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 potential therapeutic treatment of Barth syndrome. BSF is interested in providing “seed grant funding” to young investigators as well as attracting experienced investigators new to the field of Barth syndrome basic science or clinical research. We anticipate that these funds will be used for the testing of initial hypotheses and the collection of preliminary data leading to successful long-term funding by the National Institutes of Health (NIH) and other major granting institutions around the world.

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 excellent 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 (https://www.barthsyndrome.org/research/grantprogram/rfa.html). Applications responding to RFAs will be given preferential consideration in BSF Research Grant Program.

BSF Research Grant Program 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 direct costs amount as PI salary. Tenured or permanent employees of institutions are allowed only 10% of direct costs to be PI salary. In addition, for those clinically-oriented applications where volunteers must travel to a clinical research site, these travel expenses will be handled separately and will have a separate budget maximum. BSF encourages independent investigators at all professional levels to submit their best ideas. There are no geographical limitations to this funding.

Applications will follow the R21 format of the NIH as far as practicable:
Paper Size and Margins
- Use paper size no larger than standard letter paper size (8 ½” x 11”).
- Provide at least one-half inch margins (top, bottom, left, and right) for all pages.
  No applicant-supplied information can appear in the margins.

Updated 08/6/2019
Font (size, type density) and Line Spacing

- Font size: Must be 11 points or larger. Smaller text in figures, graphs, diagrams and charts is acceptable, as long as it is legible when the page is viewed at 100%. Suggested fonts: Arial, Georgia, Pacifica
- Type density: Must be no more than 15 characters per linear inch (including characters and spaces).
- Line spacing: Must be no more than six lines per vertical inch.

Page limitations by section

<table>
<thead>
<tr>
<th>Section</th>
<th>Page limit</th>
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</thead>
<tbody>
<tr>
<td>Information Form (use supplied form)</td>
<td>3 pages</td>
</tr>
<tr>
<td>Project Summary/Abstract</td>
<td>1 page</td>
</tr>
<tr>
<td>Specific Aims</td>
<td>1 page</td>
</tr>
<tr>
<td>Relevance to Barth syndrome</td>
<td>0.5 page</td>
</tr>
<tr>
<td>Research Strategy</td>
<td>6 pages</td>
</tr>
<tr>
<td>Plans for longer-term funding</td>
<td>0.5 page</td>
</tr>
<tr>
<td>References</td>
<td>No limit</td>
</tr>
<tr>
<td>Biographical Sketch</td>
<td>5 pages</td>
</tr>
<tr>
<td>Budget Form/Table (use supplied form with narrative) [For clinically-oriented applications include separate travel expenses]</td>
<td>4 pages</td>
</tr>
<tr>
<td>External Reviewers to include/exclude</td>
<td>0.5 page</td>
</tr>
<tr>
<td>Statement of human subject or vertebrate animal use</td>
<td>0.5 page</td>
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<tr>
<td>Resources and Environment</td>
<td>0.5 page</td>
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<tr>
<td>Project Personnel</td>
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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 mandatory for applications answering specific Request For Applications (RFAs) advertised by 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, 2019, and it must be sent to BSF Director of Research at the address provided. The purpose of the letter of intent is to allow for evaluation by a subset of BSF Scientific and Medical Advisory Board, which will provide feedback to the applicant about the suitability of their proposal for BSF Research Grant Program. BSF will endeavor to provide prompt feedback. 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 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, 2019. A “letter of intent” is optional for **Idea** grant applications and for applications not responding to a specific **RFA**.

2. **Summary or abstract.** The summary/abstract summarizes 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).

3. **Specific Aims.** State concisely the goals of the proposed research and summarize the expected outcome(s), including the impact that the results of the proposed research will have on Barth syndrome research. List succinctly the specific objectives of the research proposed (e.g., to test a stated hypothesis, create a novel design, solve a specific problem, challenge an existing paradigm or clinical practice, address a critical barrier to progress in the field, or develop new technology).

4. **Relevance to Barth syndrome.** Explain how this project will advance the natural history, biochemical basis, or a potential therapeutic treatment of Barth syndrome.

5. **Research Strategy.** 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. 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.

6. **Plans for longer-term funding.** Assuming a positive outcome for your project, discuss your plans for obtaining subsequent funding from the NIH or other major funding organizations.

7. **References.** Standard citations of publications referred to in the application.

8. **Biographical sketch.** (NIH format preferred)

9. **Budget Form/Table.** (please use the template provided) Fill in the budget table and provide a detailed budget narrative section for the proposed project which
fully describes and justifies the expenses listed. 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.) For clinically-oriented applications, attach a separate travel expense budget that allows for volunteers to travel and stay at your clinical research site. (see Disallowed Budget Items below)

10. External reviewers to include or exclude. Applicants should also provide a suggested list of 3 external reviewers of your application who are not part of BSF Scientific and Medical Advisory Board (https://www.barthsyndrome.org/aboutbsf/smab.html). Applicants should also note any potential reviewers (external or internal to BSF) whom you wish to be excluded from the evaluation process.

11. Statement of human subject or vertebrate animal use. 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.

12. Resources and Environment. 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.

13. Project personnel. 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.

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). 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. 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 as 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 of the budget cannot exceed 75% of the direct costs.

The categorization of “Young Investigator” or “Established Investigator” is only for budgetary purposes—all applications within each BSF Research Grant cycle are competitively evaluated with all other applications in that cycle. Applications are evaluated based on individual scientific merit (i.e. Importance and Impact, Feasibility, Prospect of Long-term Funding, see below).

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 salary and non-PI salary) 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 BSF Director of Research 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 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 Director of Research for more information. Capital expenditures for laboratory equipment are not allowed.
In addition to the application described above, several specific documents may be required, if applicable. These documents are not counted as part of the 6-page Research Strategy page limitation:

- 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.)

- 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)

Furthermore

- 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 BSF Director of Research.

- Applications are considered confidential information by BSF and by any external reviewers.

- An electronic version of the full application (in .doc format or in .pdf format, please) and any attachments must be sent to BSF Director of Research, at Erik.Lontok[at]barthsyndrome.org by Wednesday, October 31, 2019 (absolutely NO extensions).

Erik Lontok, PhD  
Director of Research  
Barth Syndrome Foundation, Inc.  
Erik.Lontok[at]barthsyndrome.org
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 BSF Scientific and Medical Advisory Board (and selected outside reviewers), and recommendations will be forwarded to BSF Board of Directors for their decision to fund or not to fund.

**The applications will be scored and evaluated 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

- Notification of BSF Board of Directors' decision regarding whether to award the grant application will be sent by e-mail and by regular mail before February 28, 2020. We request no inquiries until then. All decisions are made by the BSF Board of Directors and are final.

- Please follow these instructions **precisely**.
2019 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 Funds 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

Updated 08/6/2019
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, version 2 updated 2/20/2019, of 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 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, version 2 updated 2/20/2019, of 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 Barth Syndrome Foundation, Inc. are final and are not subject to appeal.

Name of Institutional Officer *(please print)* __________________________

Signature of Institutional Officer __________________________

Date _______________________________________

*Updated 08/6/2019*
BSF 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: 

<table>
<thead>
<tr>
<th>Time Period: _________________</th>
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<tbody>
<tr>
<td>Salaries and Benefits (listed by individual):</td>
</tr>
<tr>
<td>[if PI is “Young Investigator,” then &lt;= 75% of Direct costs]</td>
</tr>
<tr>
<td>[if PI is “Established Investigator,” then &lt;= 10% of Direct costs]</td>
</tr>
<tr>
<td>SUBTOTAL</td>
</tr>
<tr>
<td>(Subtotal not to exceed 75% of Direct costs)</td>
</tr>
<tr>
<td>Non-capital Equipment and Supplies (listed by category):</td>
</tr>
<tr>
<td>SUBTOTAL</td>
</tr>
<tr>
<td>Patient Care (itemized by type of expense):</td>
</tr>
<tr>
<td>SUBTOTAL</td>
</tr>
<tr>
<td>Other (itemized by type of expense):</td>
</tr>
<tr>
<td>SUBTOTAL</td>
</tr>
<tr>
<td><strong>TOTAL DIRECT COSTS</strong></td>
</tr>
<tr>
<td><strong>TOTAL INDIRECT COSTS</strong></td>
</tr>
<tr>
<td>(not to exceed 10% of Direct costs)</td>
</tr>
<tr>
<td><strong>TOTAL (Direct + Indirect) COSTS</strong></td>
</tr>
<tr>
<td>(NOT TO EXCEED US $50,000 FOR IDEA GRANTS OR US $100,000 FOR DEVELOPMENT GRANTS)</td>
</tr>
</tbody>
</table>
I confirm that the research entitled “XXXXXX” that I will conduct or am conducting with funds from 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, version 2 updated 8/6/2019, of 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 will be conducted or is being conducted by “YYYYYY” with funds from 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, version 2 updated 8/06/2019, of Barth Syndrome Foundation, Inc. with respect to this 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) ______________________________
Title of Institutional Officer _____________________________________________
Contact information of Institutional Officer _________________________________
Signature of Institutional Officer __________________________________________
Date ___________________________________________________________________