



Postdoctoral Research Fellowship in Myotonic Dystrophy

Solicitation Name: 2017-Fellowship-Wyck

Contracting Office Address:

Place of performance: work does not have to take place in the UK or the US

Myotonic Dystrophy Foundation

1004A O'Reilly Avenue

San Francisco, CA 94129, USA

E-mail: elizabeth.habeeb-louks@myotonic.org

Phone: 415-800-7777

Contracting Officer: John Porter, Ph.D, Interim Chief Science Officer, MDF

Date Issued: 4 April 2016

Proposals Due: 11 July 2016, 5:00 PM PDT

Notification of Selection: 15 December 2016

Period of Award: 1 January 2017 – 31 December 2018

Anticipated Overall Award: \$102,500

Number of Awards: TBD

Synopsis:

The Wyck Foundation (Wyck) 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 a NIH fellowship award) to expand the knowledge and understanding of myotonic dystrophy.

Grant Focus Areas:

Myotonic dystrophy (DM) is a chronic disease with multiple dimensions that affect 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 central nervous system (CNS) effects that sometimes manifest as white matter 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, **the Wyck Foundation and MDF are expanding the scientific research endeavors eligible for 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.**

The Wyck Foundation and MDF are 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. Degree must be received within no more than three years before the fellowship period. 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 requirements of her/his institutional country for residency, where applicable.

Payment:

- Award: \$102,500 for salary, benefits, travel and research support, paid over two years:
 - \$51,250 will be paid no later than one week prior to activation date of the first year
 - Assuming the first year report is satisfactory, \$51,250 will be paid at the beginning of the second program year.
- Wyck Foundation awards are not to be used to fund institutional capital cost recovery, overhead or other indirect costs.
- Travel funds of approximately \$1,200/year are included to fund attendance by Fellows at the MDF Annual Conference. Travel funds not used for these purposes must be returned to the Wyck Foundation at the end of each year of the funding period.
- Second year funding of a Fellowship award is contingent upon documentation of satisfactory progress. A progress report satisfactory is required eleven months after the award is activated.
- The Wyck Foundation and MDF 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 a Fellowship award can be activated. Degree must be received within no more than three years before the fellowship period.
2. **Commitment to myotonic dystrophy research.** The Wyck Foundation and MDF support

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 Fellowship 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 Fellowship 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.)

Submission Process and Requirements

Proposals cannot exceed 10 pages in length, and must be submitted in 12-point font. Proposals must be submitted via email to elizabeth.habeeb-louks@myotonic.org by 11 July 2016 at 5 PM Pacific time.

The proposal must include the following (within 10 pages):

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. (maximum one 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 the MDF (maximum one page).

Research Plan

Please write a **Research Plan** that includes the following information (total should not exceed 9 pages):

1. A summary of the specific aims of the project (1 page)
2. Anticipated collaborative agreements if applicable (1 page)
3. The background and significance of the project (max. 2 pages)
4. Preliminary studies with figures if applicable - (2 or 3 pages)
5. Methods, expected results, data analysis, interpretation and significance - (2 or 3 pages)

In addition, (not included in the 10-page count) proposals must include:

1. References
2. Detailed budget in spreadsheet or table format
3. Accompanying budget description and justification (one paragraph)
4. Description of facility(ies) and equipment, if any, that will be used for the project (one paragraph)
5. Very brief: Name, degrees, training/qualifications, experience, and percent effort for Sponsor
6. CVs of all participating team members
7. Letter of support from the Sponsor
8. Face page provided by Wyck for this RFA (see attached).

Review and Selection:

- All applications must be received by 5:00 PM Pacific Time on 11 July 2016.

- The 2016 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 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 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 two dimensions.
- Applicants are encouraged to consult with the MDF Interim Chief Science Officer Dr. John Porter (john.porter@myotonic.org) for refinement of their proposals before submission. Technical issues should be directed to MDF Grants Manager Elizabeth Habeeb-Louks at elizabeth.habeeb-louks@myotonic.org.
- The 2017 Fellowship Advisory Committee will provide final assessments of candidate applications to the Wyck Foundation and MDF Boards of Directors.
- The Wyck Foundation Board of Directors will consider the 2017 Fellowship Advisory Committee assessments and determine final grant awards.

Awards are made at the sole discretion of the Wyck Foundation Board of Directors. 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, the Wyck Foundation may choose not to award a fellowship during this grant cycle.

Progress Reports:

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

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

Expense Reports:

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

- A **final expense report** (including the original proposed budget and final expenses on the grant) submitted no later than three months 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.**

Final Publication:

Fellowship recipients are also encouraged to submit at least one scientific paper for publication within six

months of the conclusion of the research reporting the research findings. Wyck 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.

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Postdoctoral Fellowship recipients are also highly encouraged to complete one grant writing training workshop during the course of the fellowship period. A grant writing PowerPoint presentation is available for viewing on the MDF Web site at: <http://www.myotonic.org/grant-opportunities>.

Conference & Publications:

- Fellow must attend the MDF Annual Conference and biennial IDMC conference (travel and expenses are included in Fellowship award).
- Wyck Foundation/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 a Fellowship is accepted for publication or presented before a scientific organization, an advance copy of the paper must be sent electronically to info@myotonic.org. Wyck Foundation and MDF will respect journal embargos 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 we understand that publishers have varied requirements regarding republication of papers selected for publication, we require that each award recipient submit a request to the publisher encouraging them to allow us to post the paper published on the Wyck Foundation and/or MDF websites. The publisher's decision on this matter shall be final and will not affect the research award or any future grants from the Wyck Foundation and/or 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 us know this is taking place. Fellows should encourage their University press offices or outside journalists to contact us so that publicity can be coordinated. Press releases regarding the study funded by the Wyck Foundation and MDF shall be emailed to info@myotonic.org.
- All papers, exhibits, and press releases directly resulting from Wyck Foundation funding shall carry a credit line to the Wyck Foundation.
- Within 12 weeks of the conclusion of the fellowship, each recipient must submit an abstract of the results in lay language to us to be used for publication in our newsletters, annual reports and other appropriate places.
- The title of each study, 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 Wyck Foundation and MDF websites, in our newsletters, in annual reports and wherever else we deem appropriate. The Fellow will always be clearly acknowledged.