



Request for Applications: 2023 Early Career Research Grant

Myotonic Dystrophy Foundation
663 Thirteenth Street
Oakland, CA, 94612, USA

E-mail: nadine.skinner@myotonic.org
Phone +1-415-800-7777

Contracting Officer: Tanya Stevenson, Chief Executive Officer, MDF
Location: United States, Canada, and international sites that meet eligibility criteria
Date Issued: May 9, 2022
Proposals Due: September 9, 2022
Selection Notification: by December 16, 2022
Period of Award: January 1, 2023 – December 31, 2024
Anticipated Award: \$95,000 per year for 2 years; \$190,000 total
Number of Awards: To be determined, based upon mix of applicant types and available funds

Synopsis

Through this Request for Applications (RFA), the Myotonic Dystrophy Foundation (MDF) recognizes a need to retain early career scholars in the field of myotonic dystrophy (DM). MDF intends to issue up to two two-year awards of up to \$380,000 at \$190,000 per award for projects that focus on basic, translational and/or clinical research or care projects in myotonic dystrophy.

Goal

The MDF seeks to retain early career scholars who are passionate about researching in the myotonic dystrophy field.

Background

Early career scholars pursuing a career in research often struggle to obtain research funding and face job insecurity as they are reliant on short-term funding.ⁱ This funding

insecurity can impact research, as projects are not pursued or completed. This can be especially difficult with translational research and can lead to a lack of retention in medical research fields. The number of clinician–investigators, in particular, is in decline due to the duration of clinical training, limited or interrupted opportunities for research, high levels of educational debt, and stiff competition for research funding.ⁱⁱ The global pandemic has exacerbated these challenges.^{iii, iv} The MDF recognizes the importance of recruiting and retaining basic science early career researchers, as well as the critical need to recruit and retain clinical early career researchers to focus on the field of DM.

DM Overview

Myotonic dystrophy (DM) 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.^v

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 is soliciting scientific proposals for Early Career Research awards, which include research focused on improving treatment, care, and support of DM patients and their families, as well as the molecular biology and basic science of DM.**

Applications

1. Eligibility for RFA

Applications are limited to those from academic institutions and/or non-profit research institutes. For-profit organizations are not eligible for this RFA.

- Applicants or teams of applicants must have proficiency in the knowledge, resources and skills necessary to carry out the proposed research;
- Submitting PIs 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;
 - For clinical researchers or physician scientists, have a letter of commitment from their chair or clinical chief that they will have protected time from clinical service for the study;
 - For teaching faculty, have a letter of commitment from their chair or clinical chief that they will have protected time from teaching for the study;
 - Hold a Doctor of Medicine, Doctor of Philosophy, Doctor of Science or equivalent degree. The terminal degree must have been received no more than ten years prior to the time of the award*. If you have completed both residency and a PhD, your eligibility is based on when you completed residency, and you must have completed your residency no more than ten years prior to the time of the award*. If you completed a fellowship of any kind after residency, your eligibility is still based on the date you finished residency. *Exceptions to the ten-year limit include any periods of family or medical leave.
- Applications from non-U.S. academic institutions or non-profit organizations are permitted, as long as they are an accredited academic medical center or research institute.

2. Study Requirements

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

3. Submission Process and Requirements

Proposals cannot exceed 8 pages in length and must be submitted in 12-point font.

Proposals must be submitted via email to nadine.skinner@myotonic.org by September 9, 2022, at 5 PM Pacific Time.

The proposal must include the following sections:

a. Research Project Summaries

- *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).
- *Applicant Statement.* Including applicant's name, contact information, a listing of current funding, a description of other pending applications for research funding during the funding period, 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 (1 page).

b. Research Plan

- *Research Plan.* This must include the following information (total should not exceed 6 pages):
 1. Background and Hypothesis (1 page)
 2. Specific Aims of the project (1 page);

3. Translational significance of the project (one-half page);
4. Preliminary Data (1 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);

In addition, (not included in the page limit) proposals must include:

1. References;
2. Detailed budget in spreadsheet or table format (included costs are flexible);
3. Budget description and justification, including how explanations of how uncovered salary/benefits or research costs will be met (1 paragraph);
4. Description of environment, including facilities, equipment, and any leveraged funding (2 paragraphs);
5. NIH-style bio sketches of all participating team members (not to exceed 4 pages each);
6. Letter of recommendation from an individual with knowledge of the applicant and preferably knowledge of the project;
7. RFA face page provided by the MDF (see attached).

Review and Selection

- All applications must be received by 5:00 PM Pacific Time on Friday, September 9, 2022.
- The MDF Scientific Advisory Committee will score and prioritize candidates based on the following criteria:
 - The impact the proposed research could have on the quality of life of people living with DM. Reviewers will rank proposals based on the case for impact made by the applicant in the “Lay Summary” component of the application (approximately 20% of total score).
 - The strength of the early career 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 40% of total score).
 - The feasibility and scientific quality of the proposed research. This will be assessed by a subject matter expert selected by the Foundation based on

the research description provided by the applicant. Applicants may suggest expert reviewers in their field for the Foundation to consider engaging for this evaluation (approximately 30% of total score).

- Recognizing the critical need to recruit and retain clinical early career researchers, MDF will prioritize funding clinical researchers/ physician scientists.
- 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 are encouraged to consult with the MDF Chief Executive Officer, Dr. Tanya Stevenson at tanya.stevenson@myotonic.org for refinement of their proposals before submission. Technical issues should be directed to the MDF Research Coordinator, Dr. Nadine Ann Skinner at nadine.skinner@myotonic.org.
- The Scientific Advisory Committee will recommend final candidates to the MDF Board of Directors.
- The MDF Board of Directors will consider the 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, MDF may choose not to award a grant during this funding cycle.

Reporting and Publications

1. Progress Reports

Each recipient must submit six-month progress reports to MDF during the course of the two-year grant:

- Six-month **progress reports** to be received by the MDF twice per year during the award years;
- A **final report** (including an abstract in lay language) submitted to the MDF no later than one month after completion of research at the end of the second year.

2. Expense Reports

Each recipient must submit expense reports to the MDF during the course of the two-year grant:

- An **interim 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 first year.
- 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. Grantee may reallocate up to 10% of the total grant award budget 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.

3. Publications and Conferences

- Early Career Research 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 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 MDF shall be emailed to info@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.
- Early Career Research Grant recipients must attend virtually or in-person two MDF Annual Conferences and biennial IDMC conference and present at the MDF Annual Conference (those that attend in person are eligible for additional travel expenses to be paid by the MDF).
- 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 News for the greater DM community each grant year.

Timeline

Date Issued:	May 9, 2022
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ⁱ 1. Janus Laust Thomsen, Dorthe Jarbøl, Jens Søndergaard. Excessive workload, uncertain career opportunities and lack of funding are important barriers to recruiting and retaining primary care medical researchers: a qualitative interview study. *Family Practice*. 2006;23(5):545-549. doi:10.1093/fampra/cml034

ⁱⁱ 2. Hall AK, Mills SL, Lund PK. Clinician–Investigator Training and the Need to Pilot New Approaches to Recruiting and Retaining This Workforce: *Academic Medicine*. 2017;92(10):1382-1389. doi:10.1097/ACM.0000000000001859

ⁱⁱⁱ 3. Kliment CR, Barbash IJ, Brenner JS, et al. COVID-19 and the Early-Career Physician–Scientist. Fostering Resilience beyond the Pandemic. *ATS Scholar*. 2021;2(1):19-28. doi:10.34197/ats-scholar.2020-0104PS

^{iv} 4. Levine RL, Rathmell WK. COVID-19 impact on early career investigators: a call for action. *Nat Rev Cancer*. 2020;20(7):357-358. doi:10.1038/s41568-020-0279-5

^v 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.00000000000011425. doi:10.1212/WNL.00000000000011425