

MDF Fund-a-Fellow Postdoctoral Award Program Guidelines

The Myotonic Dystrophy Foundation (MDF) supports scientific investigations to:

- Enhance the quality of life of people living with myotonic dystrophy and
- Advance research focused on finding treatments and a cure for this disease.

As a part of this effort, the Foundation funds annual post-doctoral research fellowships (similar to NIH fellowship awards) to expand the knowledge and understanding of myotonic dystrophy.

Expanded 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.

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 brain lesions and patient-reported symptoms including central fatigue, excessive daytime sleepiness and difficulty in executive function, sometimes described as "brain fog." 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 has expanded the scientific research endeavors eligible for Fund-a-Fellow (FAF) 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 fellowship proposals for basic, clinical, or applied research related to myotonic dystrophy in:

- Pathogenesis of myotonic dystrophy
- DM progression related to the development of therapeutic trials
- Molecular basis of phenotypes (Type 1, 2, congenital)
- Identification and validation of drug treatment endpoints
- Best practices and clinical management, including nursing, social work, and psychology
- Standards of care and care integration
- Epidemiology, economics and support services

Therapeutic and diagnostic development

Applicants must have a Ph.D., M.D., D.O or other doctoral degree in their profession from an accredited institution. Doctoral degree must have been awarded no more than three years before the fellowship period begins. U.S. and international applicants are invited to apply.

Duration of 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 residency requirements of her/his institutional country, where applicable and be eligible to remain in her/his institutional country for the duration of the fellowship.

Payment:

- Award: \$100,000 for salary, benefits, and research support, paid over two years:
 - o \$50,000 will be paid no later than one week prior to activation date of the first year
 - o Assuming the first year report is satisfactory, \$50,000 will be paid at the beginning of the second program year.
- MDF awards are not to be used to fund institutional capital cost recovery, overhead or other indirect costs.
- Second year funding of an MDF Fund-a-Fellow award is contingent upon documentation of satisfactory progress. A progress report satisfactory to MDF is required eleven months after the award is activated.
- 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.** The applicant 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 Fund-a-Fellow award can be activated. Doctoral degree must have been awarded no more than three years before the fellowship period start date.
- 2. **Commitment to myotonic dystrophy research.** MDF supports postdoctoral new investigators with outstanding potential to make contributions to myotonic dystrophy research. Previous work in this area is not required. However, the applicant should document how previous experience and interests will enhance the Fund-a-Fellow 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. **DM Conference Participation.** MDF Fellows must attend MDF Annual Conferences and biennial IDMC (International Myotonic Dystrophy Consortium) conferences for the duration of the FAF grant period. MDF will provide travel stipends for travel, hotel and meal costs associated with conference attendance.
- 4. **Sponsor.** The applicant must identify a qualified sponsor to directly oversee and supervise the research activities. The 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. (A sponsor

who knows of a promising candidate can encourage and assist the prospective Fellow in applying.) The sponsor must attend a biennial International Myotonic Dystrophy Consortium (IDMC) conference with his/her Fellow (a stipend to cover travel and expenses will be paid by MDF to those sponsors who do not have institutional support to attend IDMC).

Review and Selection:

- All applications must be received by 5:00 PM Pacific Time on Friday, September 5, 2014.
- The 2014 FAF 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 40% of total score).
 - The likelihood that the Fellow 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 30% of total score).
 - The feasibility and scientific quality of the propose research. This will be assessed by a subject matter expert(s) 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 receive a low priority for funding regardless of the proposal's scores on the other two dimensions.
- Applicants are encouraged to consult with the MDF Executive Director for technical assistance and refinement of their proposals before submission.
- The 2014 FAF Advisory Committee will recommend final candidates to the MDF Board of Directors
- The MDF Board of Directors will consider the 2014 FAF Advisory Committee recommendations and determine final grant awards.

Awards are made at the sole discretion of the MDF Board of Directors. Availability of fellowships does not mandate 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 reports to MDF during the course of the two-year fellowship:

- A progress report to be received by MDF eleven months after commencement of the research;
- A final report (including an abstract in lay language) submitted to MDF no later than three
 months after completion of research at the end of the second year. The lay language final
 abstract will be published by MDF.

Final Publication:

Fund-a-Fellow recipients must also submit a scientific paper within six months of the conclusion of the research reporting the research findings. The recipient's work is further expected to result in other publications, conference presentations and other public contributions to the field. Fellows are

encouraged to collaborate with Sponsors on other scientific papers related to the research findings.

Timeline:

- September 5, 2014: Applications must be received by MDF by 5:00 PM Pacific Time.
- September 8-October 31, 2014: Applications reviewed by 2013 FAF Review Committee.
- December 19, 2014: Fellowship recipients receive preliminary notification via letter and by email.
- **January 5, 2015:** Confirmation of residency (if applicable), award announcement and earliest award activation date.
- **January 16, 2015:** Final deadline for recipients to notify MDF of acceptance or rejection of award. Any awards not accepted by this date will be deemed rejected and all obligation(s) on the part of the MDF to fund the fellow or sponsor shall be rescinded.
- April 17, 2015: Latest activation date of award.

Fund-a-Fellow recipients are also highly encouraged to complete one grant writing training workshop provided by MDF during the course of the fellowship period. Grant writing trainings will be offered once a year and will focus on helping Fellows develop and strengthen their grant writing skills and ability to attract research funding.

Conference & Publications:

- Fellow and Sponsor must attend MDF Annual Conferences and biennial IDMC conference (stipends for travel and expenses will be provided by MDF). In addition, to increase awareness of Fellows' DM research and inspire new researchers to join the field, MDF may invite Fellows to select one lab or research colleague to attend the biennial IDMC conference (selection to attend and a stipend for travel and expenses is at the discretion of 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 or exhibit based on the work supported by an MDF fellowship is published or presented before a scientific organization, a copy of the paper or a synopsis of the exhibit must be sent to the Myotonic Dystrophy Foundation electronically at info@myotonic.org. 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.
- Press releases regarding the study funded by MDF shall be emailed to info@myotonic.org.
- All papers, exhibits, and press releases related to the project shall carry a credit line to the Myotonic Dystrophy Foundation.
- At or before publication of any scientific papers related to the project by Fellow or Sponsor, each recipient must submit an abstract of the publication 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, 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.

Requirements for Submission:

In order to submit your application, please complete the following:

MDF Application Form

- Section 1: Administrative Information
- Section 2: Budget
- Section 3: Research Plan Summaries: one in scientific language and one in lay language;
 each not to exceed one page each.
- Section 4: Research Plan
- Candidate Curriculum Vitae including previous research and teaching experience.
- **Sponsor Biosketch** not to exceed four pages and using National Institutes of Health (NIH) form PHS 398 as a guide.
- One letter of support written by the sponsor describing the commitment and training to be provided and a brief overview of the other projects underway that might enhance the experience of the Fellow. This letter should also include a brief description of the laboratory or clinic space, equipment, and environment where the Fellow will be working.
- One letter of recommendation in addition to letter from sponsor. This letter should not be mailed to the applicant. Instead, we ask that the letter be a blind recommendation with a signed, hard copy either emailed to the Executive Director at info@myotonic.org or mailed to the MDF office and arriving at the address below (as noted below in "Where To Submit") no later than 5:00 PM Pacific Time, Friday, September 5, 2014.
- 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.
- **Proof of applicant's** successful completion of **Ph.D., M.D., D.O. or other doctoral degree** in the applicant's field. This requirement may be satisfied following the application date, provided that the applicant submits proof of successful completion prior to Friday, December 19, 2014.

Application by the Fellow and Sponsor shall evidence agreement to execute a fellowship agreement substantially in the form of the sample agreement attached hereto as Exhibit "A". Substantial additions or deletions to the fellowship agreement shall not be permitted and could cause MDF to terminate the Fellowship award. Fellows are encouraged to have their institutional representatives review this agreement and application prior to submission.

Where to Submit:

All application materials must be sent in one package to the Myotonic Dystrophy Foundation and received no later than 5:00 PM Pacific Time on Friday, September 5, 2014.

MYOTONIC DYSTROPHY FOUNDATION P.O. Box 29543 San Francisco, CA 94129 USA

Applicants must also submit an electronic copy of the entire application package to info@myotonic.org with the subject line "MDF Postdoctoral Fellowship Application" by the deadline. Please use the list of requirements above as a checklist when submitting your application, and keep a copy for your records. If you have specific questions about completing the application, you may contact MDF at info@myotonic.org. Final fellowship awards will be published on the MDF website in January, 2015.