



MYOTONIC
DYSTROPHY
FOUNDATION

MDF Request for Applications (RFA) AWARD POLICY

The Myotonic Dystrophy Foundation (MDF) is the world's largest patient organization focused solely on myotonic dystrophy. Our mission, "Care and a Cure," is to enhance the quality of life of people living with myotonic dystrophy (DM) and advance research focused on treatments and a cure.

To this end, MDF may, from time to time, issue Requests for Applications (RFAs) on particular research topics. These guidelines govern MDF awards made as part of an RFA from the Foundation.

Updated October 22, 2015

Terms of this policy may be revised or altered from time to time. Please ensure that you have the most current copy of our policy on file by downloading it at http://www.parentprojectmd.org/site/PageServer?pagename=Advance_grantees

MDF RFA Award Elements Quick Glance

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|-------------------------------|--|
| Overhead Allowed | Not allowed, p.4 |
| Travel Expenses Allowed | \$2000/year, p.4 |
| Equipment Purchases Allowed | Allowed, p.4 |
| Progress Reports | Annually and a Final |
| Report of Expenditures | Within four weeks of the end of the year or twelve weeks after close of the grant, p.5 |
| Current IND, IRB Approvals | On file with MDF before funds will be released, if humans will be used in research, or as described in Award Letter for multi-year projects, p.6 |
| Current IACUC | On file with MDF before funds will be released if animals are used in research, p.7 |
| Grant Disbursements | Annual disbursements or as specified in Award Letter, p.4 |
| Request for no-cost extension | Received at least two weeks before the end of the grant term, p.5 |
| Publications | Should be submitted to MDF upon acceptance; embargos will be honored, p.5 |

This Quick Glance guide is provided as a convenience for PIs and University Financial Officers, but does not contain all of the requirements or information pertaining to this award. Please be familiar with the entire grant policy manual, below.

MDF Investigator Award

I. Purpose

The Myotonic Dystrophy Research Foundation, Inc., is a national organization formed as a non-profit corporation under the laws of the State of California (“**MDF**”). MDF’s mission, “Care and a Cure,” is to enhance the quality of life of people living with myotonic dystrophy (DM) and advance research focused on treatments and a cure.

From time to time MDF may issue Requests for Applications (RFAs) on various topics designed to further MDF’s strategic mission for research.

MDF awards made through this mechanism are typically one to two years and the payments are contingent on the availability of the research funds, submissions of respective progress reports and report of expenditures. Checks are made payable to Grantee’s Institution and are issued annually unless the award payments are designed to be subject to achievement of milestones, as noted in the RFA. The institution’s financial officer should establish an account from which the research expenses may be paid under the terms of the approved award.

II. Eligibility

Those eligible to apply for an MDF Investigator Award must:

1. Be a professional or faculty member at an appropriate educational, medical or research institution and be qualified to conduct and supervise a program of original research;
2. Have both administrative and financial responsibility for the grant;
3. Have access to institutional resources necessary to conduct the proposed research project; and
4. Hold a Doctor of Medicine, Doctor of Philosophy, Doctor of Science or equivalent degree.

III. Award Requirements

1. Progress reports at designated time periods
2. Record of appropriate institutional/federal regulatory requirements
3. Timely publication of results

IV. Duration of Grant

Grant period is for 1-2 years from the start date of the award. Renewals for most RFAs are not considered, although the individual terms of the RFA shall take precedence over this guideline.

V. Review Criteria

The applications in response to specific RFAs will be evaluated on

- Overall significance of proposal/impact towards achieving the goals stated in the RFA
- Ability to leverage other funding sources and resources
- Appropriateness of research plan to achieve project goals
- Investigator has access to the appropriate equipment, space and personnel
- Quality and experience of the applicant demonstrate an ability to carry out the project plan
- Budget is realistic and commiserate with the proposed aims
- Responsiveness to any other terms of the particular RFA

VI. GRANT PAYMENT

Checks are made payable to the Principal Investigator's institution. Non-milestone dependent award checks are issued annually or semi-annually, according to the Award Letter terms; milestone-dependent award checks are issued as milestones are completed, according to the terms of the Award Letter. For both types of award, the institution's financial officer should establish an account from which research expenses may be paid under the terms of the approved award. 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.

VII. Authorized Expenses

When MDF deems them justified by the research, the expenses identified below are permitted under the MDF RFA:

1. Salary and fringe benefits of any qualified professional at an appropriate level for the institution or industry as justified by the percent effort described in the application budget justification, except where already supported through other mechanisms.
2. Equipment and supply expenses necessary to fulfill the project's specific aims.
3. Travel expenses directly related to the implementation of the proposed MDF Award and capped at a maximum of \$2000 per year, unless otherwise specified in the award letter. Travel expenses must be justified in the reports of expenditures due each year for active MDF grants.
5. Disbursements to collaborators including institutions or contractors, *if approved* as line items in the project budget. Such disbursements should be documented in expense reports.

VIII. Unauthorized expenses

The following expenses are not permitted under the MDF RFA:

1. MDF awards are not to be used to fund institutional capital cost recovery, overhead or other indirect costs.
2. Expenses normally covered by the indirect cost of the Principal Investigator's institution or business;
3. Fees for tuition, 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
4. Any purpose other than to pay direct research costs as described in the project budget

IX. Change in Budget

Changes within budget categories of an awarded project are allowed as long as they conform to the authorized expenses described in Section VII.

X. Change in Status

MDF has the option of canceling an award at any time with notice for any of the following reasons:

1. If within six months from the scheduled funding start date or the established deadline date for receipt of required reports, MDF has not received the applicable institutional supporting documentation (i.e., copy of IRB, IACUC approval letters); MDF progress/milestone reports; or other documentation as defined by the MDF RFA Award Policy or noted in the Award Letter.
2. Availability of Organization resources are limited to the extent that continuation of funding of research grants must necessarily be placed on temporary or indefinite hold.

3. For any violation of the guidelines governing MDF's RFA Award as defined by the MDF RFA Award Policy.
4. If the grantee has made for the same project a simultaneous resubmission to NIH that is awarded during the duration of the MDF RFA Award.
5. Repayment of unexpended funds, less any uncancelable obligations, may be required by MDF if a researcher dies or terminates his or her employment with the Institution or company at any time prior to the end of the award period, and if MDF chooses not to allow the institution or company to name a new principal investigator. Under these circumstances, any remaining unexpended funds, less any uncancelable obligations, must be returned to MDF within four weeks and the grant will be considered terminated.

XI. PATENT AND LICENSING POLICY INFORMATION

Grants awarded through MDF's RFA Award are subject to the Foundation's Intellectual Property Policy (see page 7).

XII. Reporting Requirements

MDF requires regular reports on expenditures, progress and regulatory compliance in order that we may meet the expectations of our donors 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" 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.

1. Report of Expenditures

An annual Report of Expenditures (ROE) is required documenting the amount of funding received to date by the institution or company, the disbursement of these funds by the investigator in the various budget categories, and the amount of any pending funds remaining. The ROE must be completed and returned to MDF no later than four (4) weeks after the end of each year of the active grant. The final ROE is also due no later than twelve (12) weeks after the completion of the award and should be submitted along with a check for any unexpended funds. All ROEs must be certified by the appropriate institutional or corporate financial officer. A carry forward from one budget year to the next of no more than 20% must be requested and approved in writing at least two weeks before the end of the grant year. A request for a "no-cost extension" of the grant period, if required, must be submitted in writing at least two weeks before the end of the grant year for which the extension is requested and may be granted for no more than six months.

2. Report of Progress

Progress reports must be submitted at least four weeks before the end of each annual award period and no later than (12) weeks following the expiration dates the grant period. If an award is milestone-dependent, MDF may require additional progress reports as established by the time line for accomplishing project milestones and noted in the Award Letter. Grantees who do not submit final progress reports by the deadline stated here may be ineligible for future funding from the Foundation.

3. Publications, Publicity and Confidentiality

- a) The title of each study funded by MDF, together with the lay language abstract of the research, the names of the grantee and the institution, will be published on the MDF

website, in MDF newsletters, in annual reports and wherever else MDF deems appropriate. The grantee will always be clearly acknowledged.

- b) No other information about funded projects or grant applications will be made known to anyone outside of the organization or outside of the scientific advisory committee (SAC) or other such reviewers as identified by the organization. All MDF SAC members and reviewers sign confidentiality agreements and conflict of interest statements.
- c) RFA Award recipients are also encouraged to submit at least one scientific paper for publication within six months of the conclusion of the research reporting the research findings. MDF should be clearly credited as a funding source for the work. The recipient's work is further expected to result in other publications, conference presentations and other public contributions to the field.
- d) When a paper based on the work supported by an MDF grant is accepted for publication or presented before a scientific organization, an advance copy of the paper must be sent to the Myotonic Dystrophy Foundation electronically at info@myotonic.org. MDF will respect journal embargos —the grantee will not be violating the authorship terms by providing us with an advance copy. We need advance copies so that we can determine, what, if any, publicity we may wish to provide around the research. No public disclosures of the contents will be made until after the journal embargo date. Also, while MDF understands that publishers have varied requirements regarding republication of papers selected for publication, MDF requires that each award recipient submit a request to the publisher encouraging them to allow MDF to post the paper published on its website, www.myotonic.org. The publisher's decision on this matter shall be final and will not affect the research award or any future grants by MDF to the award recipient.
- e) If the grantee is aware that a press release is being prepared about the work or the grantee has been contacted by a journalist, please let MDF know this is taking place. Grantees should encourage their Company or Institution press offices or outside journalists to contact MDF so that publicity can be coordinated. Press releases regarding the study funded by MDF shall be emailed to info@myotonic.org.

XIII. Regulatory Compliance

MDF must have documentation on file indicating that federal and institutional regulatory standards for human research, if applicable, are being met by the investigator for a MDF-funded project. The requirements described below are the minimum requirements and individual Award Letters may request more detailed reporting to MDF.

1. Clinical Protocols: When human subjects, tissues and/or materials are to be used in a research project, it is the responsibility of the Principal Investigator and the institution to ensure that the institution has the following on file:
 - a. A complete copy of the research protocol approved by the Institution's Human Subjects Review Board and a copy of that Board's current approval notice.
 - b. A copy of the patient informed consent form(s) to be used.

A copy of the IRB's current approval notice *must be submitted to MDF* either at the start of the award or in time for a milestone that involves human subjects, as indicated on the applicant award letter; when the IRB approval expires, usually within one year, a new current copy must be sent to MDF if the project is still ongoing. Projects must be in compliance with all policies, rules and regulations governing clinical trials including those of the federal regulatory agencies, the respective university and institution and MDF. Failure to provide current documentation of IRB approval within four weeks of the start date of a project (unless otherwise noted in the award

letter) or within four weeks of the expiration of version on file may result in project payments being “held.” A project that is on hold for more than six months may be subject to cancellation.

2. Food and Drug Administration: When experimental drugs and/or experimental medical devices are to be administered to patients, it is the responsibility of the Principal Investigator and the institution to ensure that the institution has the following on file:
 - a. A complete copy of the Investigational New Drug (IND) and/or Investigational Device Exemption (IDE) application approved by the Federal Food and Drug Administration (FDA); and
 - b. Copies of all correspondence during the application and award periods between the FDA and the MDF Principal Investigator pertaining to the experimental drug(s) and/or device study.

XIV. Patient Charges

MDF requires that patients participating in experimental drug and/or device studies for which funds are requested by MDF **not be charged directly** for any research procedures included under the project's approved protocol. Patients must be fully advised about their responsibility for ancillary costs relating to participation in a research project -- travel, lodging, food, etc.

XV. ANIMAL RESEARCH

MDF investigators should use animals and animal tissues for research purposes only when reasonable and practical alternatives do not exist. When attainment of the specific aims of a project require the use of animals and/or animal tissues, a detailed justification must be included in the research grant application submitted to MDF. The justification shall include statements confirming that institutional guidelines:

1. Are at least as protective as those of the National Institutes of Health;
2. Conform to all applicable laws and regulations;
3. Meet prevailing community standards for responsible scientific research;
4. Apply throughout the project to ensure the humane treatment of any animals involved in the project.

If animals are used in a MDF-funded project, a copy of the current Institutional Animal Care and Use Committee (IACUC) approval must be on file with MDF before any disbursements of funds will occur. The IACUC should cover the specific project by name funded by MDF.

XVI. Conflict of Interest

Any potential conflict of interest the Principal Investigator(s) or collaborator(s) may have relating to the project must be revealed in the appropriate section of the application. Such conflict would include (but may not be limited to) having a proprietary interest that may be affected by the outcome of a research project. It is expected that MDF grantees will observe the highest ethical standards in the conduct of research.

Myotonic Dystrophy Foundation.
INTELLECTUAL PROPERTY POLICY

The Myotonic Dystrophy Foundation is a national organization formed as a non-profit corporation under the laws of the State of Ohio ("**MDF**"). MDF's primary purpose is to support finding viable treatments and a cure for Myotonic Dystrophy through a coordinated and collaborated international, multidisciplinary, multi-institutional, focused approach to research.

Although the primary purpose of MDF in funding scientifically meritorious research is to advance its mission to support finding viable treatments and a cure for Myotonic Dystrophy, MDF recognizes that inventions having public health, scientific, business or commercial application or value may be made in the course of research supported by MDF. It is the desire of MDF 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 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.

1. 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.
2. If the institution receiving or disbursing MDF funds which 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:
 - a. 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.
 - b. 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.
 - c. 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 partie(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.
 - d. MDF is willing to provide assistance, if required, in identifying an appropriate licensee.
3. 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:

- a. Decide that a patent application should be or not be filed, or other appropriate measures be taken to protect intellectual property rights in the invention.
- b. Release the invention to the institution, inventor(s) or their respective designee.
- c. Submit the invention to a qualified organization for administration and licensing.
- d. 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.
- e. 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.
- f. 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.
- g. 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 3.