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2021 BSF RESEARCH GRANT PROGRAM APPLICATION GUIDELINES AND REQUIREMENTS

Barth Syndrome Foundation, Inc. (BSF) and our <u>International Affiliates</u> welcome innovative applications that address the basic, translational, and clinical research challenges of Barth syndrome. Through this program, BSF seeks to provide seed grant funding to young and established investigators in order to generate the preliminary data required for successful follow-on funding available from major grant-making institutions such as the National Institutes of Health (NIH).

Key Dates:

- Letter of Interest (LOI) Due Wednesday, September 9, 2020. The LOI is a one (1) page document that describes the applicant's research idea. This optional step is an opportunity for researchers seeking input and guidance from the BSF SMAB on the scope and relevance of their proposed work for the funding program. Submitted LOI will be returned in two weeks (September 23, 2020) alongside SMAB comments. This engagement is highly encouraged for first-time applicants and is intended to provide guidance on the suitability of the proposed research, however, will not be binding on any final funding decisions.
- Full Applications Due Monday, November 2, 2020
- Grantees Announced and Earliest Award Start Date March 2021

Application Types:

- Idea, US \$50,000 maximum Total Cost, 1-2 years in duration. Idea applications are well-suited for basic research/discovery science applications and have a lower threshold for the amount of preliminary data required compared to Development applications.
- **Development**, US \$100,000 maximum Total Cost, 2-3 years in duration. Development applications have a higher threshold of preliminary data and are ideally suited for proposals with clear implications for therapeutic development.

Key Considerations:

- There are no geographical limitations to this funding program.
- Applicants must be independent investigators (e.g. faculty appointment), postdoctoral fellows cannot apply. BSF has launched a predoctoral and postdoctoral fellowship program with the American Heart Association, with the cycle opening in Fall 2020
- Young/non-tenured Investigators are allowed to budget up 75% of the total grant amount as PI salary.
- For clinically-oriented research applications that involve research participant travel, these budget outlays will be handled separately and is excluded from the aforementioned budget maximums, as they are an expense that it not directly pertinent towards evaluating the scientific merit of an application.
- Letters of Support from collaborators are encouraged components of Full Applications.
- Total Cost is composed of Indirect and Direct Cost, with Indirect Cost limited to 10% of Direct Cost

Evaluation Criteria:

- 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
- 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. <u>Prospect of Long-term Funding</u> 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 by March 2021. We request no inquiries until then. All decisions are made by the BSF Board of Directors and are final.

Full Application Page Limitations by Section.

Section	Page limit
Information Form (use supplied form)	3 pages
Project Summary/Abstract	1 page
Specific Aims	1 page
Relevance to Barth syndrome	0.5 page
Research Strategy	6 pages
Plans for longer-term funding	0.5 page
References	No limit
Biographical Sketch	5 pages
Budget Form/Table (use supplied form with narrative)	4 pages
[For clinically-oriented applications include separate travel expenses]	
External Reviewers to include/exclude	0.5 page
Statement of human subject or vertebrate animal use	0.5 page
Resources and Environment	0.5 page
Project Personnel	0.5 page

Full Application Format. Please follow the R21 format of the NIH as far as practicable:

- Use 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.
- Font size must be 11 points or larger. Smaller text in figures, graphs, diagrams and charts is acceptable, if legible when the page is viewed at 100%.
- 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.

Full Application Sections

Each full application *must* contain the following elements to be considered complete. Incomplete applications will not be considered for review.

<u>Information Form.</u> A completed Information Form, wherein the applicant must choose the **Idea** or **Development** application type, as well as provide institutional details. Please use the <u>downloadable Information Form and Budget Table</u> template.

<u>Summary</u> or <u>abstract</u>. 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 wil be used for BSF publicity and external reviewer engagement).

<u>Specific Aims</u>. 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 novel technology).

<u>Relevance to Barth syndrome</u>. Explain how this project will advance the natural history, expand the etiology, or a develop a potential therapeutic treatment for Barth syndrome.

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 intends 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.

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

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

Biographical sketch. (NIH format preferred)

Budget Form/Table. Please use the downloadable Information Form and Budget Table template. Fill in the budget table and provide a detailed budget narrative section for the proposed project which fully describes and justifies the expenses listed. Based on this budget, grant funds will be disbursed by BSF semi-annually. The start of the fiscal year for the grant will be the date the first check is issued. Unless specifically stated 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 research participants to travel and stay at your clinical research site. (see Clinically-oriented applications below)

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

<u>Statement of human subject or vertebrate animal use</u>. 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.

<u>Resources and Environment.</u> 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.

<u>Project Personnel</u>. 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.

Total Cost. Funding requests may be as much as **US \$50,000** for **Idea grants and US \$100,000** for **Development grants** <u>in total</u> (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. <u>Indirect costs</u> included in the project are limited to 10% of the direct costs.

<u>Budget Categories.</u> 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.

- "Young Investigator" category (e.g. when the applicant is non-tenured, assistant professor, adjunct employee, etc.) the Salary and Benefits of the PI can be budgeted to up to 75% of the direct costs.
- "Established Investigator" (i.e. associate professor and higher, permanent employee), 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 other project personnel such as 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.

<u>Clinically-oriented applications</u> that involve the testing or treatment of Barth syndrome individuals often have significant budget outlays for the travel of research participants 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 participants 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 research participants <u>will not</u> 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.

<u>No Cost Extensions.</u> Requests for no-cost extensions (NCE) of existing approved grants are requested via email and submitted to BSF Director of Research 30 days before the next scheduled payment of funds. Generally approved, the PI and Director of Research will correspond to determine the duration of the requested NCE.

<u>Disallowed budget items</u> in BSF Research Grant Program include: travel expenses of the PI or colleagues to conferences or meetings, publication fees, and personal expenses. Capital expenditures for laboratory equipment are not allowed.

Other Sections. 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 (<u>PDF preferred</u>) and any attachments must be sent to BSF Director of Research, <u>erik.lontok@barthsyndrome.org</u>, by <u>Monday</u>, <u>November 2</u>, <u>2020</u>
- 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 application.
- 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.