

## 2024-2025 MSF RESEARCH GRANT PROGRAM APPLICATION GUIDELINES AND REQUIREMENTS

Myhre Syndrome Foundation (MSF) welcomes innovative applications that address Myhre syndrome's basic, translational, and clinical research challenges. Through this program, MSF seeks to provide grant funding to investigators to generate preliminary data required for successful follow-on funding available from major grant-making institutions such as the National Institutes of Health (NIH).

### Key Dates:

- Optional Letter of Intent- rolling acceptance
- Full Applications - **Due Thursday, February 29, 2024**
- Grantees notified by **April 5, 2024**

### Application Types:

- **Idea**, US \$50,000 maximum Total Cost, 1-2 years duration. Grant 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 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 and currently hold a full-time position at any level (post-doctoral, research scientist, assistant/associate/full professor, etc.) at an academic institution or in the pharmaceutical/biotechnology industry.
- Letters of Support from collaborators are encouraged components of Full Applications.

Grant recipients shall provide yearly and final Research Grant Progress Reports summarizing results obtained with funds provided by the MSF. The reports must indicate plans

for publication of research findings in scientific or medical journals. A template for the progress report is available here <https://www.myhresyndrome.org/grants>

#### Evaluation Criteria:

1. Importance and impact – the extent to which the research outlined could significantly contribute to the understanding of Myhre 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
4. Proposal Quality
5. Research Goals Addressed- based on the goals outlined in the MSF Research Roadmap focusing on one or more of the following:

**Basic Research to Determine Cellular Mechanisms:** We invite researchers to submit projects aimed at uncovering the cellular-level mechanisms responsible for the manifestations of Myhre syndrome, including biomarkers. These projects should have a strong foundation in cellular and molecular biology with the goal of contributing to the fundamental knowledge of this rare disorder.

**Establishing and Characterizing Human Cell Lines:** We are looking for researchers interested in creating and characterizing human cell lines specific to Myhre syndrome. The development of reliable cell lines is critical for drug discovery and the development of therapeutics. Projects should outline strategies for cell line establishment and characterization.

**Identifying and Testing Potential Therapies, utilizing animal models:** These projects will focus on the pre-clinical phase, preparing for future clinical trials. Proposals should include methodologies for testing potential therapies and their relevance to Myhre syndrome.

The grant review form can be viewed at <https://www.myhresyndrome.org/grants>.

Notification of MSF Board of Directors' decision regarding whether to award the grant application will be sent by e-mail by April 5, 2023. We request no inquiries until then. All decisions are made by the MSF Board of Directors and are final.

Letter of Interest (LOI)

The LOI is a one (1) page document that describes the applicant's research idea. This **optional** step allows researchers to seek input and guidance from the MSF Professional Advisory Board (PAB) on the scope and relevance of their proposed work for the funding program. The submitted LOI will be returned in two weeks alongside PAB comments. This engagement is **highly encouraged for first-time applicants** and is intended to guide on the suitability of the proposed research; however, it will not be binding on any final funding decisions.

Section	Page Limit
Information Form (use online form)	3 pages
Project Summary/Abstract	1 page
Specific Aims	1 page
Relevance to Myhre 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 online form)	4 pages
External Reviewers to include/Reviewers to exclude	0.5 page
Statement if 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 it is 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.

Information Form. A completed Information Form. Please use the **Online Information Form and Budget Table** template.

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 Myhre syndrome or the treatment of individuals with the disorder, and a description of how the project will be accomplished.

(Note: this abstract will be used for MSF publicity).

Specific Aims. List succinctly the specific objectives of the research proposal and how your proposed research will meet the needs of the MSF research goals.

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Include a summary of the expected outcome(s), including the impact the proposed research results will have on the MSF's goal of finding treatments and, one day, a cure for Myhre syndrome.

Relevance to Myhre syndrome. List which MSF research goals you are addressing and how your project is related to these priorities.

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.

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.

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

Biographical sketch. (NIH format preferred)

Budget Form/Table. Please use the downloadable 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. The start of the fiscal year for the grant will be the date the first check is issued.

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. Indirect costs included in the project are limited to 10% of the direct costs.

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

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 MSF, as stated below.

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.

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.

No Cost Extensions. Requests for no-cost extensions (NCE) of existing approved grants are

requested via email and submitted to the MSF Director of Research 30 days before the next scheduled payment of funds. Generally approved, the Principal Investigator (PI) and Director of Research will correspond to determine the duration of the requested NCE.

Disallowed budget items in the MSF Research Grant Program include travel expenses of the PI or colleagues to conferences or meetings except for MSF conferences or meetings to present research findings. Publication fees, personal expenses, and 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 MSF until the final IRB approval has been secured, and a copy, including the approved informed consent form, has been received by MSF.)
- 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 MSF until the final Animal Use and Protection Committee approval has been secured and a copy has been received by MSF.)

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 the MSF Scientific Committee.
- Applications are considered confidential information by MSF and by any external reviewers.
- Applications must be made by **Thursday, February 29, 2024.**
- Acknowledgment of MSF's grant application receipt will be emailed to the principal investigator within seven days of receipt.
- Grant applications will be reviewed by selected MSF Professional Advisory Board members and/or selected outside reviewers, and recommendations will be forwarded to the MSF Board of Directors for their decision to fund or not to fund.