



**MYOTONIC
DYSTROPHY**
FOUNDATION

Care and a Cure

PhD. Trainee and Postdoctoral Research Fellowships in Myotonic Dystrophy

Solicitation Name: 2020-Fellowship-MDF

Contracting Office Address:

Myotonic Dystrophy Foundation
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San Francisco, CA 94129, USA
E-mail: elizabeth.habeeb-louks@myotonic.org
Phone (Toll-Free): 86-MYOTONIC or 866-968-6642 (U.S. only)
Direct: 415-800-7777

Contracting Officer: Molly White, Chief Executive Officer, Myotonic Dystrophy Foundation
Place of Performance: United States, Canada or International sites that meet eligibility criteria
Date Issued: April 19, 2019
Proposals Due: September 6, 2018, 5:00 PM PDT
Notification of Selection: by December 20, 2019
Period of Award: January 1, 2020 – December 31, 2021
Anticipated Overall Award:
 Ph.D. Trainee: \$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:

The Myotonic Dystrophy Foundation (MDF) solicits applications, conducts peer review and makes fellowship and research grant awards to advance disease understanding and therapy development for myotonic dystrophy.

As a part of this effort, the Foundation annually funds Ph.D. trainee and postdoctoral research fellowships (similar to a U.S. National Institutes of Health F31 or F32 award). Traineeships and 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. Population-based prevalence in DM1 is 1:2300 (Johnson, 2018).

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 also experience CNS effects that sometimes manifest as white matter lesions and patient-reported symptoms including central fatigue, excessive daytime sleepiness and difficulty in executive function. A variation of DM1 called congenital myotonic dystrophy includes weak muscle tone (hypotonia), breathing problems, CNS involvement, delayed development, and intellectual disability.

Recognizing that the symptoms and the severity of the condition vary widely among affected people and often severely impact activities of daily living and functioning, **MDF is expanding the scientific research endeavors eligible for Ph.D. Trainee and Postdoctoral Fellowship awards to include research focused on improving treatment, care and support of the DM patient and his/her family, as well as molecular biology and basic science.**

MDF is actively soliciting Ph.D. trainee and 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

Ph.D. Trainee 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 be received within no more than three years completed by the time of the award. The sponsor 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 Ph.D. Traineeship or Postdoctoral Fellowship

The traineeship or 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:

- Ph.D. Trainee and Postdoctoral Fellow awards are made to the applicant organization on behalf of the trainee or fellow
- Predoctoral Award: \$55,000 for salary, benefits, travel and research support, paid over two years:
 - \$30,000 will be paid promptly prior to activation date of the first year
 - Assuming the first year report is satisfactory, \$25,000 will be paid at the beginning of the second program year. \$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:
 - \$55,000 will be paid promptly prior to activation date of the first year
 - Assuming the first year report is satisfactory, \$50,000 will be paid at the beginning of the second program year. \$5,000 of the award is for required travel to the MDF Annual Conference, and to the biennial IDMC meetings (see below).
- 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 MDF Ph.D. Trainee and Postdoctoral Fellows at the biennial 2021 International Myotonic Dystrophy Consortium meeting (IDMC) and the 2020 and 2021 MDF Annual Conferences. Travel funds not used for these purposes must be returned to MDF at the end of each year of the funding period.
- Second year funding of an MDF Ph.D. Traineeship or Postdoctoral Fellowship award is contingent upon documentation of satisfactory progress. A progress report satisfactory to 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.

Eligibility Requirements:

1. **Degree requirements. Ph.D. Trainee** 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 Fellow** 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 an 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.
2. **Commitment to myotonic dystrophy research.** MDF supports Ph.D. and postdoctoral trainees 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.

3. **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 Ph.D. Trainee or Fellow with application.

Submission Process and Requirements

Proposals cannot exceed 13 pages in length, and must be submitted in 12-point font. Proposals must be submitted via email to elizabeth.habeeb-louks@myotonic.org by September 6, 2019, at 5 PM Pacific Time.

The proposal must include the following sections:

A. Research Project Summaries

1. **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. (1 page)
2. **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 MDF (one-half page).
3. **Statement from the Applicant** 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 fellowship training to address important questions related to myotonic dystrophy, and demonstrable, long-term commitment to research related to myotonic dystrophy (2 pages).

B. Research Plan

The **Research Plan** must include the following information (total should not exceed 10 pages):

1. Specific Aims of the project (1 page);
2. Anticipated collaborative agreements, if applicable (1 page);
3. Background and Significance of the project (max. 2 pages);
4. Preliminary Data (2 or 3 pages);
5. Methods, Data Analysis Plan (including interpretation, expected results, and significance), and Pitfalls/Alternative Strategies (2 or 3 pages).

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

1. References;
2. Detailed budget in spreadsheet or table format;
3. Budget description and justification (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 MDF (see attached).

Review and Selection:

- All applications must be received by 5:00 PM Pacific Time on Monday, September 6, 2019.
- The 2020 Fellowship 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. Foundation staff 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 Ph.D. Trainee or Fellow and the likelihood that they will continue 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 propose 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 two dimensions.
- Applicants are encouraged to consult with the MDF Chief Executive Officer, Molly White (molly.white@myotonic.org) for refinement of their proposals before submission. Technical issues should be directed to MDF Research Coordinator Elizabeth Habeeb-Louks at elizabeth.habeeb-louks@myotonic.org.
- The 2020 Fellowship Advisory Committee will recommend final candidates to the MDF Board of Directors.
- The MDF Board of Directors will consider the 2020 Fellowship 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 fellowships does not signify a commitment to award any fellowships. If no applicant is deemed of sufficient scientific merit, expertise and/or skill, MDF may choose not to award a fellowship during this grant cycle.

Progress Reports:

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

- A **progress report** to be received by MDF 4 weeks after the end of each award year;
- A **final report** (including an abstract in lay language) submitted to MDF no later than one month

after completion of research at the end of the second year.

Expense Reports:

Each recipient must submit an expense report to MDF during the course of the two-year fellowship:

- An **interim expense report** (including the original proposed budget and final expenses on the grant) submitted to MDF no later than one month after completion of research at the end of the first year and should be submitted
- A **final expense report** (including the original proposed budget and final expenses on the grant) submitted to 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.
- 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.

Publication:

Ph.D. and Postdoctoral Fellow 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. The Myotonic Dystrophy Foundation 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, which should also acknowledge support from the Myotonic Dystrophy Foundation.

Timeline:

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Ph.D. Trainees and Postdoctoral Fellowship recipients are also highly encouraged to complete one grant writing training workshop provided by MDF during the course of the fellowship period. These are held at the MDF Annual Conference. A grant writing PowerPoint presentation is available for viewing on the MDF website at: <https://www.myotonic.org/grant-opportunities-awards>.

Conference & Publications:

- Ph.D. Trainee and Fellow must attend two MDF Annual Conferences and biennial IDMC conference and present a poster at the MDF Annual Conference (travel and expenses to be paid by MDF).
- MDF may publish the lay summary that accompanies the application for funded projects. This description should not contain information the Fellow does not wish to divulge to the general public.
- When a paper based on the work supported by an MDF fellowship is accepted for publication or presented before a scientific organization, an advance copy of the paper must be sent to MDF electronically at info@myotonic.org. MDF will respect journal embargoes and you are not

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.

- If the fellow is aware that a press release is being prepared about the work or the fellow has been contacted by a journalist, please let MDF know this is taking place. Fellows should encourage their University 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.
- All papers, exhibits, and press releases directly resulting from MDF funding shall carry a credit line to the Myotonic Dystrophy Foundation.
- Within four weeks of the conclusion of the fellowship, each recipient must submit an abstract of the results in lay language to MDF to be used for publication in its newsletter, annual report and other appropriate places.
- The title of each study funded by MDF, together with the lay language abstract of the research, the names of the Fellow, and the sponsor and the institution, will be published on the MDF website, in MDF newsletters, in annual reports and wherever else MDF deems appropriate. The Fellow will always be clearly acknowledged.