# GRANT GUIDELINES AND FORMAT

A watchful preparation of your application will avoid delays in the review process and will ensure that the application receives the attention and the careful examination that it deserves. An understanding of how your application will be reviewed can help you build a solid application. The review process is completed by a convening group of highly qualified, experienced experts in the field of GNE Myopathy, therapy development, basic research and Research and Development (R&D) fields.

Proposals are scored based on **significance**, **relevance to the NDF mission**, and **scientific merit**. An application does not need to be strong in all categories to be judged likely to have major impact. For example, a project that by its nature is not innovative, may be essential to advance the field.

## GRANT FORMAT AND LAYOUT INSTRUCTIONS

*Grants should not exceed 5 pages in length. Lay Summary and Bibliography are excluded from the page count. Biosketches, Budget and Budget Justification are not considered part of the grant application and are subject to the limitations (if any) indicated in the specific instructions provided below to complete those forms.*

Grants should include the following sections:

I. **Lay Summary** (Max 250 words)

A Lay Summary is a brief summary of a research project that is used to explain complex ideas and technical and scientific terms to people who do not have prior knowledge about the subject. It should be written in a language understandable to people with a basic education and should not contain jargon, acronyms or technical language. The summary is intended to explain how the research being proposed will address/fulfil specific lacunas in the field of GNE Myopathy and how the studies are critical to the successful development of a therapy for the disease. Please note that the summary will be distributed to the general public, GNE patients, their family as well as members of the Board of Directors and donors. This provides a mechanism for ensuring good communication to public, stakeholders and transparency of the peer review process. In addition, the Lay Summary helps NDF raise awareness of current studies funded by NDF, increases participation in research of people living with GNE,

demonstrates accountability to donors and funders for the use of funds and helps attract the support and confidence of the public.

## II. RESEARCH PLAN

The research plan should describe the proposed research, stating its significance and how it will be conducted. Remember, your application has two audiences: the majority of Reviewers who will probably not be familiar with your techniques or field and a smaller number of experts who will be familiar.

### a. Specific Aims

Provide a brief summary of the key aspects of the disease that will be addressed by the proposed studies and include all background information needed to understand its impact and relevance. Describe concisely and realistically the goals of the proposed research and summarize the expected outcome(s), including the impact the proposed research will have on the research fields involved.

Describe how the proposed work supports the NDF mission and its Scientific Priorities as outlined above. Emphasize how this work help advance NDF's therapy program. Does the work address basic biology, animal model development, biomarker development of preclinical testing? How does it improve or compare to prior research done?

### b. Preliminary Data

Hypothesis and proposed experimental plan should be supported by preliminary data and should provide evidence that the work and timelines are technically feasible. Demonstrate that you have experience with the experimental methods proposed. Demonstrate that you have the appropriate collaborations and/or consultants in place. If you are new to the disease field in which your grant is focused, consider adding a collaborator with experience.

Provide clear hypotheses and specific aims (not to exceed four aims). This is particularly important for grants directed at defining the role of a "new" gene/protein, where often proposals list a series of studies to define a role without stating a hypothesis or putative relevance. Consider alternative aims/experiments in case the aim is not successful.

If you have received prior NDF support, clearly outline the outcome of that work (i.e., results and publications). If the new proposal is related, describe how the new proposal builds on prior NDF funded research.

### c. Approach

This section is one of the most critical parts of the application and should describe, in detail, the plans and experiments proposed to achieve the proposed studies. Write and organize your application so that the Reviewer can grasp and understand the proposed studies.

The section should include the overview of the strategy, experimental design, a description of methods and analyses to be used to accomplish the specific aims of the project, a discussion of potential difficulties and limitations and how these will be overcome or mitigated and expected results, and alternative approaches that will be used if unexpected results are found.

For Experimental Design, include power analysis to justify the experimental design. We encourage applicants to review to the Experimental Design Assistance for guidance.

Include a detailed description of methodologies used, their reproducibility and whether they represent an improvement over the existing ones.

Provide a description of planned statistical analyses, and if necessary, consider adding a collaborator with the necessary skills/qualification to provide statistical analyses. Justify the number of repeats, animals and experimental outcomes necessary to demonstrate the hypothesis.

To aid in the preparation of a proposal, we suggest Investigators review the following, as applicable:

- For Preclinical Studies, we encourage applicants to review to the Experimental Design Assistance for guidance in designing experiments, and the FDA Guidance on Preclinical Assessment of Investigational Cellular and Gene Therapy Products.
- For studies that will focus on developing animal models, please refer to the FDA Animal Model Qualification Program for guidance.

For Biomarkers studies, please refer to FDA is currently updating their Biomarker Guidance.

d. Data Sharing

Specify sharing of original data, images and results, and the generation and sharing of protocols, Standard Operating Procedures (SOPs), and other necessary information needed to facilitate reproducibility of research.

e. Bibliography

Provide a bibliography of any references cited in the Research Plan. Each reference must include the names of all authors (in the same sequence in which they appear in the publication; you can use "et al." convention in place of listing all authors in a citation), the article and journal title, book title, volume number, page numbers, and year of publication. Make sure that only bibliographic citations are included. Be especially careful to follow scholarly practices in providing citations for source materials relied upon when preparing any section of the application.

## III. Investigators, Key Personnel and Collaborators

Please use the standard NIH format for your biosketch. Forms and Instructions on preparing a biosketch can be found here: https://grants.nih.gov/grants/forms/biosketch.htm

All senior/key personnel and other significant contributors (OSCs) must include biographical sketches (biosketches). Use the sample format on the Biographical Sketch Format Page to prepare this section for all grant applications. Figures, tables (other than those included in the provided format pages), or graphics are not allowed in the biosketch. Do not embed or attach files (e.g. video, graphics, sound, data).

The biosketch may not exceed five pages per person. This five-page limit includes the table at the top of the first page. Attach this information as a PDF file. See the Format Attachments page.

The biosketch should highlight whether the Principal Investigator (PI), key personnel or collaborators possess the qualifications, expertise, training to successfully complete the project. If the project is collaborative, include biosketches for all collaborators and Key Personnel involved in the study so as to clarify whether the Investigators have the complementary and integrated expertise necessary to successfully complete the project.

## IV. Budget and Budget Justification

Specify size and scope of the research project and specific resources needed.

An applicant's budget request is reviewed for compliance with the governing cost principles and other requirements and policies applicable to the type of recipient and the type of award. Any resulting award will include a budget that is consistent with these requirements. Make

sure that the personnel have appropriate scientific expertise and training. Make sure that the budget is reasonable and well-justified.

Budget items to consider:

Personnel: Indicate effort to the project of each individual involved in the project, including base salary and percentage of effort required to complete the proposed studies, even if they are not requesting salary support. Fringe benefits rates are based on your institution's policy.

Materials and Supplies: In the Budget Justification, indicate general categories such as glassware, chemicals, animal costs, and include a dollar amount for each category.

Animal Costs: Include the number of animals you expect to use, the purchase price for the animals (if you need to purchase any), and your animal facility's per diem care rate, if available. Details are especially helpful if your animal care costs are unusually large or small. For example, if you plan to follow your animals for an unusually long time period and do not include per diem rates, the Reviewers may think you have budgeted too much for animal costs and may recommend a budget cut.

Publication Costs: You may include the costs associated with helping you disseminate your research findings from the proposed research. If this is a new application, you may want to delay publication costs until the later budget periods, once you have actually obtained data to share.

Other Expenses: Overhead should not exceed 10% of the overall budget. Please limit expenses for office supplies to a maximum of $600, including software and computers. Everything in the budget must be justified by the proposed work. Budget for travel should be limited to attendance at meetings that will be used to promote the research sponsored by the NDF, to raise awareness of the disease and should not exceed $1,000.