that BSF has pursued so far this year
    b. Included discussion regarding how the Arrhythmia Project interacts with various programs and initiatives across BSF's research and development portfolio
    c. Similarly, the Barth Syndrome Registry and Repository is another effort that connects to various programs and initiatives across the portfolio
    d. Erik forecasted that BSF is improving how we analyze and visualize the data contained in this registry given how critical it is in identifying eligible participants for clinical studies and trials
    e. The registry will also serve as a foundation as we link clinical efforts through a global unique identifier (GUID)
    f. Melissa Huang then provided an introduction to and recap of the Mayo Clinic Study to develop US-based expertise in conducting an MLCL:CL test for Barth syndrome
    g. The goal is to recruit 30-40 US-based English-speaking children and adults with Barth syndrome
    h. Mayo Clinic has noted a discrepancy in their in-house patient database and have not had success in recruiting participants thus far
    i. Barth syndrome has agreed to lead a revised recruitment strategy given their current challenges and that this study requires 25-32% of all known individuals with Barth syndrome in the United States to participate
    j. Melissa then reviewed the Mayo Clinic IRB-approved methods for recruitment and how the foundation intends to recruit within these requirements
    k. The foundation is asking for approval for additional recruitment methods
    l. This will involve significant effort from foundation staff, but will have a high return given the potential to conduct Research / GUID verification
    m. Emily Milligan reinforced that this is a bigger initiative in way of staff time than when it was originally approved by the board in May
3. Vote: Gene Therapy Novel Capsid Program

a. Erik shared a brief recap of BSF's overall gene therapy efforts and introduced Clive Spiegler

b. Clive reviewed achievements since the last board meeting and upcoming milestones

c. Since the last board meeting, Myo-AAV novel capsid has emerged as the target novel capsid based on feedback from AskBio and the recommendation to optimize cargo and screen muscle-specific promoters

d. Clive then reviewed the novel capsid timeline, including nonclinical, technical, clinical, and regulatory activities and the critical path

e. Expectation is to select a final novel capsid candidate in April 2023, at a similar time BSF expects to receive a decision from the BGTC regarding potential support of a more traditional AAV9 approach to gene therapy for Barth syndrome

f. At that point the decision will be how far BSF wants to take the novel capsid approach

g. Should BSF move forward with BGTC funding, the recommended time to "park" the novel capsid program would be following a pre-IND meeting

h. Kate noted that while AAV9 is tried and true, the reason we are considering a novel capsid is that AAV9 has potential toxicity issues at high dosage levels (like those needed to treat Barth syndrome) that need to be addressed

i. Discussion followed

j. Clive then reviewed the funding required to move to the next stage of this process, final novel capsid candidate selection

    i. $68,750 for the implementation of expert reviewed candidate optimization work to get to final vector candidate

    ii. $10,000 for the expert recommendation on manufacturing strategy and choice of manufacturer

    iii. $25,000 for the implementation of activities to next milestone and refinement of strategy, plan., and funding to subsequent milestones

k. Gene Therapy Resource Program (GTRP) is a potential source of funding for manufacturing and other aspects of the novel capsid program, however, given GTRP is scheduled to end in Q4 2023 (unless renewed) there is uncertainty about its potential for BSF

l. Kate and Erik discussed the background and work that has allow BSF to arrive at this point

m. This program is running parallel to BSF’s typical grant review process and before the next stage of this process, the pre-IND meeting, BSF will use a more typical external review before moving forward

n. Discussion followed

o. Decision made to postpone the vote on this funding for a short period of time

p. In the interest of time, the board skipped the discussion on enzyme replacement therapy

4. Vote: Arrhythmia Retrospective Study

    a. Emily reviewed how the arrhythmia study enables the development of the registry at NYU that can then be used for other studies and applications

    b. BSF has been negotiating with NYU over the ongoing maintenance and ownership of the registry

    c. After negotiations, the overall cost has increased by $19,162 to a total of $350,616 across two years

    d. These costs will be offset by 100,000€ donated each year by Barth France

    e. Highlights of the contractual terms include

        i. BSF retains final decision making authority related to the registry

        ii. BSF will have a non-exclusive, worldwide, perpetual, sublicensable right to the registry

        iii. BSF retains right of access

        iv. If the registry with NYU terminates, NYU will deliver all documents, work product, and other materials to BSF and permanently erase all information from their systems

    f. There are two outstanding items regarding proposed costs:

        i. Maintaining the registry beyond the terms of the arrhythmia project

        ii. Depositing data from other research into the registry

    g. Discussion followed

    h. Emily noted that this will be a milestone-driven agreement so expenses will hit the BSF budget once certain milestones have been achieved

    i. **APPROVED**: Contingent approval for BSF to spend $350,616 on the retrospective arrhythmia project and natural history registry

5. Adjournment

    a. Kate asked board members to share any updates to the board minutes

    b. Kate adjourned the meeting at 2:00 p.m. ET

Respectfully submitted,

Jamie Baffa – Secretary