

**Minutes of the
BARTH SYNDROME FOUNDATION
BOARD OF DIRECTORS
July 8, 2008**

The Board of Directors of The Barth Syndrome Foundation, Inc. (BSF) met on Tuesday, July 8, 2008, via teleconference. All members of the Board of Directors were present, as follows:

Members in Attendance

Stephen B. McCurdy (Steve)	Chairman of the Board, Chief Financial Officer
Valerie “Shelley” Bowen	Board Member, President of BSF
Michaela Damin	Board Member
Stephen Kugelmann (Steve)	Board Member
Katherine R. McCurdy (Kate)	Board Member
Susan S. Osnos (Suze)	Board Member
Susan V. Wilkins (Sue)	Board Member
Randy Buddemeyer	Board Member

Additional Invited Attendees

Matthew J. Toth, PhD, BSF Science Director joined the meeting to present an update on ongoing Barth syndrome research as well as an overview of the Scientific/Medical portion of BSF’s 2008 Conference.

Jan Kugelmann, 2008 Conference Coordinator joined the meeting to present an overview of the planning of BSF’s 2008 Conference.

Lynda Sedefian attended the meeting in the role of Secretary to the Board of Directors.

Steve McCurdy called the meeting to order.

RATIFICATIONS

It was **PROPOSED** and **SECONDED** that the Board of Directors accept and ratify the November 17-18, 2007 Minutes of the Board of Directors, as submitted.

RESOLVED, that the Board of Directors officially accept and ratify the November 17-18, 2007 Minutes of the Board of Directors, as submitted.

It was **PROPOSED** and **SECONDED** that the Board of Directors accept and ratify the January 24, 2008 Minutes of the Board of Directors, as submitted.

RESOLVED, that the Board of Directors officially accept and ratify the January 24, 2008 Minutes of the Board of Directors, as submitted.

It was **PROPOSED** and **SECONDED** that the Board of Directors accept and ratify the February 16, 2008 Minutes of the Board of Directors, as submitted.

RESOLVED, that the Board of Directors officially accept and ratify the February 16, 2008 Minutes of the Board of Directors, as submitted.

It was **PROPOSED** and **SECONDED** that the Board of Directors accept and ratify the March 30, 2008 Minutes of the Board of Directors, as submitted.

RESOLVED, that the Board of Directors officially accept and ratify the March 30, 2008 Minutes of the Board of Directors, as submitted.

It was **PROPOSED** and **SECONDED** that the Board of Directors accept the November 17-18, 2007 and February 12, 2008 Executive Committee Minutes, as submitted.

RESOLVED, that the Board of Directors officially accept the November 17-18, 2007 and February 12, 2008 Executive Committee Minutes, as submitted.

Goal: *To inspire and make effective use of an organization dedicated to reaching our vision ...A world in which no one will suffer or perish from Barth syndrome*

EXECUTIVE DIRECTOR

The Board of Directors is very pleased to announce that BSF has hired Linda Stundis as its first Executive Director. Linda is a strong but quiet leader who offers an excellent fit with BSF at this stage of our development. She is bright and has a great ability to listen, organize and build effective teams. In addition, she has a real passion for helping children and young people. She fully appreciates the opportunity to build upon the hard work that has brought us this far and the great potential to make a real difference. Linda will work out of her home in Massachusetts where she lives with her son. She will assume her new position on July 14, 2008 and is looking forward to joining us at the BSF International Scientific, Medical and Family Conference in Clearwater, Florida from July 21-26, 2008.

Goal: *To stimulate the development of successful treatments for Barth syndrome, a multi-system disorder, and enable their delivery.*

2008 INTERNATIONAL SCIENTIFIC, MEDICAL & FAMILY CONFERENCE

Family Registration

- There are a total of 31 families who have registered to attend to date
 - 30 pedigrees / 36 affected individuals
 - 11 new affected individual attendees

Scientists/Physicians/Clinicians

- Over 50 science and health professionals will attend
 - 90% are either speaking or presenting posters
- Eight SMAB members will attend

Travel Stipends for Presenters at Poster Session

Matthew J. Toth, PhD, Science Director advised the Board that there will be 17 posters presented at the 2008 Poster Session. It was **PROPOSED** and **SECONDED** that the Board of Directors accept and ratify the Executive Committee's recommendation to award eight travel stipends for poster presenters to attend BSF's 2008 Conference for a total budget of Six Thousand Dollars (\$6,000US).

The purpose of adding the posters sessions, beginning with our last conference, is three-fold:

1. to increase the number of topics and the amount of data that could be presented at any single conference (especially since we meet only every other year);
2. to allow a venue for younger scientists and physicians to present their work; and
3. to allow anyone, young or experienced, a way to present: a) good work that may not be of a scale yet to warrant full oral presentation, b) additional data to support an oral presentation in more detail, or c) work that is very interesting but only to a subset of the people who are likely to attend the meeting.

RESOLVED, that the Board of Directors officially accept and ratify the Executive Committee's recommendation to award eight travel stipends for poster presenters to attend BSF's 2008 Conference for a total budget of Six Thousand Dollars (\$6,000US).

Barth Syndrome Registry & DNA Bank

Carolyn Spencer, MD, Co-Principal Investigator of the Barth Syndrome Registry & DNA Bank will be distributing the following consent forms to be completed prior to the Conference:

- Barth Clinical Research Project Informed Consent
- Barth Syndrome Registry and DNA Bank Informed Consent
- Registry Intake for New Enrollees
- Registry Update (for those previously enrolled)

Math Skills Development Project – Cognitive Development

Michele Mazzocco, PhD, Principal Investigator is continuing her research project into the cognitive development of Barth syndrome. The purpose of this project is to help understand the development and cognitive and academic skills of young children with Barth syndrome and to assess the level of these skills in preschool and primary school children with Barth syndrome.

Barth Syndrome Clinics/Consultations

Baseline information collected provides the clinician with the tools to justify a potential trend that should be investigated. Data collected in a precise and consistent manner further validates an assertion of the findings one would anticipate from a control/healthy population. From there, successful approaches can be benchmarked and further outlined to improve palliative treatment protocols and clinical approach in patient care management. Clinics will consist of the following sub-specialties:

- Cardiology
- Education Round Table Discussions
- General Inquiries
- Genetics
- Metabolism and Nutrition
- Neurology
- Occupational Therapy/Physical Therapy

Webcast of Scientific/Medical and Family Sessions

Shelley Bowen is working with Bright Circle, Inc., Lucas Productions and WebEx in an effort to have both the Scientific/Medical and Family Sessions webcasted to our broader audience on July 24-25, 2008. More information will be forthcoming as this project is worked through.

Ground Transportation

Leslie Buddemeyer has been working on obtaining ground transportation for off-site activities that have been arranged. After a thorough discussion, it was **PROPOSED** and **SECONDED** that the Board of Directors approve a budget of up to Three Thousand Dollars (\$3,000) to cover ground transportation for these off-site activities.

RESOLVED, that the Board of Directors officially accept and ratify a budget of up to Three Thousand Dollars (\$3,000) to cover ground transportation for these activities.

Venue Location for 2010 International Scientific, Medical and Family Conference

Jan Kugelmann presented an overview of her search for venue location for BSF's 2010 conference. The search consisted of visiting sites in both Orlando, Florida and Boston, Massachusetts. Pending further discussion with Linda Stundis and the Board, there was a consensus among the Board that BSF's 2010 Conference should be held at the Hilton located in the Walt Disney World Resort, Lake Buena Vista, Florida, but that we consider a different time of year in late October/early November. It was proposed that we look at October 25-30, 2010.

Goal: *To encourage, guide and fund additional research to improve diagnosis and treatment and ultimately to develop a cure for Barth syndrome.*

UPDATE ON BARTH SYNDROME RESEARCH

Matthew J. Toth, PhD, Science Director informed the Board that there are nine ongoing research projects stemming from BSF's 2007 grant cycle and four ongoing research projects stemming from BSF's 2006 grant cycle. Almost all the grant recipients from 2006, 2005, and 2004 will be speaking at BSF's 2008 Conference.

Nemours Children's Clinic (Wilmington, DE)

The diagnostic group at Nemours Children's Clinic (Wilmington, DE) is a clinical group with limited funds to pursue research. Last year they expressed a need for DNA samples to explore the frequency of mutations (SNPs) at the tafazzin locus. They identified two commercial sources of DNA samples which will augment their current efforts of maintaining the tafazzin gene mutation database and allow for publication.

It was **PROPOSED** and **SECONDED** that the Board of Directors accept and ratify the decision to award One Thousand Three Hundred Twenty Two Dollars (\$1,322) to the diagnostic group at Nemours Children's Clinic (Wilmington, DE) to support its purchase of additional DNA samples to explore the frequency of mutations at the tafazzin locus.

RESOLVED, that the Board of Directors officially accept and ratify the decision to award One Thousand Three Hundred Twenty Two Dollars (\$1,322) to the diagnostic group at Nemours Children's Clinic (Wilmington, DE) to support its purchase of additional DNA samples to explore the frequency of mutations at the tafazzin locus.

Covance - Antibody Update

BSF has spent Five Thousand Dollars (\$5,000) with a private company (Covance) to provide antibody reagent (rabbit and chicken) against human tafazzin—an important tool. Results were somewhat disappointing—antibody works only against overproducing cell lines and not with lymphoblasts. It is suspected that tafazzin protein expression in tissues or in cell culture is naturally very low. Current material will be useful for publications, but it was hoped that the antibodies would be more sensitive.

Dr. Toth has encouraged that the laboratories look into “phage antibodies” using the BSF grant process and BSF is currently working with others to increase utility. This issue will also be discussed at the upcoming Scientific, Medical and Advisory Board meeting scheduled for Saturday, July 26, 2008 at BSF’s 2008 Conference.

Taconic/Artemis - Tafazzin Knockdown Mouse Update

Taconic/Artemis has completed steps 1 and step 2 of the process to knockdown tafazzin expression. Only one shRNA sequence out of six showed a decent response (~ 66%) which is borderline as to whether to proceed. Dr. Toth favors looking at three more shRNA sequences to see if we can find something better, but this will require approval of additional funding.

Unfortunately there are no real alternatives if we want a “Barth mouse” and we are at a decision point—do we go forward or stop now. Dr. Toth has asked Taconic/Artemis to have some of the cell samples sent to another laboratory to conduct a cardiolipin analysis to see if the cardiolipin levels have changed.

The Board of Directors has asked Dr. Toth to contact Taconic/Artemis to request appraisals for additional numbers of the shRNA sequence to determine whether BSF should move forward with the continued funding of this project. It was also suggested that this topic be added to the agenda of the Scientific and Medical Advisory Board’s upcoming meeting scheduled for Saturday, July 26, 2008, and that Dr. Toth collaborate with a multi-disciplinary team to brainstorm as to whether there are any alternatives left untried in the development of a tafazzin knockdown mouse.

Clinical Fellowship

The BSF Board agreed that there is a deficiency in translating scientific information into relevant clinical investigations. BSF has received a request to support a clinical fellowship focused on Barth syndrome. Usually these clinical fellowships are set up in conjunction with a Medical School/NIH which provides the mentors and stipends of \$50K to \$70K per year for 1-2 years. It may be possible to partner with NIH to help with costs and mentoring.

The Board of Directors agreed that this type of fellowship program is an excellent concept and would like to take the time to think through what the priorities of this type of a program would be and whether it would be beneficial for BSF to adopt such a program. It was agreed that Dr. Matt Toth and Kate McCurdy will take the time to investigate this type of program and will come back to the Board in the fall of 2008 with a formal proposal.

VARNER AWARD FOR PIONEERS IN SCIENCE AND MEDICINE

Sue Wilkins submitted to the Board of Directors the 'Selection Criteria for the Barth Syndrome Foundation Varner Award for Pioneers in Science and Medicine' for review and approval, as follows:

1. The recipient may be an MD or a PhD. He/she will be involved in Science and or Medicine related to Barth syndrome in a way that strongly supports our mission - advancing a treatment and a cure for Barth syndrome
2. The award will be presented for at least 10 years at each of the next five BSF International Scientific and Family Conferences
3. The Varner Family, with the help of the BSF Board of Directors, the Presidents of the BSF International Affiliates, the BSF President, the BSF Executive Director, and the BSF Science Director, will recommend a potential candidate for the Varner Award to the BSF Board of Directors who will make the final decision on the recipient.
4. We aim to choose and announce the Varner Award recipient approximately one year before the BSF conference to assure the recipient can attend.
5. The Varner Award recipient will be given a \$500 honorarium at the time the award is given and an etched Tiffany crystal bowl.
6. The Varner Award recipient will have his/her travel expenses to the BSF conference, as well as up to three nights of lodging, funded from the Varner Fund.
7. The BSF Executive Director will notify the Varner Award recipient that he or she has been given the award, and a member of the Varner family will present the award at the BSF conference.
8. The Varner Award recipient will be expected to attend the conference and give a short acceptance speech at the Awards Event.

It was **PROPOSED** and **SECONDED** that the Board of Directors accept and ratify the recommended selection criteria for recipients of the Varner Award for Pioneers in Science and Medicine, as outlined.

RESOLVED, that the Board of Directors officially accept and ratify the recommended selection criteria for recipients of the Varner Award for Pioneers in Science and Medicine, as outlined.

ADJOURNMENT

The meeting was adjourned at 11:00 PM EST. The next scheduled Board of Directors meeting will be held at an off-site location on Friday, November 14, 2008 thru Monday, November 17, 2008. The next scheduled Executive Committee meeting will be held on Tuesday, September 16, 2008 @ 8:30 PM EST via teleconference.

Respectfully submitted,



Lynda M. Sedefian
Secretary to the Board