

Request for Applications: 2023 Doctoral and Postdoctoral Research Fellowships in Myotonic Dystrophy

Myotonic Dystrophy Foundation
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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: Doctoral: \$55,000 (includes funding for required travel)
Postdoctoral: \$105,000 (includes funding for required travel)
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) seeks to engage new scholars in the field of myotonic dystrophy (DM) to advance disease understanding and therapy development for DM. Through this effort the MDF, intends to fund doctoral and postdoctoral research fellowships for projects that focus on basic, translational and/or clinical research or care projects in myotonic dystrophy.

Fellowships may be awarded for projects with qualified sponsors in appropriate academic, research institute, or pharmaceutical/biotechnology company environments. Projects may focus on basic, translational and/or clinical research or care projects in myotonic dystrophy.

Grant Focus Areas

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.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 is soliciting scientific proposals for Doctoral and Postdoctoral Fellowship 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.**

The MDF is actively soliciting Doctoral and Postdoctoral fellowship proposals 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

Doctoral applicants must be enrolled and in good standing in a Ph.D. program at an accredited academic medical center or research institute. By the time of award, the applicant must have completed two years of a doctoral program, including formal coursework and any qualifying examination requirements, and have selected a sponsor who focuses a major portion of his or her lab's activities on DM.

Postdoctoral applicants must have a Ph.D., M.D., D.O. or equivalent degree from an accredited institution. Degree must have been received within no more than three years of the time of the award. The sponsor of the applicant must focus a major portion of his or her lab's activities on DM.

Co-sponsors are encouraged when projects utilize a multidisciplinary approach (e.g., two co-sponsors, one in a biotechnology company and the other in academia, for an early stage drug discovery and development project).

Duration of Doctoral or Postdoctoral Fellowship

The fellowship is for a two-year period, with second year funding dependent on successful completion of the first year's stated objectives. Prior to the actual awarding of the grant, the applicant must have fully met all of the requirements of her/his institutional country for residency, where applicable.

Payment

- Doctoral and Postdoctoral Fellow awards are made to the applicant organization on behalf of the fellow.
- *Doctoral Award*. \$55,000 for salary, benefits, travel, and research support, paid over two years:
 - \$5,000 of the award is for required travel to the MDF Annual Conference, and to the biennial IDMC meetings (see below).
- *Postdoctoral Award*. \$105,000 for salary, benefits, travel, and research support, paid over two years:
 - \$5,000 of the award is for required travel to the MDF Annual Conference, and to the biennial IDMC meetings (see below).
- The MDF awards may not be used to fund institutional capital cost recovery, overhead or other indirect costs.
- Travel funds of \$5,000 are included to fund attendance by the MDF Doctoral and Postdoctoral Fellows at the biennial International Myotonic Dystrophy Consortium

- meeting (IDMC) and the MDF Annual Conferences. Travel funds not used for these purposes must be returned to the MDF at the end of each year of the funding period.
- Second year funding of a MDF Doctoral or Postdoctoral Fellowship award is contingent upon documentation of satisfactory progress. A progress report satisfactory to the MDF is required four weeks after the end of each award year.
 - The Foundation can cancel the award for non-compliance with any of the eligibility rules herein, or due to non-performance.

Applications

1. Eligibility for RFA

- *Degree requirements.* Doctoral applicants must be enrolled and in good standing in a Ph.D. program at an accredited academic medical center or research institute. The applicant must have already completed two years of a doctoral program, including formal coursework and qualifying examination requirements, and have selected a sponsor who focuses a major portion of their lab's activities on DM. Postdoctoral applicants must have a Ph.D., M.D., D.O. or other doctoral degree in his/her profession from an accredited domestic or foreign institution before a MDF Fellowship award can be activated. Degree must be received within no more than three years before the fellowship period. Documentation of successful completion of a doctoral degree must be submitted prior to the start of the award.
- *Commitment to myotonic dystrophy research.* The MDF supports Ph.D. and postdoctoral scholars with outstanding potential to make contributions to myotonic dystrophy research. Previous work in this area is strongly encouraged but not required. However, the applicant should document how previous experience and interests will enhance the proposed research to address important questions related to myotonic dystrophy. The applicant and sponsor must demonstrate a strong and enduring commitment to current and future research in this area.
- *Sponsor.* The applicant must identify a qualified sponsor to directly oversee and supervise the research activities. That sponsor must have background experience in myotonic dystrophy or a field of study relevant to the candidate's application and be willing to actively support the applicant. The sponsor must document the availability of space, facilities, and research support to provide an outstanding training experience. For applicants who propose continued work at an institution where they have been engaged for more than one year, it is important that the sponsor document how the MDF

award will enhance the opportunity for new research experiences and broaden the scientific and/or clinical background of the applicant. It is expected that the sponsor will encourage and assist the prospective Doctoral or Postdoctoral Fellow with the application.

2. 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. Name, degrees, training/qualifications, experience, and percent effort for sponsor(s) (1 page for each sponsor);
6. NIH-style bio sketches of all participating team members (not to exceed 4 pages each);
7. Letter of support from the Sponsor and another individual with knowledge of the applicant;
8. 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).
- 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 end-of-year 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

- Doctoral and Postdoctoral Fellowship 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.
- Doctoral and Postdoctoral Fellowship recipients must attend virtually or in-person two MDF Annual Conferences and biennial IDMC conference and present at the MDF Annual Conference (travel funds are included).
- 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
Proposals Due:	September 9, 2022
Selection Notification:	by December 16, 2022
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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