

Request for Applications: 2025 Pilot Grant Program Spring Cycle

Myotonic Dystrophy Foundation
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Contracting Officer: Tanya Stevenson, Chief Executive Officer, MDF
Location: United States, Canada, and eligible international sites
Date Issued: November 4, 2024
Proposals Due: January 24, 2025
Selection Notification: by March 21, 2025
Period of Award: May 1, 2025 – April 30, 2026
Anticipated Award: \$50,000
Number of Awards: To be determined based on applicant mix and available funds

Synopsis

Through this Request for Applications (RFA), the Myotonic Dystrophy Foundation (MDF) acknowledges the scarcity of funding for researchers to conduct innovative investigations, generate preliminary data, and pave the way for future research endeavors. In 2024, MDF first launched a semiannual application process for a one-year Pilot Grant in myotonic dystrophy (DM) research.

Goal

The goal of the Pilot Grant program is to fund original projects covering basic, translational, and/or clinical research in myotonic dystrophy. Through this funding, the program aims to achieve three objectives: (1) supporting new and cutting-edge avenues of inquiry in myotonic dystrophy, (2) fostering collaborative and interdisciplinary approaches in DM research, and (3) attracting new researchers to the DM field. This funding provides an opportunity to have a broader impact beyond individual projects,

strengthening collective efforts to understand the complexities of myotonic dystrophy and develop effective interventions for those affected by the disease.

Background

Innovative ideas and pilot studies often struggle to secure significant funding due to intense competition and stringent requirements of traditional research grants. With small, one-year grants, researchers can embark on exploratory studies, proof-of-concept experiments, or novel research approaches without the burden of extensive preliminary data or long-term commitments. This flexibility fosters creativity and risk-taking, leading to groundbreaking discoveries and promising leads for future investigations. Through this funding, the program aims to achieve three objectives: (1) supporting new and cutting-edge avenues of inquiry in myotonic dystrophy, (2) fostering collaborative and interdisciplinary approaches in DM research, and (3) attracting new researchers to the DM field.

The grant program focuses on funding DM researchers to explore innovative ideas and conduct preliminary investigations. This funding enables DM researchers to gather essential data and generate preliminary findings that can lay the foundation for larger-scale research projects, empowering them to take risks and explore new avenues of inquiry.

Additionally, the Pilot Grant Program seeks to foster collaboration and interdisciplinary approaches by incentivizing researchers from different disciplines to collaborate and pool their expertise. This multidisciplinary collaboration accelerates research and enhances the likelihood of breakthrough discoveries and novel therapeutic strategies.

Furthermore, the Pilot Grant Program hopes to attract talented researchers to the field with a commitment to supporting research efforts in DM, making it an attractive area for scientists seeking impactful opportunities. This influx of talent brings fresh perspectives, novel methodologies, and new technologies to the study of myotonic dystrophy, while also providing early-career researchers with an opportunity to establish themselves in the field.

Grant Focus Areas

Myotonic dystrophy is a chronic disease with multiple dimensions that affects the lives of DM patients and their families every day. There are two major types of myotonic dystrophy: type 1 (DM1) and type 2 (DM2). Both types of myotonic dystrophy are inherited autosomal dominant disorders affecting all areas of the body. The primary

clinical physical manifestation is characterized by progressive muscle wasting and weakness affecting the lower legs, hips, hands, shoulders, neck, and face in DM1 and progressive muscle wasting and weakness affecting the proximal leg muscles, hips, shoulders, and neck in DM2. Research indicates that as many as 1 in 2,100 individuals in the United States are affected by myotonic dystrophy or at risk of passing the disease to the next generation.¹

People with this disorder may have prolonged muscle contractions (myotonia) and may not be able to relax certain muscles after use, affecting grip and speech, for example. They may also develop cataracts, cardiac conduction defects, and infertility. Many patients demonstrate CNS effects, including white matter abnormalities that are associated with central fatigue, excessive daytime sleepiness, and difficulties in executive function. A variation of DM1 called congenital myotonic dystrophy includes weak muscle tone (hypotonia), breathing and swallowing problems, delayed development, and CNS involvement that results in intellectual disability at birth.

Opportunity for DM Research

Recognizing that the symptoms and the severity of DM vary widely among affected people and often severely impact activities of daily living, mobility, and independence, **the MDF invites scientific proposals to explore innovative ideas and conduct preliminary investigations in DM research.**

Duration of the Award

Grants are awarded for one year, and applicants may apply once per calendar year. Once receiving an award, grantees are not eligible to apply for three calendar years. Applicants may only apply for one type of MDF grant at a time and may only receive one grant during the duration of their award. The Small Grants Program is an exception to this rule (please see the specific RFA for more information).

Payment

Awards are made to the applicant organization on behalf of the grantee. Awards are \$50,000 for salary, benefits, travel, and research support. The MDF awards may not be used to fund institutional capital cost recovery, overhead, or other indirect costs. A progress report satisfactory to the MDF is required four weeks after the end of the award year. The Foundation can cancel the award for non-compliance with any of the eligibility rules herein, or due to non-performance.

Applications

Eligibility Requirements

Applications are limited to those from academic institutions and/or non-profit research institutes. For-profit organizations are not eligible for this RFA. Applications from non-U.S. academic institutions or non-profit organizations are permitted, as long as they are accredited academic medical centers or research institutes.

1. *Principal Investigator requirements.* The submitting principal investigator must:
 - Be employed at an appropriate educational, medical, or other non-profit research institution and be qualified to conduct and supervise a program of original research.
 - Have both administrative and financial responsibility for the grant.
 - Have access to organizational resources necessary to conduct the proposed research project.
 - Hold a Doctor of Medicine, Doctor of Philosophy, Doctor of Science, or equivalent degree.
2. *Study Requirements.* Applicants or teams of applicants must have proficiency in the knowledge, resources, and skills necessary to carry out the proposed research. Proposals may be submitted 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

Submission Process and Requirements

Proposals cannot exceed 6 pages in length and must be submitted in 12-point font. Proposals must be submitted via the Proposal Central application system by January 24, 2025, at 5 PM Pacific Time. The proposal must include the following sections:

Applicant

- Professional Profile
- ORCID iD
- NIH-style applicant bio sketches (not to exceed 4 pages each)

Applicant Institution Information

- Applicant Institution Profile
- IRS EIN or TIN Number
- Signing Official Email
- Financial Official Email

Abstract

- **Abstract of Research Plan.** A complete, scientific description of the proposed work so that it may be separated from the application. This abstract will not be made public (one-half page).
- **Lay Summary.** A general, non-scientific description of the proposed work. If funded, the lay summary is to be used and published in appropriate places by the Myotonic Dystrophy Foundation (one-half page).

Budget

- Detailed budget (included costs are flexible)
- Budget description and justification, including explanations of how uncovered salary/benefits or research costs will be met (1 paragraph)
- Description of other sources of funding (1 paragraph)

Publications

- List of current and relevant publications by the PI

Attachments

- **Applicant Statement.** Including the applicant's name, contact information, a listing of current funding, a description of other pending applications for research funding during the funding period, and a description of how previous experience and

research interests will align with this project to address important questions related to myotonic dystrophy, and demonstrable, long-term commitment to research related to myotonic dystrophy (one-half page).

- **Description of environment.** Including facilities, equipment, and any leveraged funding (2 paragraphs).
- **Key Personnel Bio-Sketches.** NIH-style bio sketches of all participating team members (not to exceed 4 pages each).
- **Research Plan.** This must include the following information (total should not exceed 5 pages):
 1. Background and hypothesis (one-half page)
 2. Specific aims of the project (one page)
 3. Translational significance of the project (one-half page)
 4. Preliminary data, optional (one-half page)
 5. Methods, data analysis plan (including interpretation, expected results, and significance), and pitfalls/alternative strategies (2 pages)
 6. Anticipated collaborative agreements, if applicable (one-half page)
 7. Project timeline, with proposed milestones within the award period (one-half page)
- **References.**
- **Support Letters.** Letter of recommendation from an individual with knowledge of the applicant and preferably knowledge of the project.

Review and Selection

All applications must be received by 5:00 PM Pacific Time on Friday, January 24, 2025. The MDF Scientific Advisory Committee will score and prioritize candidates based on the following criteria:

- *The impact of the proposed research on the quality of life for people living with DM.* Reviewers will prioritize projects that address critical issues or barriers in DM research and are grounded in strong scientific principles. They will also evaluate the potential for the project to advance scientific knowledge, technical capabilities, or clinical practices, as well as its capacity to introduce transformative changes to methods, technologies, treatments, services, or preventative interventions in the DM field. Key considerations include the potential for breakthrough discoveries, innovative approaches to major challenges, and the scalability or generalizability of the findings. Proposals will be ranked based on the strength of the impact case made

in the "Lay Summary" and "Research Plan" sections (approximately 40% of the total score).

- The strength of the researcher's commitment to the research and the likelihood that they will pursue independent research that continues to advance knowledge relevant to improving the quality of life of people living with DM. This will be assessed based on the information provided by the applicant in the "Statement from the Applicant" component of the application and via the submitted letters of support (approximately 20% of the total score).
- Reviewers will assess the innovation, feasibility, and scientific quality of the proposed research. Reviewers will evaluate how the project challenges existing paradigms and introduces novel approaches or concepts in the DM field. They seek projects that demonstrate creativity and originality, aiming to push boundaries, refine current methods, or develop new interventions. The overall strategy, methodology, and analysis will be examined to ensure they are robust, well-justified, and appropriate for achieving the research aims. Reviewers will also assess the feasibility of completing the research within the proposed timeframe, budget, and resources, as well as plans to address biological variables. Applicants may suggest expert reviewers for consideration (approximately 40% of the total score).

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

Applicants may consult with the MDF Chief Scientific Officer, Dr. Andy Rohrwasser (Andy.Rohrwasser@myotonic.org) for refinement of their proposals before submission. Technical issues should be directed to the MDF Research Grants Manager, Dr. Nadine Ann Skinner at nadine.skinner@myotonic.org.

After initial screening by MDF staff members, the Scientific Advisory Committee and selected experts will review applications and recommend final candidates to the MDF Board of Directors. The MDF Board of Directors will consider the Scientific Advisory Committee recommendations 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 applicant is deemed of sufficient scientific merit, expertise, and/or skill, the MDF may choose not to award a grant during this funding cycle.

Reporting and Publications

Progress Reports

Each recipient must submit a **final report** (including an abstract in lay language) submitted to the MDF no later than one month after the completion of research at the end of the year.

Expense Reports

Each recipient must submit 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 completion of research at the end of the second year and should be submitted along with a check for any unexpended funds on the grant. The grantee may reallocate up to 10% of the total grant award budget between line items without prior approval.

A request for a “**no-cost extension**,” 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.

Publications and Conferences

- Pilot Grant 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.

Timeline

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ⁱ 5. Johnson NE, Butterfield RJ, Mayne K, et al. Population Based Prevalence of Myotonic Dystrophy Type 1 Using Genetic Analysis of State-wide Blood Screening Program. *Neurology*. Published online January 20, 2021:10.1212/WNL.0000000000011425. doi:10.1212/WNL.0000000000011425