

Myotonic Research Fellows Fund-A-Fellow Program

Who We Are

The Myotonic Dystrophy Foundation (Myotonic) is the world's largest patient organization dedicated solely to myotonic dystrophy (DM). Through community care, research, and advocacy, Myotonic's mission is to enhance the quality of life of people living with DM and accelerate research toward treatments and a cure.

Myotonic Dystrophy

Myotonic dystrophy is the most common form of the muscular dystrophies, with the most severe effects. At least 1 in 2,350 people worldwide have myotonic dystrophy type 1 (DM1) (Johnson 2018). However, the number may be far greater than this estimate, as misdiagnosis is still common. DM1 is the most common form of myotonic dystrophy with the most severe effects. DM1 can present at birth (congenital DM), during childhood (juvenile-onset DM), or as an adult-onset disorder. Myotonic dystrophy (DM2), also known as proximal myotonic myopathy (PROMM), is characterized as an adult-onset disorder only and is more frequently un- or mis-diagnosed than DM1. DM is a highly variable, multi-systemic genetic disorder for which targeted treatments are several years away and no cure exists.

Research for Treatment and a Cure

Myotonic supports scientific investigations to enhance the quality of life of people living with DM and advance research focused on finding treatments and a cure for this disease.

Why Fund-A-Fellow?

Since 2009, Myotonic has provided two-year pre- and postdoctoral research fellowships to support new and innovative studies relevant to the pathogenesis of DM, disease progression, best practices in clinical management of the disorder, and therapeutic and diagnostic development for myotonic dystrophy. Through this program Myotonic supports up-and-coming pre- and postdoctoral fellows to expand the base of committed DM researchers. Since 2009 Myotonic has committed over \$3M in total research funding to 34 fellows from 17 different institutions in five countries.

The goal of the Research Fellows program is to support adequate early career and ongoing funding in order to establish and drive a robust professional research community with a long-term commitment to the disease. The specific objectives that the Research Fellows program focuses on to achieve those goals are to:

1. Support young investigators pursuing DM discovery
2. Increase the scope and quality of publications of DM research

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Fellows also attend the Myotonic Annual Conference supporting their ability to interact with patients, learn about the disease, network, and gain motivation in their research areas. At the Conference, the fellows are able to present on their work, giving them a chance to share their research findings with the community working in the disease space. In addition to presenting their work at the Conference, fellows have presented at a diverse array of conferences and forums globally.

The program's success is measured via tracking publications by research fellows and annual project reporting from the fellows. During a 2019 survey of all of the former fellows, 100% of the respondents reported that they were satisfied with the program administration and research support. The fellows responded positively about their experience with the fellowship, specifically that they appreciated the opportunity to be a part of the program and a part of DM research. Many former fellows remained in the field after completing their fellowships. One example of this is a former fellow that now runs their own lab focused on diseases caused by repetitive DNA sequences that can be transcribed into toxic RNA and toxic proteins, particularly looking at myotonic dystrophy. The lab was awarded a 2018 Department of Defense Peer Review Medical Research Program grant to fund their DM work. The fellows that are still in the field cited that they remained in the field due to their interaction with the community, interest in the disease, and the research skills they gained.

The application process for fellows occurs annually. Fellows are selected by a panel of internationally recognized DM research experts that serve as Myotonic's Scientific Advisory Committee.

Past Fellowship Research

Past fellowship topics have included: exploring the design of synthetic proteins with potential therapeutic value, improving the current understanding of molecular events contributing to the cardiac symptoms in DM1 patients, generating a mouse model of DM2 to better understand the contribution of both the mutant Ribonucleic acid (RNA)s and mutant RAN proteins, and providing new mechanistic insights into central nervous system-associated behavioral symptoms in DM.

How You can Help

If you would like to support myotonic dystrophy research that will move us closer to treatment and a cure, please consider investing in academic research by Funding-A-Fellow. Your gift of \$110,000 will fund a two-year fellowship. Please contact Myotonic's Research Coordinator, Dr. Nadine Ann Skinner, at nadine.skinner@myotonic.org, or call our office directly at 415-800-7777.