

Doctoral and Postdoctoral Research Fellowships in Myotonic Dystrophy

Solicitation Name: 2021-Fellowship-Myotonic

Contracting Office Address:

Myotonic
663 Thirteenth Street
Oakland, CA, 94612, USA

E-mail: nadine.skinner@myotonic.org

Phone (Toll-Free): 86-MYOTONIC or 866-968-6642 (U.S. only)

Direct: 415-800-7777

Contracting Officer: Tanya Stevenson, Chief Executive Officer, Myotonic

Place of Performance: United States, Canada, or International sites that meet eligibility criteria

Date Issued: May 14, 2020

Proposals Due: September 18, 2020, 5:00 PM PDT

Notification of Selection: by December 18, 2020

Period of Award: January 1, 2021 – December 31, 2022

Anticipated Overall 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

Myotonic 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 doctoral and postdoctoral research fellowships (similar to a U.S. National Institutes of Health F31 or F32 award). 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, **Myotonic is expanding the scientific research endeavors eligible for Doctoral 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.**

Myotonic 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.
- **Predoctoral 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 Myotonic 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 Myotonic Annual Conference, and to the biennial IDMC meetings (see below).
- Myotonic 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 Myotonic Doctoral and Postdoctoral Fellows at the biennial 2021 International Myotonic Dystrophy Consortium meeting (IDMC) and the 2021 and 2022 Myotonic Annual Conferences. Travel funds not used for these purposes must be returned to Myotonic at the end of each year of the funding period.
- Second year funding of a Myotonic Doctoral or Postdoctoral Fellowship award is contingent upon documentation of satisfactory progress. A progress report satisfactory to Myotonic 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. 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 Myotonic 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.** Myotonic 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 Myotonic 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.

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 nadine.skinner@myotonic.org by September 18, 2020, 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 Myotonic (one-half page).
3. **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 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

Research Plan. This 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 Myotonic (see attached).

Review and Selection

- All applications must be received by 5:00 PM Pacific Time on Friday, September 18, 2020.
- The 2021 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 Doctoral or Postdoctoral Fellow’s commitment to the research 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 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 Myotonic Chief Executive Officer, Tanya Stevenson (tanya.stevenson@myotonic.org) for refinement of their proposals before submission. Technical issues should be directed to Myotonic Research Coordinator, Nadine Ann Skinner at nadine.skinner@myotonic.org.
- The 2021 Fellowship Advisory Committee will recommend final candidates to the Myotonic Board of Directors.
- The Myotonic Board of Directors will consider the 2021 Fellowship Advisory Committee recommendations and determine final grant awards.

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

Progress Reports

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

- A **progress report** to be received by Myotonic 4 weeks after the end of each award year;
- A **final report** (including an abstract in lay language) submitted to Myotonic 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 Myotonic 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 Myotonic 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 Myotonic 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.

Publications

Doctoral 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. Myotonic 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 Myotonic.

Timeline

Proposals Due: September 18, 2020, 5:00 PM PDT

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Doctoral and Postdoctoral Fellowship recipients are also highly encouraged to view the following grant writing PowerPoint presentation on the Myotonic website at: <https://www.myotonic.org/grant-opportunities-awards>.

Conference & Publications

- Doctoral and Postdoctoral Fellows must attend two Myotonic Annual Conferences and biennial IDMC conference and present a poster at the Myotonic Annual Conference (travel and expenses to be paid by Myotonic).
- Myotonic 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 Myotonic fellowship is accepted for publication or presented before a scientific organization, an advance copy of the paper must be sent to Myotonic electronically at info@myotonic.org. Myotonic 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 Myotonic understands that publishers have varied requirements regarding republication of papers selected for publication, Myotonic requires that each award recipient submit a request to the publisher encouraging them to allow Myotonic 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 Myotonic 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 Myotonic know this is taking place. Fellows should encourage their University press offices or outside journalists to contact Myotonic so that publicity can be coordinated. Press releases regarding the study funded by Myotonic shall be emailed to info@myotonic.org.
- All papers, exhibits, and press releases directly resulting from Myotonic funding shall carry a credit line to Myotonic.
- Within four weeks of the conclusion of the fellowship, each recipient must submit an abstract of the results in lay language to Myotonic to be used for publication in its newsletter, annual report, and other appropriate places.
- The title of each study funded by Myotonic, 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 Myotonic website, in Myotonic newsletters, in annual reports and wherever else Myotonic deems appropriate. The Fellow will always be clearly acknowledged.
- Fellows are required to provide one (1) 500-750 word research article to be potentially included in the Myotonic Research News each grant year.