

MDF Grant Award Policy Guidelines

The Myotonic Dystrophy Foundation (MDF) supports scientific investigations to enhance the quality of life of people living with myotonic dystrophy and advance research focused on finding treatments and a cure for this disease. As a part of this effort, the MDF offers funding opportunities for DM researchers and supports research efforts. These guidelines govern MDF awards made as part of a Request for Applications (RFA).

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Purpose

The Myotonic Dystrophy Foundation (MDF) is a non-profit corporation formed under the laws of the State of California in the United States. MDF's mission is to support and connect the myotonic dystrophy community, provide resources and advocate for care, and accelerate research toward treatments and a cure. The Myotonic Dystrophy Foundation may issue Requests for Applications (RFAs) for basic, clinical, or applied research directly related to myotonic dystrophy in:

- Pathogenesis
- Molecular basis underlying phenotype differences (Type 1, 2, congenital)
- Development of diagnostics and biomarkers
- Progression/natural history
- Identification and validation of drug treatment endpoints
- Standards of care and care integration, including nursing, social work, and psychology
- Epidemiology, economics, and support services
- Therapeutic development, particularly, but not limited to, early-stage projects where success can leverage larger investments

Award Eligibility

MDF grants are only available to applicants from academic institutions or non-profit research institutes. For-profit organizations cannot apply. Non-U.S. academic institutions or non-profit organizations are eligible if they are accredited academic medical centers or research institutes. The submitting principal investigator of the application must be employed or enrolled at an appropriate educational, medical, or other non-profit research institution and be qualified to conduct the proposed research project.

To be eligible for a grant, applicants or teams of applicants must:

- Demonstrate proficiency in the knowledge, resources, and skills needed to carry out the proposed research.
- Assume both administrative and financial responsibility for the grant.
- Have access to institutional resources necessary to conduct the proposed research project.
- Meet all other eligibility requirements specified in the relevant RFA.

Failure to comply with any of these eligibility rules or non-performance may result in the Foundation canceling the award.

Eligibility Limitations

Applicants may apply for a grant once per calendar year. After receiving an award, grantees are ineligible to apply for three calendar years. Applicants may only apply for one type of MDF

grant at a time and can only receive one grant during their award period. The Small Grants Program is an exception to this rule (refer to the specific RFA for details).

Application Review Process

Applications are reviewed by MDF's Scientific Advisory Committee and other reviewers appointed by MDF depending on the RFA. Reviewers score applications and provide scores to the MDF Board of Directors and Chief Executive Officer. The MDF Board of Directors will consider the reviewer scores and comments and determine final grant awards. Awards are made at the sole discretion of the MDF Board of Directors and are contingent upon the availability of funds. Availability of funds does not signify a commitment to award any grants. If no application is deemed of sufficient scientific merit, expertise, and/or skill, MDF may choose not to award a grant during a funding cycle.

Application Review Criteria

All applications are reviewed for the following criteria:

- ✓ *Overall impact on myotonic dystrophy.* This criterion evaluates the potential impact of the proposed research on improving the quality of life for individuals affected by DM. Reviewers assess how the research outcomes could lead to advancements in understanding, diagnosing, treating, or managing DM, ultimately resulting in tangible improvements in the lives of patients and their families. Factors considered may include the potential for breakthrough discoveries, innovative approaches to addressing key challenges in DM, and the scalability or generalizability of the proposed interventions or findings.
- ✓ *Commitment to myotonic dystrophy research.* This criterion focuses on evaluating the applicant's dedication to DM research and their likelihood of continuing to contribute to this field in the future. Reviewers assess the strength of the applicant's commitment based on various factors, including their past research track record, demonstrated passion for DM research, ongoing involvement in relevant initiatives or collaborations, and plans for future contributions to advancing knowledge and improving outcomes for individuals with DM. Emphasis is placed on identifying applicants who are committed to making sustained and impactful contributions to the DM research community.
- ✓ *The feasibility and scientific quality of the proposed research.* This criterion assesses the practicality, viability, and scientific rigor of the proposed research plan. Reviewers evaluate whether the proposed project is feasible within the stated timeframe, budget, and resources available. They also scrutinize the scientific quality of the research design, methodology, and analytical approach, ensuring that they are sound, well-justified, and capable of generating reliable and interpretable results. Proposals deemed infeasible or lacking scientific quality, such as those with flawed experimental designs, insufficient sample sizes, or inadequate data analysis strategies, are

considered low priority for funding, irrespective of their scores on other evaluation dimensions.

Applications deemed to be infeasible or of poor scientific quality will be a low priority for funding regardless of the application's scores on the other dimensions.

Grant Payments and Budgets

Payment Process

Funds for research projects are sent directly to the Principal Investigator's institution. Depending on the terms of the Award Letter, payments can be issued annually, semi-annually, or upon completion of project milestones. The institution's financial officer should establish an account to manage these funds, ensuring they are used solely for approved research expenses. The institution may not grant to any third party any security interest in the pending funds and should deposit the funds in an interest-bearing account, using and applying any interest earned as additional proceeds of the grant. All payments are made in US dollars and must comply with MDF guidelines.

Authorized Expenses

The following expenses may be allowed under MDF grant awards unless stated otherwise in the RFA.

- *Salary and fringe benefits.* Payment for the work of qualified professionals involved in the project, as long as their level of pay matches industry standards and the percentage of effort they contribute to the research.
- *Equipment and supplies.* Purchases necessary to achieve the specific goals of the project.
- *Travel expenses.* Costs directly related to carrying out the MDF grant award, such as attending conferences or meetings, are allowed, up to a maximum of \$2,500 per year, unless stated differently in the RFA or award letter. Travel expenses need to be justified in reports for active MDF grants.
- *Payments to collaborators.* Money given to other organizations or individuals working on the project, if those payments were planned and included in the project budget. These payments should be recorded in expense reports.

Unauthorized Expenses

The following expenses are not allowed under MDF grant awards unless stated otherwise in the RFA.

- *Institutional costs.* MDF funds cannot be used to cover general costs, including building maintenance, utilities, institutional capital cost recovery, overhead, or other indirect costs.
- *Indirect expenses.* Money typically covered by the institution or business of the Principal Investigator, including administrative fees or office supplies, is not allowed.
- *Educational fees.* Payments for tuition fees, registration, or other fees relating to academic studies, membership dues, subscriptions, books or journals, and/or for or related to moving from one institution to another are not allowed.
- *Payments to governing body members.* No payments can be made to the trustees or members of the governing body or persons (or business entities) connected to the trustees or members of the governing body of the organization receiving the grant without the prior written agreement of the MDF.
- *Illegal or inappropriate payments.* Any payments that go against the organization's rules or U.S. laws are not allowed.
- *Non-research purposes.* MDF funds should only be used for direct research costs as outlined in the project budget.

Changes to the Budget

The grant recipient may reallocate up to 10% of the total grant award budget between line items without prior approval from MDF. Any additional changes to the grant budget must be approved by MDF at least two weeks prior to reporting deadlines. Requests for budget reallocations should come on official institution letterhead, be signed by the person in charge of the project (the Principal Investigator or PI), and be sent to grants@myotonic.org.

For multi-year grants, funds may be carried over from one annual budget to the next with prior approval from the MDF at least two weeks prior to reporting deadlines. Requests for carrying over funds from one year to the next should come on official institution letterhead, signed by the person in charge of the project (the Principal Investigator or PI), and sent to grants@myotonic.org.

The Principal Investigator may request a no-cost extension of up to six months. A no-cost extension must be approved by the MDF at least two weeks prior to the termination of the grant. Requests for no-cost extensions should come on official institution letterhead, signed by the person in charge of the project (the Principal Investigator or PI), and sent to grants@myotonic.org.

Award Management

Duration of the Grant

Grants provided by the MDF are awarded for specific periods outlined in the relevant RFA. Second-year funding in multi-year grants hinges upon the successful achievement of objectives

set for the first year. Funding for grant awards with funding based upon completion of project milestones hinges on the successful achievement of specified milestones. While most RFAs do not allow for renewals, the specific terms of the RFA supersede this guideline.

Changes to Status

MDF reserves the right to cancel a grant under certain circumstances, which include:

- Failure by the grant recipient to provide required documentation within six months from the funding start date or established deadline, such as Institutional Review Board (IRB) or Institutional Animal Care and Use Committee (IACUC) approval letters, progress reports, or other specified documents.
- Limitations in the MDF's resources that necessitate a temporary or indefinite halt to funding research grants.
- Violation of any of the guidelines outlined in the RFA, grant contract agreements, or this document by the grant recipient.
- If the grant recipient receives simultaneous funding from the National Institutes of Health (NIH) or any other major government funder for the same project during the MDF grant period which would cover the total research expenses of the project.
- In the event of the principal investigator's death or termination of employment or enrollment at the institution or organization before the end of the grant period, and if MDF opts not to allow the appointment of a new principal investigator, unexpended funds (after accounting for any outstanding obligations) may need to be repaid within four weeks, terminating the grant.

Award Requirements

Grant recipients are required to adhere to the following:

- Submit progress reports at specified intervals.
- Maintain records of appropriate institutional and federal regulatory requirements.
- Publish results in a timely manner.

Reporting and Publications

Reporting

MDF requires regular reports on expenditures, progress, and regulatory compliance so that we may meet our donors' expectations for fiduciary diligence. Failure to comply with any MDF grant reporting requirement, as described below, may result in pending payments being "held" until the project is in compliance, and any grant "on hold" for more than six months may be terminated. Failure to receive final reports after completion of a project will be noted in subsequent applications for funding by the investigator.

Reporting schedules are determined by the specific RFA but may include narrative progress reports during the grant period and a final report (including an abstract in lay language) submitted to the MDF no later than one month after the completion of research.

Grant recipients are also required to submit expense reports in accordance with the RFA schedule. This will generally include progress reports and a final expense report (including the original proposed budget and final expenses on the grant) submitted to the MDF no later than one month after the completion of the research. Final expense reports should be submitted along with a check for any unexpended funds on the grant.

Publications

Award recipients are encouraged to submit at least one scientific paper for publication, within six months of the conclusion of the research, reporting the research findings. All papers, exhibits, and press releases directly resulting from MDF funding shall carry a credit line to the MDF.

If the grant recipient is aware that a press release is being prepared about the work or the grant recipient has been contacted by a journalist, please let the MDF know this is taking place. Grant recipients should encourage their university press offices or outside journalists to contact the MDF so that publicity can be coordinated. Press releases regarding the study funded by the MDF shall be emailed to grants@myotonic.org.

MDF encourages an open-access policy that enables the unrestricted access and reuse of all peer-reviewed published research funded, in whole or in part, by the MDF. MDF shall pay reasonable fees required by a publisher or repository to effect immediate, open access to the accepted article. This includes article processing charges and other publisher fees. While not needed to fulfill the open-access policy requirements, grantees are encouraged to deposit funded research consisting of their submitted manuscript, and its subsequent versions, on a preprint server.

The title of each study funded by MDF, together with the lay language abstract of the research, the names of the grant recipient, and the institution, will be published on the MDF website, in MDF newsletters, in annual reports, and wherever else MDF deems appropriate. The Grant recipient will always be clearly acknowledged. The lay summary description should not contain information the grant recipient does not wish to divulge to the general public.

Grant recipients are required to provide one (1) 500-750 word research article to be potentially included in the MDF Dispatch Newsletter for the greater DM community each grant year.

Intellectual Property Policy

The Myotonic Dystrophy Foundation recognizes that inventions having public health, scientific, business, or commercial application or value may be made in the course of research supported

by MDF. MDF desires that such inventions be administered in such a manner that they are brought into public use at the earliest possible time. MDF recognizes that this may be best accomplished through patenting, copyrighting, and/or licensing of such inventions.

"Invention" is defined here as any discovery, material, method, process, product, program, software, or use, whether or not patented or patentable or copyrighted or copyrightable, that has an application of value such that its use, licensing, lease, or sale can generate revenue.

All inventions discovered or first reduced to practice in the performance of research supported in whole or in part by MDF shall be promptly reported in writing to MDF when the invention is disclosed to the institution where the work was done, and prior to any public disclosure.

If the institution receiving or disbursing MDF funds that supported the invention has an established and applicable patent, intellectual property, or technology transfer policy and procedure for administering inventions, MDF will defer to that policy with the following restrictions:

- 1) No patent or patent application, copyright, or other intellectual property protection shall be abandoned without prior notification by the institution or inventor(s) to MDF and giving MDF the opportunity to take title to the invention to the extent permitted by law and after having deferred to other pre-existing claimants' rights to do the same.
- 2) Notwithstanding any other provision of this policy, MDF shall participate in the income derived from the invention. MDF's participation shall be determined within one year, or a reasonably prompt time, after reporting of the invention to MDF by mutual agreement between the institution or other titleholder, and MDF, with MDF's rights hereunder not being affected if such determination is not made within said time period. The amount of MDF's participation shall be guided by the principle that MDF's sharing of income shall be in proportion to MDF's portion of support for the work or research-giving rise to the invention. MDF waives receipt of income until the royalty income (net of any direct out-of-pocket patenting costs and of the amount of standard overhead that was not paid by MDF as part of the original award) from the invention exceeds \$500,000.
- 3) MDF expects that the Institution or other titleholder will make a good faith effort to license any IP arising from the project to appropriate third party(s) and to include provisions in the license obligating the licensee to commercialize the invention in a diligent manner and include appropriate diligence requirements and milestones, and that the institution shall monitor performance of the licensee.
- 4) MDF is willing to assist, if required, in identifying an appropriate licensee.

If the institution has no established and applicable patent, intellectual property, or technology transfer policy and procedure for administering inventions, MDF shall have the right to determine the disposition of the invention rights if no other parties have superior rights. In such cases, MDF may:

- 1) Decide whether a patent application should be or not be filed, or other appropriate measures be taken to protect intellectual property rights in the invention.
- 2) Release the invention to the institution, inventor(s), or their respective designee.
- 3) Submit the invention to a qualified organization for administration and licensing.
- 4) Determine by negotiation the fair share of the royalty income to be paid to the institution, inventor(s), or other parties having a right in the invention.
- 5) License or make other arrangements for the application and use of the invention on an exclusive or non-exclusive, royalty or royalty-free basis as seems reasonable in the circumstances.
- 6) If the invention is made with the joint support of MDF and an agency or department of the United States Government, to the extent permitted by law or otherwise and without waiving any rights of appeal or contest, MDF may defer to the patent, intellectual property or technology transfer policy of that agency or department upon receipt of a written statement by the appropriate agency or department notifying MDF of its policy and procedure and identifying the rights and interests of MDF in the invention in question.
- 7) If any invention is made with the joint support of MDF and some other health agency or funding organization, not an agency or department of the United States Government, the institution shall agree to negotiate with MDF, the inventor(s) and that other organization for a mutually satisfactory disposition of the invention rights.

The right of MDF to participate in revenue derived from an invention is not waived under this paragraph.

Ethics, Regulatory Compliance, and Conflicts of Interest

Award recipients must adhere to all relevant legislation, codes of practice, and recommendations. Any significant deviation from these regulations must be promptly reported to the Myotonic Dystrophy Foundation.

Award recipients are responsible for obtaining any necessary regulatory approvals for research involving human and/or animal testing. They must also maintain records of regulatory compliance and other related documents, including:

- Compliance with clinical protocols for human subjects, tissues, and materials used in research projects including with all policies, rules, and regulations governing clinical trials including those of applicable federal or country regulatory agencies, the respective university and institution, and the MDF.
- Approval from the award recipient organization's Institutional Review Board/Human Subjects Review Board or equivalent, and a copy of that Board's current approval notice to be submitted to the MDF in advance of the grant award, or such other time as specified by the MDF.
- Patient informed consent forms.

- For experimental drugs and medical devices, documentation of approval from relevant authorities like the United States FDA, the European Medicines Agency, or such other relevant authority.
- Institutional Review Board (IRB) and/or Institutional Animal Care and Use Committee (IACUC) documentation must be provided before the first grant payment is issued.

Patient Charges

MDF requires that patients participating in research studies funded by MDF should not be directly charged for any research procedures outlined in the approved protocol. Patients must be fully informed about any ancillary costs associated with their participation, such as travel and lodging expenses.

Conflict of Interest

Principal Investigators and collaborators must disclose any potential conflicts of interest, including proprietary interests that could be influenced by the project's outcome. MDF expects grantees to maintain the highest ethical standards in their research conduct.

Reviewer Conflicts

Reviewers must disclose conflicts that disqualify them from assessing specific applications, such as collaboration with the applicant, institutional affiliation, financial gain, direct competition with the application, or personal relationships. Additionally, conflicts that do not disqualify but should be disclosed include providing tools or reagents, serving as an advisor, or recent collaboration or publication with the applicant. These disclosures ensure fairness and objectivity in the review process.