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2017 RESEARCH GRANT APPLICATION GUIDELINES AND REQUIREMENTS

The Barth Syndrome Foundation, Inc. (BSF) and its international affiliates announce the availability of funding for basic science and clinical research on the natural history, biochemical basis, and treatment of Barth syndrome. **There are two basic categories: IDEA** grants for 1-2 years and **DEVELOPMENT** grants for 2-3 years with budgetary maximums of US \$50,000 or \$100,000, respectively. Although BSF will consider any research proposal related to Barth syndrome, it is particularly interested in supporting research in the areas identified by **REQUEST FOR APPLICATIONS (RFAs)** that are posted on its website. RFAs for work in clinical/scientific areas that BSF considers to be high priority areas of investigation may have increased budgetary maximums and other requirements (see the BSF website for details about any RFAs). Applications responding to RFAs will be given preferential consideration in the BSF Research Grant Program.

BSF's Research Grant Program now requires all applicants to be independent investigators (*e.g.*, faculty appointment). Postdoctoral fellows cannot apply. BSF allows young, non-tenured investigators to include in their submitted budget up to 75% of the total grant amount as PI salary. In addition, for those clinical applications where volunteers must travel to a clinical research site, these travel expenses will be handled separately and will be excluded from the budget maximums mentioned above. We encourage independent investigators at all professional levels to submit their best ideas. There are no geographical limitations to this funding.

Each grant application submitted to the Barth Syndrome Foundation, Inc. (BSF) must: be no more than 6 pages in length for the Research Strategy section (including figures and tables), be single-sided, use a font size of at least 11 points for the text, a maximum of 6 lines/inch, and have margins of 0.5 inch. Figures and tables may use smaller type but must remain easily legible. The following will NOT be included in the 6-page limitation for the Research Strategy section: Information Form, the Budget Form/Table, Abstract, IRB or IACUC approvals if applicable, resources and environment, project personnel, references, budget narrative, and biographical sketch. Applicants should also provide a suggested list of 3 external reviewers, and also note any potential reviewers (external or internal to BSF) whom you wish to be excluded from the evaluation process.

Letter of Intent: The letter of intent is a one-page document describing the applicant's research plan/idea in applying for funding. The letter of intent is **mandatory** for **Development** grant applications and for applications answering specific **Request For Applications (RFAs)** advertised by the BSF Research Grant Program. The letter of intent is **optional** for **Idea** grant applicants who would like to receive feedback from BSF. The deadline for the letter of intent is September 7, 2017, and it must be sent to the BSF Science Director at the address provided. It is recommended that the letter of intent be submitted as soon as possible to allow ample time for BSF to provide feedback and (assuming the feedback is positive) for the applicant to assemble the proposed application. The purpose of the letter of intent is to allow for evaluation by a subset of the BSF Scientific and Medical Advisory Board, which will provide feedback to the applicant about the suitability of their proposal for the BSF Research Grant Program. BSF will endeavor to provide this feedback within 2 weeks of receipt of a letter of intent. Although the feedback will be intended to provide guidance on the suitability of the proposed research for possible funding, this feedback will not be binding on any final funding decisions of the BSF Research Grant Program.

Each grant application *must* contain the following elements to be considered complete:

1. A completed Information Form (please use the template provided). In this form, the applicant must choose to be considered for an **Idea** grant or a **Development** grant. If applying for a **Development** grant or if applying to a specific **RFA**, a "letter of intent" must be sent to BSF no later than September 7, 2017. A "letter of intent" is optional for **Idea** grant applications and for applications not responding to a specific **RFA**.
2. A completed Budget Form/Table (please use the template provided).
3. An Abstract summarizing the project, including a statement of what issue(s) the project addresses, a description of why the work is important to either the furthering of knowledge about Barth syndrome or the treatment of individuals with the disorder, and a description of how the project will be accomplished. (*Note:* this abstract may be used for BSF publicity purposes). (1 page or less)
4. The following Research Strategy section, which has a total limit of 6 pages. Section lengths are recommendations.
 - a. A section presenting the specific aims of the project, stating both the objectives and the hypotheses to be tested in the project. (1 page)
 - b. A discussion of the background and significance of the work proposed, including a critical evaluation of previous research and existing knowledge, specifically identifying the gaps that the project is intended to fill, and explicitly stating the importance of the proposed research. (1 page)

- c. A *thorough* summary of research design and methods, describing the experimental design and methods that will be used to accomplish the specific aims. Include how the data will be collected, analyzed and interpreted. Present any preliminary data and/or findings. Describe any new methodology and its advantage over existing techniques. Discuss any potential difficulties and limitations of the proposed procedures and what alternative approaches could be used to achieve the project's aims. Provide a timetable for the project. (4 pages)
5. A statement indicating whether human subjects or vertebrate animals will be involved in the research. If the project will include either or both, then additional documentation will be required by BSF, as stated below. (less than a page)
6. A description of the resources and environment available for the project. This should include facilities, laboratory space and major equipment that are pertinent to the project. Also describe any procedures, materials or situations that may be hazardous to personnel and the planned precautions to be exercised. (1 page or less)
7. A list of project personnel (name, title, institution, and role on the project), including the percentage effort that each person is expected to devote to the project. (less than a page)
8. A list of references.
9. A detailed budget narrative section for the proposed project is required. This budget section explains the Budget Form/Table of section 2. This budget narrative fully describes and justifies the expenses. Grant funds will be disbursed by BSF semi-annually, based on this budget. The start of the fiscal year for the grant will be the date the first check is issued—usually April of the year following the application deadline. Unless you specifically state otherwise, it will be assumed that one-half of the first-year funds will be disbursed (and spent) in each of the two six-month periods comprising the first fiscal year for that grant. (If you would like to request disbursement at a different rate, then a separate budget form for each six-month period must be completed.)
10. A biographical sketch of the application (NIH format is fine).

Funding requests may be as much as **US \$50,000 for Idea grants and US \$100,000 for Development grants in total** (direct + indirect costs) **and may be larger for RFA applications as detailed in the RFA description**. All budgets will be scrutinized for allowable costs (see “Disallowed budget items” below). Applicants should only consider budget item requests that are necessary for the completion of the project. The funds will be payable over 1-2 years for **Idea** grants and 2-3 years for **Development**

grants; the duration of **RFA** funding will be stipulated in the RFA itself. Indirect costs included in the project are limited to 10% of the direct costs.

In the interest of having a fair competition of ideas, there will be two budget categories when considering the Salary and Benefits expenses for the PI—the “Young Investigator” category and the “Established Investigator” category. In the “Young Investigator” category (*e.g.*, when the applicant is non-tenured, assistant professor, adjunct employee, etc.) the Salary and Benefits of the PI cannot amount to more than 75% of the direct costs. If the application is from an “Established Investigator” (*i.e.*, associate professor and higher, permanent employee), then no more than 10% of the direct costs may be budgeted Salary and Benefits for the PI. The “Established Investigator” category may include the Salary and Benefits of post-doctoral students, graduate students, laboratory technicians, etc.; however, in all cases the subtotal of the Salary and Benefits category cannot exceed 75% of the direct costs.

The categorization of “Young Investigator” or “Established Investigator” is only for budget purposes—all applications within each BSF Research Grant cycle are competitively evaluated based on individual scientific merit with all others in that cycle.

Clinically-oriented applications that involve the testing or treatment of Barth syndrome individuals often have significant budget outlays for the travel of volunteers to certain clinical research sites—an expense that is not directly pertinent towards evaluating the scientific merit of the application. For this reason, any application which includes the travel of research volunteers to specific clinical research sites may submit a separate budget sheet that provides the details of these travel expenses (include transportation, room, and board). This **Travel Expenses Budget** for volunteers will not be part of the total budget limitations, and these expenses will not be used in evaluating the merit of the application. However, the total of these “travel expenses” cannot exceed \$25,000 for either an **Idea** grant or a **Development** grant. BSF may fund these travel expenses directly or it may develop alternative arrangements with the grant recipient to accomplish the same task. Applicants submitting this type of clinically-related projects should include a separate Travel Expenses Budget sheet detailing these expenses.

Salary expenses for persons other than the PI can be included; however, in all cases, the “Salary and Benefits” subcategory subtotal shown on the Budget Form/Table (PI and non-PI salaries) cannot exceed 75% of the direct costs. Please note that for “Established Investigators”, there is a PI Salary and Benefits limit of 10% of the direct costs as detailed above.

Requests for “no-cost” time extensions of existing approved grants must be justified in writing and submitted to the BSF Science Director 90 days before the next scheduled payment of funds. Decisions about granting “no-cost” time extensions are at BSF’s discretion.

Disallowed budget items in the BSF Research Grant Program include: travel expenses of the PI or colleagues to conferences or meetings, publication fees, and personal

expenses. Other BSF funds may be available to help with worthy research expenses; therefore, successful grant recipients are urged to contact the Science Director for more information.

In addition to the application described above, several specific documents are required, if applicable. These documents are not counted as part of the 6-page Research Strategy limitation:

1. Biographical information regarding the principal investigator(s), preferably in NIH format. If needed, an explanation of this format can be found at: <http://grants.nih.gov/grants/forms/biosketch.htm>
 2. Regulations require that all institutions maintain appropriate policies and procedures for the protection of human subjects. If applicable, please provide a copy of the informed consent form for any human subjects for the proposed project as well as proof of current or pending approval by an Institutional Review Board (IRB) (or a similar oversight group). (NOTE: Funding for the project, if the application is approved, will not be released by BSF until the final IRB approval has been secured and a copy, including the approved informed consent form, has been received by BSF.)
 3. In keeping with BSF policy, all proposals must conform to regulations for the safe and humane treatment of animals. If applicable, please provide proof of current or pending project approval by the Institution's Animal Use and Protection Committee (or a similar oversight group). (NOTE: Funding for the project, if the application is approved, will not be released by BSF until the final Animal Use and Protection Committee approval has been secured and a copy has been received by BSF.) (*See Addendum A for a general statement from BSF*)
 4. A list of three individuals (names, titles, addresses, and telephone and e-mail contact information) whom you think would be appropriate outside reviewers for your grant proposal. These individuals must be qualified to review your application, have not worked with you during the last three years, and have no conflict of interest. Conversely, if there are individuals you do not want to review your application, please note this in the application.
- Only proposals written in English can be considered.
 - Incomplete applications will not be accepted.
 - Resubmission of an application from a previous year will not be accepted unless the new application is substantially changed in the opinion of the BSF Science Director.
 - An electronic version of the full application (in .doc format or in .pdf format, please) and any attachments must be sent to the BSF Science Director, Matthew J. Toth,

PhD, at matthew.toth@barthsyndrome.org or mtothbsf@comcast.net by **Tuesday, October 31, 2017** (absolutely NO extensions).

- In addition, one paper copy of the grant application, with appropriate signatures, should be received by **Tuesday, October 31, 2017** (absolutely NO extensions) at the following address:

Matthew J. Toth, Ph.D.
Science Director
Barth Syndrome Foundation, Inc.
132 Creemer Avenue
Iselin, NJ 08830
USA

Submissions by fax will not be accepted, and **there will be no extensions of the deadline.**

- Acknowledgement of BSF's receipt of the grant application will be sent by e-mail to the principal investigator within 7 days of the receipt of the document.
- Grant applications will be reviewed by the BSF Scientific and Medical Advisory Board (and selected outside reviewers), and recommendations will be forwarded to the BSF Board of Directors for their decision to fund or not to fund. **The applications will be scored on the basis of:**
 1. Importance and impact – the extent to which the research outlined could make a significant contribution to the understanding of Barth syndrome or the treatment of the disorder
 2. Feasibility – the qualifications of the investigator(s) to carry out the proposed work based on expertise, experience, and commitment; as well as the availability of all the elements required for the project (such as technology, reagents, and human subjects)
 3. Prospect of Long-term Funding – the likelihood of subsequent NIH or other major funding for any potential follow-on work (if appropriate)
- Notification of the BSF Board of Directors' decision regarding whether to award the grant will be sent by E-mail and by US mail by February 28, 2018. We request no telephone inquiries. All decisions are final.
- Please follow these instructions *precisely*.



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2017 BSF GRANT APPLICATION INFORMATION FORM

Principal Investigator:

Name (Last, First, Middle Initial) _____

Title _____

Department _____

Institution _____

Full Mailing Address _____

City _____ State _____ Postal Code _____ Country _____

Telephone _____ Fax _____

E-Mail _____

Institutional Information:

Division or Department Head:

Name _____

Title _____

Telephone _____ Fax _____

E-Mail _____

Authorized Institutional Officer Signing for Organization:

Name _____

Title _____

Mailing Address _____

City _____ State _____ Postal Code _____ Country _____

Telephone _____ Fax _____

E-Mail _____

Fiscal Official to Whom Funds Should be Sent:

Name _____

Title _____

Mailing Address _____

City _____ State _____ Postal Code _____ Country _____

Telephone _____ Fax _____

E-Mail _____

Exact Name of Institution or Account to Which Checks Should be Made Payable:

Project Title:

Application Type:

_____ **Idea** grant (\$50,000 maximum budget; 1-2 years)

_____ **Development** grant (\$100,000 maximum budget; 2-3 years)

Is this application responding to RFA? _____ Yes _____ No

If Yes, what RFA? _____

Letter of Intent Sent to BSF—REQUIRED FOR DEVELOPMENT GRANT

APPLICATIONS: _____ Yes _____ No

Proposed Starting Date for the Project: _____

Project Funding Request:

TOTAL Funds Requested \$ _____ (U.S.) – NOT TO EXCEED US \$50,000
FOR IDEA GRANT OR \$100,000 FOR DEVELOPMENT GRANT

To be Paid Over: _____ years

This Project Will Involve the Following:

Human Subjects: No _____ Yes _____ - *If yes, please plan to submit institutional approval if awarded—funds cannot be issued without institutional approval*

Vertebrate Animals: No ____ Yes ____ - *If yes, please plan to submit institutional approval if awarded—funds cannot be issued without institutional approval*

For budget purposes, PI is:

“Young Investigator” ____ (non-tenured position or temporary employee)

“Established Investigator” ____ (tenured position or permanent employee)

X
X
X

I confirm that all of the information contained in this grant application is accurate and not misleading. I agree to accept responsibility for the scientific direction and conduct of this project. I certify that I have read the attached research grant policies of the Barth Syndrome Foundation, Inc. and that I will abide by them, if this grant is awarded. I acknowledge that I am aware that all decisions about grant applications made by the Barth Syndrome Foundation, Inc. are final and are not subject to appeal.

Name of Applicant (*please print*) _____

Signature of Applicant _____

Date _____

I confirm that all of the information contained in this grant application is accurate and not misleading. I agree to accept oversight, legal and financial responsibility for this project. I certify that I have read the attached research grant policies of the Barth Syndrome Foundation, Inc. and that the institution I represent will abide by them, if this grant is awarded. I acknowledge that I am aware that all decisions about grant applications made by the Barth Syndrome Foundation, Inc. are final and are not subject to appeal.

Name of Institutional Officer (*please print*) _____

Signature of Institutional Officer _____

Date _____

BARTH RESEARCH GRANT APPLICATION BUDGET FORM/TABLE

Complete one Budget Form for the **TOTAL** project budget and a separate one for each year of the project (NOTE: the annual figures will serve as the basis for funds distribution -- one half of the annual amount will be disbursed semi-annually unless explicit six-month budgets are submitted in addition); **list all amounts in US \$.**

Budget Categories as listed:

Time Period: _____

Salaries and Benefits (listed by individual): [if PI is "Young Investigator," then <= 75% of Direct costs] [if PI is "Established Investigator," then <= 10% of Direct costs]	
SUBTOTAL (Subtotal not to exceed 75% of Direct costs)	
Equipment and Supplies (listed by category):	
SUBTOTAL	
Patient Care (itemized by type of expense):	
SUBTOTAL	
Other (itemized by type of expense):	
SUBTOTAL	
TOTAL DIRECT COSTS	
TOTAL INDIRECT COSTS <i>(not to exceed 10% of Direct costs)</i>	
TOTAL (Direct + Indirect) COSTS (NOT TO EXCEED US \$50,000 FOR IDEA GRANTS OR US \$100,000 FOR DEVELOPMENT GRANTS OR BUDGET MAXIMUM FOR RFA GRANT AS SPECIFIED IN RFA)	



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Barth Syndrome Foundation, Inc. Research Grant Policies

- Protecting the rights and welfare of human research participants is a top priority of the Barth Syndrome Foundation, Inc. (BSF). Federal regulation and BSF policy require that all institutions maintain appropriate policies and procedures for the protection of human subjects. In addition, BSF encourages a research environment in which ethical and productive investigation is valued. If applicable, a copy of the informed consent form for any human subjects for the project as well as proof of current project approval by an Institutional Review Board (IRB) (or a similar oversight group) must be provided to BSF before funding for a project will be released by BSF.
- Similarly, it is a BSF policy that all projects must conform to regulations for the safe and humane treatment of animals (see Addendum A). Specifically, BSF stipulates that animals can be used in BSF-funded research only when no other means of obtaining scientifically sound, valid and useful results are available. Furthermore, only the minimum number of appropriate animals required to obtain and validate results should be used; and in cases requiring the death of an animal, only the most appropriate and humane form of animal sacrifice consistent with the purpose of the research shall be employed. If applicable, a copy of proof of current project approval by the institution's Animal Use and Protection Committee (IACUC or a similar oversight group) must be provided to the BSF before any funding for the project will be released by BSF.
- It is BSF's expectation that any results and accomplishments from research it has funded will be made public (preferably through a high quality, peer-reviewed journal article) in order to maximize progress toward improved understanding, treatments and ultimately the discovery of a cure for Barth syndrome.
- BSF also expects that Materials resulting from research funded by the organization will be made available to other researchers on a reasonable and quick basis. "Materials" means reagents useful for scientific or preclinical investigation, animal and cellular models and any other physical inventions useful in further scientific or preclinical work other than intellectual property that are at least partially funded by this award and unencumbered by either i) existing University intellectual property or contractual obligations as of the Project Start

Date and/or ii) subsequent University intellectual property or contractual obligations related to work that was not funded by BSF. BSF considers an optional one-year period of exclusivity for the creator laboratory to be reasonable, and distribution should be able to commence with the first publication. Furthermore, it is expected that such Materials will be made available to non-commercial laboratories at cost and to commercial enterprises for a reasonable licensing fee.

- BSF recognizes that in the effort to develop therapies for Barth syndrome the grant recipient may need to maintain confidentiality so that intellectual property rights can be preserved and patent applications filed appropriately. The grant recipient may enter into a for-profit enterprise to pursue and/or commercialize discoveries made in whole or in part with funding from BSF. For those situations where a for-profit enterprise is anticipated or the grant recipient is part of a for-profit company, please see Addendum B.
- BSF will not challenge any decisions concerning patent applications that might be made by a grantee for inventions resulting from work performed under a BSF research grant. All inventions or discoveries made in whole or in part with funding from BSF must be reported to BSF at the earliest practical time. The grantee also must notify BSF immediately of any decision to apply for a patent or other legal protection. This information will be kept confidential.
- BSF will forward funds for the project to the institution every six months in accordance with the project's approved budget, following BSF's receipt of a signed statement from the principal investigator and an authorized institutional officer confirming adherence to BSF policies and stating that the project is proceeding in accordance with the original plan, timetable, and budget (or explaining variations from the original expectations). BSF will send the required statement to the principal investigator 30 days prior to the time when the next payment of funds is anticipated, and it must be signed and returned before funds can be released. (A sample of the standard BSF form for this is included at the end of this document, for your information—Please contact your institutional official to make sure the language of this “Statement” is acceptable.)
- In any published report of research funded in part or in whole by a BSF research grant, BSF must be cited as a source of funds, and BSF must be sent a copy of the published material or paper. If an affiliate of the BSF has provided funds for a BSF research grant, then the BSF affiliate must be cited by name in addition to BSF in any published report. The specifics of citation of acknowledgement will be noted in the initial award letter.
- Within 60 days of the end of the grant period, it is required that a report by the Principal Investigator on the research that resulted from the BSF grant be sent to BSF. In addition, a layman's summary (which can be published by BSF as is or in edited form) must be submitted. If the grant is for more than a one-year period,

then a type-written progress report must be issued by the Principal Investigator at the end of each year on the grant commencement date anniversary and sent to BSF.

- A final detailed accounting of the funds spent is required to be sent to BSF within 60 days of the end of the grant period. If the grant period is longer than one year, then annual financial reports must be filed with BSF on the grant commencement date anniversary. At the end of the grant period, any unused funds must be returned to BSF, unless a letter explaining the situation and requesting an extension of the grant period (without additional funds) is submitted at least 90 days ahead of the end of the grant period and approved by BSF.
- Any change in a budget category equal to or greater than 10% of the total research grant amount must be requested ahead of time by the Principal Investigator to BSF in writing and must be approved by BSF before being executed.
- BSF must be notified immediately if there is to be any change of either the Principal Investigator or the Institution with which he/she is associated. Furthermore, if the Principal Investigator is to be absent from the grantee Institution for a time of more than 30 days, then BSF must be notified. BSF reserves the right to request a written status report on the progress of the research project as well as an accounting of the funds spent as of the request date and to determine the final dispensation of any research grant funds remaining in any such situations.
- BSF reserves the right to suspend or cancel a grant at any time at its sole discretion for failure to abide by BSF policies governing research grants. Upon receipt of notice of project suspension or cancellation by the Institution, BSF's financial support of further work on the project will cease. At that point, the Principal Investigator must prepare and submit a project status report to BSF, while the grantee's institution must submit a complete accounting of funds expended to date. All unused funds must be returned to BSF immediately upon its request. A grantee may terminate a grant by sending notice in writing to BSF, providing BSF with both a written accounting of funds expended to date and a project status report sufficient in detail for a third party to replicate and continue the research project, and returning all unused funds to BSF.
- To properly evaluate the applications, each research grant application will be reviewed by members of the BSF Scientific and Medical Advisory Board (SMAB) who have not also submitted applications in that grant cycle. Outside reviewers may be utilized as well, if the subject matter of a particular application warrants enlisting outside expertise. The applicant should furnish BSF with a list of three individuals who would be appropriate outside reviewers. These individuals must be qualified to review your application, have not worked with you during the last three years, and have no conflict of interest. In addition, the applicant should

make known to BSF at the time of application submission if there are any BSF SMAB members or other outside experts whose review of the application would constitute a conflict of interest.

- Some information about research grants awarded by BSF will be made available to its constituents and to the general public. This information will include the title of the project, the Principal Investigator, the grantee Institution, the amount of the grant award, and the abstract provided as part of the grant application. No privileged or confidential information or trade secrets previously identified as such to BSF will be divulged.
- The nature of this arrangement is a funding agreement; no employment or agency relationship is hereby created.
- The Principal Investigator and the grantee Institution indemnify and hold harmless BSF, its Board, officers, agents, advisors and constituents from any claim, judgment, award, damage, settlement, liability, negligence or malpractice arising from research or investigation related to this BSF research grant.

GENERIC SAMPLE PROVIDED TO SHOW WHAT THE PI AND INSTITUTION WILL BE ASKED TO SIGN IF AWARDED FUNDS BY THE BSF



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STATEMENT REQUIRED TO BE SIGNED EVERY SIX MONTHS BEFORE DISBURSEMENT OF BARTH SYNDROME FOUNDATION, INC. RESEARCH GRANT FUNDS

I confirm that the research entitled "XXXXXX" that I am conducting with funds from the Barth Syndrome Foundation, Inc. and/or its affiliates is proceeding as was outlined in the research grant proposal in terms of the project design, methods and personnel and that it is on schedule and within budget. I certify that I have abided by the research grant policies of the Barth Syndrome Foundation, Inc. and shall continue to do so. I have described any deviations from expectations in any of these facets below:

Changes in research design, methods or personnel: _____

Changes in schedule: _____

Changes in budget or spending: _____

Name of Principal Investigator (*please print*) _____

Signature of Principal Investigator _____

Date _____

I confirm that the research entitled "XXXXXX" that is being conducted by "YYYYYYY" with funds from the Barth Syndrome Foundation, Inc. and/or its affiliates is proceeding as was outlined in the research grant proposal. I verify that the work is being executed according to the timetable described in the research grant proposal and that funds are being expended according to plan in terms of the use of funds, amounts and timing of expenditures. I certify that the institution I represent has abided by the research grant policies of the Barth Syndrome Foundation, Inc. with respect to this

(Continued)

research project and that it shall continue to do so. I have described any deviations from expectations in any of these facets below:

Changes in timetable: _____

Changes in spending: _____

Name of Institutional Officer (*please print*) _____

Signature of Institutional Officer _____

Date _____

Addendum A



**BARTH SYNDROME FOUNDATION
NATIONAL HEALTH COUNCIL**

USE OF ANIMALS IN BIOMEDICAL RESEARCH

Because the use of animals in biomedical research continues to be necessary to achieve scientifically sound and valid results, the National Health Council requires all its voluntary health agency members that conduct and/or fund biomedical research that involves the use of animals to have a Board-approved written policy that adheres to the following principles:

It is a BSF policy that all projects must conform to regulations for the safe and humane treatment of animals. Specifically, BSF stipulates that animals can be used in BSF-funded research only when no other means of obtaining scientifically sound, valid and useful results are available. Furthermore, only the minimum number of appropriate animals required to obtain and validate results should be used; and in cases requiring the death of an animal, only the most appropriate and humane form of animal sacrifice consistent with the purpose of the research shall be employed. If applicable, a copy of proof of current project approval by the institution's Animal Use and Protection Committee (or a similar oversight group) must be provided to BSF before any funding for the project will be released by BSF.

This policy was adopted by the Barth Syndrome Foundation Board of Directors on January 21, 2010, and will be distributed to all researchers working with animals on behalf of the Barth Syndrome Foundation.

Addendum B



BARTH SYNDROME FOUNDATION

BSF RESEARCH GRANT PROGRAM AND AWARDEES AT FOR-PROFIT INSTITUTIONS OR ANTICIPATING FOR-PROFIT INVOLVEMENT

Because the development of pharmaceuticals to treat human disease often requires the participation of for-profit biotechnology or pharmaceutical companies, BSF recognizes that special considerations may need to be implemented. For example, to maintain intellectual property rights that allow commercialization, the need to publish may be delayed for some period of time. Often the researcher will need to patent discoveries to preserve commercial viability. In consideration of these special circumstances with a for-profit enterprise, or with an awardee that licenses Intellectual Property or Materials, that receives funding from BSF the following is to be understood:

The mission of the Barth Syndrome Foundation (BSF) is to save lives through education, advances in treatment, and finding a cure for Barth syndrome (BTHS). As a 501(c)(3), charitable organization, BSF intends to do all it can to encourage the commercial development of safe and effective treatments for BTHS. BSF understands that the cost for developing commercial products for extremely rare diseases that also meet regulatory requirements may exceed the revenues.

The awardee of a BSF Research Grant that is part of a for-profit enterprise or a University that licenses Intellectual Property or Materials will:

- Adhere to the BSF Research Grant Policies with the following clarification:
 - o That publication of study results shall be sufficient to comply with the BSF policy that "Materials resulting from research funded by the organization will be made available to other researchers on a reasonable basis;"
 - o That it shall not be required, at the early stage of development, to make Materials resulting from research funded by the organization "available to non-commercial laboratories at cost and to commercial enterprises for a reasonable licensing fee." After publication of study results describing Materials, then the usual requirements for allowing others to repeat the experiments of the published author will take precedence.
- Be expected to diligently pursue opportunities to license Materials and "Intellectual Property" that is funded, at least in part, by BSF. If the awardee does

pursue licensing opportunities based on research funded, at least in part, by BSF, the awardee has an obligation to notify BSF as soon as practicable. If the awardee does not or chooses not to diligently pursue such licensing opportunities, the awardee will notify BSF such that BSF can pursue other options and will negotiate in good faith to permit use of the Intellectual Property by BSF or third parties identified by BSF.

- Agree that, if and when any Intellectual Property or Materials arise from work that was funded, at least in part by BSF, BSF shall:
 - o Share in any Net Revenue received from any Intellectual Property (the "IP Consideration"). "Net Revenue" means licensing fees, royalties, or any other income derived from Intellectual Property or Material ("Income") less unreimbursed out of pocket expenses that are directly related to licensing or the filing prosecution, maintenance or enforcement of a patent for Intellectual Property or Material. In addition, the awardee shall not enter into any agreement that will derogate BSF's right to the IP Consideration and shall notify BSF promptly and in writing of any license, lease, sale, or other agreement concerning an invention developed under the BSF Research Grant Program. BSF's participation shall be calculated on a *pro rata* basis, determined by multiplying any Net Revenue by a fraction, the numerator of which is BSF's grant amount, and the denominator of which is the total direct cost for the Intellectual Property, including all Intellectual Property patent and licensing costs. Notwithstanding the forgoing, the awardee's share pursuant to the forgoing calculation shall not be less than 50%.

 - o Share in any Net Revenue, less unreimbursed out of pocket expenses directly related to review of Material encumbrances ("Material Revenue") received from licensing of a Material (the "Material Consideration"). BSF's portion shall be calculated on a *pro rata* basis, determined by multiplying any Material Revenue by a fraction, the numerator of which is BSF's award amount, and the denominator of which is the total direct cost of the development of such Material, including patent and licensing costs. Notwithstanding the forgoing, the awardee's share pursuant to the forgoing calculation shall not be less than 50%.