



## Policy on Clinical Trials and Clinical Studies

Adopted as of September 19, 2013

The mission of the Barth Syndrome Foundation (BSF) is: *Saving lives through education, advances in treatment, and finding a cure for Barth syndrome.* In order to fulfill that mission the membership of BSF will necessarily have to be involved in clinical trials or clinical studies as they occur. BSF seeks to encourage voluntary participation in clinical trials by its membership in a general sense, but is prohibited from endorsing any particular trial or study. BSF will strive to provide as much information to its membership as it is able, and BSF will advertise the availability to voluntarily participate in clinical trials or studies as they occur. In the final analysis the decision to participate in clinical trials or studies is up to the individual or the individual's parents/guardians.

Any reputable clinical trial or clinical study will require the approval of an Institutional Review Board (IRB). The IRB is a committee of individuals charged with overseeing the clinical study that is being performed at our under the direction of a particular institution, like a University or Medical Center.

1. All researchers or institutions requesting BSF to help them in performing a clinical trial/study will have to submit documentation of their approved IRB application and a copy of the official approval letter.
2. BSF's Science Director will forward all relevant information to BSF's Scientific and Medical Advisory Board (SMAB) or to a subgroup of the SMAB for an evaluation as to whether the proposal requires BSF's assistance (*see 3 below*). This subgroup should be composed of the SMAB chair, BSF's Science Director, and at least two other SMAB members.
3. BSF's Science Director will report on the comments of the SMAB and/or the SMAB subcommittee to BSF's Executive Committee.\* The Executive Committee will decide to approve or to not approve. Approval will commit BSF to:
  - a. Posting an advertisement on the listserv for families stating that a researcher is asking for assistance with a Barth syndrome related project. The post will not be an outright recommendation by BSF, but it will take the form of an advertisement. The IRB-approved flyer and/or letter from the researcher will be attached to the post.
  - b. Posting a similar advertisement of the project on BSF's website.
  - c. Posting a similar advertisement in BSF's newsletter if appropriate/expedient.
4. BSF's Science Director will communicate to the relevant parties the decision of BSF's Board of Directors.

\*When a study involves any direct patient interaction, the entire BSF Board of Directors will be asked to approve/deny such requests.