Welcome to MDF's 2022 Advocacy Week Webinar!

Thursday, April 21st

Welcome!

- National Advocacy Committee Introduction & Goals
- MDF's Major Advocacy Victories
- Congressional Overview
- MDF 2022 Advocacy Goals
- How to Schedule and Do A Congressional Meeting

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MDF Board & National Advocacy Committee

National Board

David Berman, California
Martha Montag Brown, California *
John Day, MD, PhD, California
John Fitzpatrick, Texas
Liz Florence, Texas
David Herbert, Minnesota
Jeremy Kelly, California
Tom McPeek, Ohio
Joel Revill, Wyoming
Charles Thornton, MD, New York

Advocacy Committee

Martha Montag Brown, California *
Rebecca Coplin, Oregon
Belen Esparis MD, Pennsylvania
Charles Hunt, Georgia
Emily Jones, New York
Mindy Kim, North Carolina
Eric Wang, PhD, Florida
Rob Besecker, Illinois

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MDF's NAC Charge

- Develop new relationships and grow existing relationships with U.S. Representative and Senate staff from home state/congressional district
- Recruit new MDF grassroots advocates
- Identify possible MDF grasstops advocates (influence national or community leaders who have influence with federal elected officials)
- Assist MDF in hosting at least two grassroots advocate training webinars and a spring virtual lobby day

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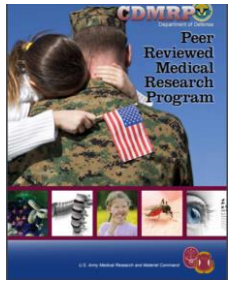
MDF's Advocacy Timeline



2014

- Kayla Vittek and her mom Lisa Harvey testified before Congress in support of reauthorization of the MD-CARE Act.
- $9M NIH funding

2015

- MDF Annual Meeting in Washington, DC features congressional briefing on DM Research Funding.
- $9M NIH funding

2016

- MDF hosts Patient-Focused Drug Development meeting, with FDA senior leadership, to highlight perspectives of patients and caregivers to accelerate DM treatments and a cure.
- $9M NIH funding

2017

- MDF convinces Social Security to add Congenital DM to Compassionate Allowance Program, enabling individuals to quickly qualify for disability benefits including health insurance coverage.
- Myotonic Dystrophy Voice of the Patient report delivered to FDA.
- $11M NIH funding

2018

- U.S. Senate adds DM to list of eligible conditions for research funding under the Dept of Defense Peer Reviewed Medical Research Program (PRMRP).
- $3.1M PRMRP
- $13M NIH funding

2019

- Tim Haylon testified before the House Appropriations Committee to increase federal funding for DM research at the National Institutes of Health (NIH).
- $2.4M PRMRP
- $12M NIH funding

2020

- DM Research Provision in House Appropriations Report: "...support current efforts to develop the first ever FDA approved treatment for this inherited genetic disorder."
- $2.3 PRMRP
- $13M NIH funding

2021

- Senator Tim Kaine introduces Senate Resolution 336, Declaring Sept 15th International Myotonic Dystrophy Awareness Day!
- Grant awards for PRMRP pending
- NIH awards pending, $11M estimated

Notes: NIH also funded $63M in 2008-2013, totaling over $150 million since 2008.

International Myotonic Dystrophy Awareness Day
September 15th

- **Global Alliance for DM Awareness:** 51 organizations, institutions, and companies joined worldwide, with MDF facilitating and leading monthly meetings since June 2021
- **Senate Resolution to declare 9/15 International DM Awareness Day:** S.Res.336 was introduced on 8/4 by lead sponsor Sen. Tim Kaine and co-sponsor Sen. Amy Klobuchar. Senators Lummis (WY), Collins (ME), and Smith (MN) have also signed on. Senate vote expected in 2022.
- **Virtual celebration on 9/15:** MDF created and shared a video to celebrate the global DM community, Global Alliance, standout DM advocates, and DM research and drug development progress, with over 660 views to date
- **Inspiring our Global Alliance partners:** **CureDM in the UK** campaigned to light up 44+ landmarks/monuments in green using MDF's letter template; **Partners in Canada** successfully passed an official DM Awareness Day Proclamation in Manitoba Province using the US Senate Resolution template; **Multiple orgs** used the MDF Social Media Toolkit to create social media campaigns

Resources for our Community

Social Media Toolkits – *ready-to-post templates with facts about DM*

Write a Letter to Your Senator - *instructions and templates for gathering support for S.Res.336*

1-page DM Fact Sheet – *printable and sharable at-a-glance DM fact sheet*

Awareness T-Shirts & Mugs - *branded t-shirts and a mug to show support and start conversation*

Window Sign Campaign – *printable signs with the Awareness Day logo & sample request letter*

Story Booth – *partner org collecting patient stories in a medical research archive. 1 DM story already posted, 2 more stories on the way*

***Events in a Box** – templates for creating local awareness or fundraising events*

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Homepage: myotonic.org/international-dm-day | 9/15 Virtual Celebration: https://youtu.be/FLsyUK6YCCI

Senate Resolution: www.congress.gov/bill/117th-congress/senate-resolution/336

Meet the Global Alliance: https://youtu.be/HjUYBjQ1esY | Merch: www.bonfire.com/store/myotonic-dystrophy-foundation

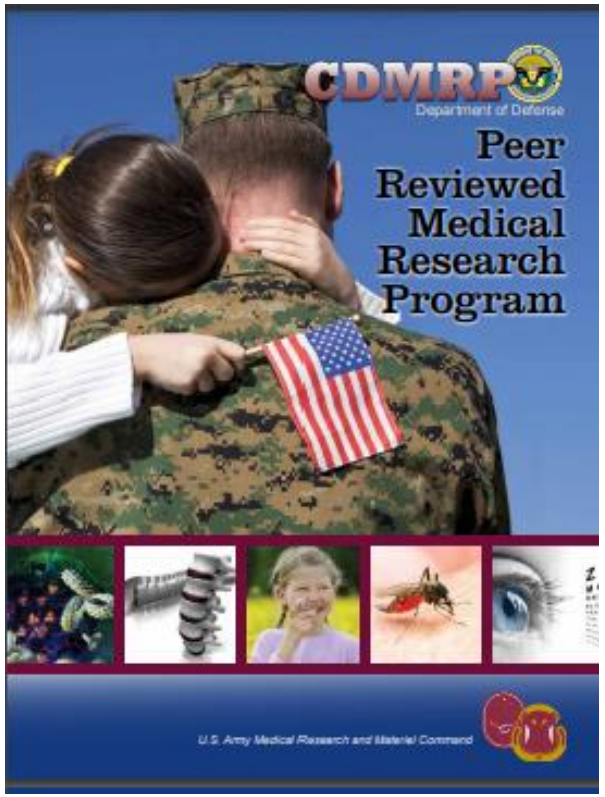
Myotonic Dystrophy Included In PRMRP 5th Year In A Row

PRMRP Has Funded $7.7 M (FY2018-20) In New DM Research Funding

- FY21 DM Research Awards (Pending)

PRMRP DM Senate Requests

- Senators Kaine (D-VA), Brown (D-OH), Coons (D-DE) Capito (R-WV), Murphy (D-CT), Feinstein (D-CA), Menendez (D-NJ), Cornyn (R-TX), and Barrasso (R-WY)



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MDF Grassroots Advocacy Success

- Martha Montag Brown (MDF Vice Chair)
- Senator Dianne Feinstein (D-CA)
  - Senior Democrat on Senate Appropriations Committee
- Mobilized California Friends & Family
- Met with Senator's CA & DC Staff
- Secured 1st Letter of Support for DM PRMRP Funding

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Congressional Overview

- Democrats Hold Narrow House & Senate Majorities
- Partisanship Remains High
- November Mid-Term Elections Coming Into Focus
- Final FY22 Omnibus Passage Starts FY23 Appropriations Process
- PDUFA, Pandemic Preparedness, Mental Health, and Other Anticipated Bills

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Congressional Leadership

Speaker of the House
Nancy Pelosi
(D-CA)

Senate Majority Leader
Chuck Schumer
(D-NY)

House Minority Leader
Kevin McCarthy
(R-CA)

Senate Majority Leader
Mitch McConnell
(R-KY)

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Appropriations Process

- Federal fiscal year is October 1 to September 30
- FY23 Presidential Budget Submission (March)
- Adoption of Congressional Budget Resolution
- Appropriations Hearings
- House Bill Passage (June/July)
- Senate Follows House
- House – Senate Conferences

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2022 MDF Advocacy Goals

- Fiscal Year 2023 U.S. Senate Defense Appropriations Report Includes Myotonic Dystrophy As Eligible Condition As Part Of Department of Defense Peer Review Medical Research Program (PRMRP)

- Secure House Support for Myotonic Dystrophy Line Item In Fiscal Year 2023 Congressionally Directed Medical Research Program (CDMRP)

- Fiscal Year 2023 Labor Health and Human Services Appropriations Report Includes NIH Repeat Expansion Diseases Initiative Report Language

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Proposed FY23 Labor, HHS Report Language

Repeat Expansion Diseases.—*The Committee recognizes the rapidly emerging science on DNA repeat expansions, which causes over 50 distinct diseases, including myotonic dystrophy (DM1 and DM2), the most common genetic cause of amyotrophic lateral sclerosis/frontotemporal dementia (C9ORF72), and Huntington's disease. Due to recently developed sequencing technologies, a common thread has recently emerged, that repeat expansions may underlie multiple neurodegenerative conditions. The Committee encourages NIH to increase federal funding for research on repeat expansions and consider new funding mechanisms across multiple institutes to support scientific discoveries that will lead to treatments and cures for these genetic disorders and related conditions. The Committee requests an update on these activities in the fiscal year 2023 Congressional Budget Justification.*

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Successful Congressional Meetings

- In-Person vs. Virtual Meetings
  - Zoom or Calls
- Member and/or Staff Meetings
- Tell Your Story
- Share Leave Behind Materials
- Staff Questions
- Close With Our Asks
- Always Follow-Up

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Congressional Leave Behinds

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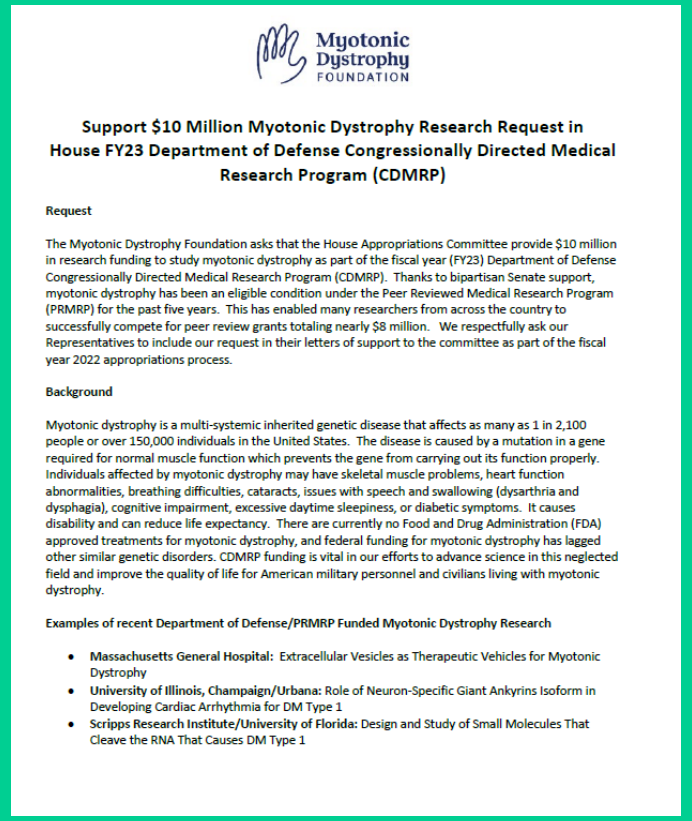
Support the NIH Repeat Expansion Disease Initiative (REDI)
Accelerate Discoveries in Myotonic Dystrophy and Related Genetic Diseases

Repetitive sequences comprise most of the human genome and have been a major roadblock to obtaining the full sequence of the human genome. It was only recently that the NIH-funded T2T consortium was able to generate full, end-to-end sequencing of all human chromosomes, published in *Science*, in April 2022. These repetitive sequences are the source of over 50 (and growing) distinctive disorders caused by DNA repeat expansions. Myotonic dystrophy type 1 and 2 are repeat expansion diseases and have served as paradigms for a class of diseases caused by repeat instability and toxic RNA, which includes C9ORF72/amyotrophic lateral sclerosis/frontotemporal dementia, Huntington's disease, and many common forms of dominantly inherited ataxia. Over the past several decades, researchers have begun to understand how these mutations drive pathogenesis in many of these diseases, but new repeat expansion diseases continue to be discovered, and the recent genome sequencing data described above provides a new roadmap for both normal and pathologic functions of repeats.

Myotonic dystrophy type 1 (DM1) and type 2 (DM2) are caused by a CTG repeat expansion in the DMPK gene and a CCTG repeat expansion in the CNBP gene, respectively. These repeats are typically present in <30-50 contiguous copies in healthy individuals, but can expand to hundreds or thousands of copies in patients. These expansions prevent cells in the muscles, heart, and brain from functioning normally, leading to symptoms of myotonic dystrophy. Affecting as many as 1 in 2,100 individuals, myotonic dystrophy is the most common form of adult muscular dystrophy and considered the most variable of all known conditions; however, there is currently no cure and there are no approved treatments.

While U.S.-based researchers at the University of Florida, University of Rochester, Stanford University, Emory University, Baylor College of Medicine, and others are making progress to study how repeat expansions disrupt healthy gene regulation and cause disease symptoms, federal funding and coordination have been limited. Recognizing the current exciting landscape in biomedical research, the recently completed *full* sequence of the human genome, and emerging broad interest in repeat expansion diseases among patient advocacy groups and basic scientists, the Myotonic Dystrophy Foundation and our Scientific Advisory Committee urges Congress and the NIH to establish a new trans-NH Repeat Expansion Disease Initiative (REDI) within the Office of the NIH Director to fund new research and accelerate scientific discovery in this important new field.

For example, we believe new federal research investments in the use of short- and long-read sequencing, high-throughput/high-resolution imaging, high throughput DNA synthesis technologies, and use of patient-derived cell biological tools/reagents will help scientists to better understand how the repeating RNAs cause a myriad of symptoms in virtually all tissues of the body, including skeletal, cardiac, and smooth muscle, and tissues of the central nervous system. We believe that new investments in studies of DM pathogenesis and RNA regulation will accelerate efforts to identify treatments and eventually cures for DM and other related diseases.



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Support $10 Million Myotonic Dystrophy Research Request in House FY23 Department of Defense Congressionally Directed Medical Research Program (CDMRP)

Request

The Myotonic Dystrophy Foundation asks that the House Appropriations Committee provide $10 million in research funding to study myotonic dystrophy as part of the fiscal year (FY23) Department of Defense Congressionally Directed Medical Research Program (CDMRP). Thanks to bipartisan Senate support, myotonic dystrophy has been an eligible condition under the Peer Reviewed Medical Research Program (PRMRP) for the past five years. This has enabled many researchers from across the country to successfully compete for peer review grants totaling nearly $8 million. We respectfully ask our Representatives to include our request in their letters of support to the committee as part of the fiscal year 2022 appropriations process.

Background

Myotonic dystrophy is a multi-systemic inherited genetic disease that affects as many as 1 in 2,100 people or over 150,000 individuals in the United States. The disease is caused by a mutation in a gene required for normal muscle function which prevents the gene from carrying out its function properly. Individuals affected by myotonic dystrophy may have skeletal muscle problems, heart function abnormalities, breathing difficulties, cataracts, issues with speech and swallowing (dysarthria and dysphagia), cognitive impairment, excessive daytime sleepiness, or diabetic symptoms. It causes disability and can reduce life expectancy. There are currently no Food and Drug Administration (FDA) approved treatments for myotonic dystrophy, and federal funding for myotonic dystrophy has lagged other similar genetic disorders. CDMRP funding is vital in our efforts to advance science in this neglected field and improve the quality of life for American military personnel and civilians living with myotonic dystrophy.

Examples of recent Department of Defense/PRMRP Funded Myotonic Dystrophy Research

- **Massachusetts General Hospital:** Extracellular Vesicles as Therapeutic Vehicles for Myotonic Dystrophy
- **University of Illinois, Champaign/Urbana:** Role of Neuron-Specific Giant Ankyrins Isoform in Developing Cardiac Arrhythmia for DM Type 1
- **Scripps Research Institute/University of Florida:** Design and Study of Small Molecules That Cleave the RNA That Causes DM Type 1

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Key Advocacy Dates

- Congressional Appropriations Submissions (March/April)
- First Advocacy Webinar
  - April 21st @ 3PM EDT
- MDF Virtual Advocacy Week
  - May 2-6
- House Completes L-HHS Appropriations (June/July)
- Senate Completes L-HHS Appropriations (July/September)
- Second Advocacy Webinar
  - September 15th @ 3PM “International DM Day”

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MDF Advocacy Tracker

- Please Email Kevin Brennan(kbrennan@bluebird-strategies.com) to confirm your virtual meetings and/or emails/calls with your Senators & Representatives and their staff
  - This allows us to better understand how we are building awareness and to follow-up on these requests
- We are also available to answer follow-up questions regarding your requests and meetings

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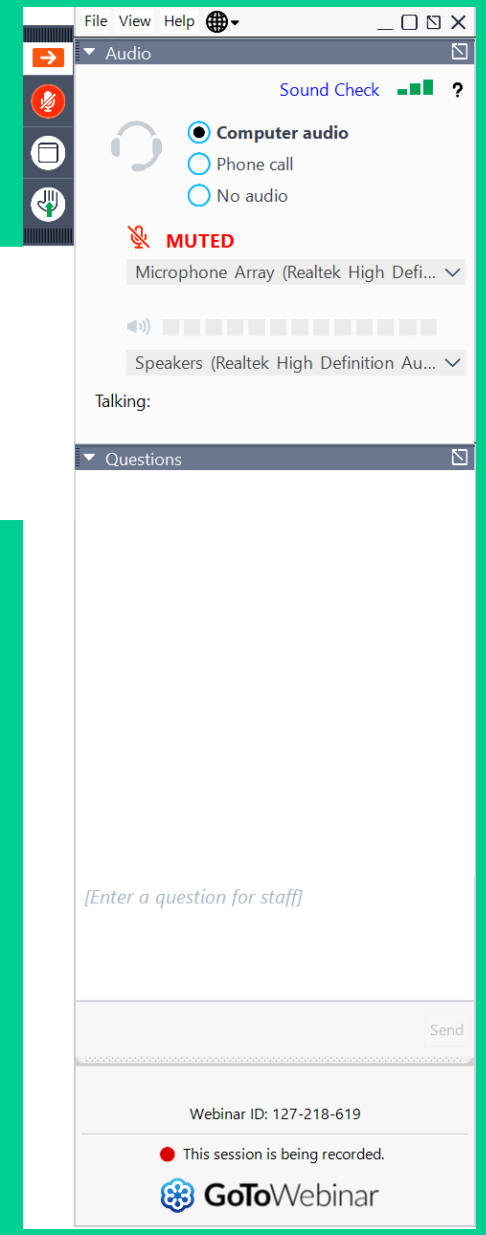
Ask Questions Live!

Desktop:

1. Open to the “Questions” tab.
2. Type your question & click send!

Smart Phone:

1. Click on the “?” icon at the top of the screen.
2. Type your question & click send!



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Thank You!