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
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
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
# 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.
- $9M 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 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 Lummins (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
- **Virtual participation around the world**: In addition to our own social media campaign, MDF re-shared hundreds of social media posts by other community members around the world
- **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

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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 | I-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 |

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


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)
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
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
Congressional Leadership

Speaker of the House
Nancy Pelosi
(D-CA)

House Minority Leader
Kevin McCarthy
(R-CA)

Senate Majority Leader
Chuck Schumer
(D-NY)

Senate Majority Leader
Mitch McConnell
(R-KY)
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
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
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.
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
Support the NIH Rare Event Disease Initiative (REDI)
Accelerate Discoveries in Myotonic Dystrophy and Related Genetic Diseases

Exome sequencing comprised of the human genome and have been a major tool to
estaging the clinical features of patients with myotonic dystrophy. It is only recently
that the field of redi has advanced, allowing for the identification of new genes and
mutations that contribute to the disease. This is a significant development as it opens
the door to potential new therapeutic strategies for patients with myotonic dystrophy.

Myotonic dystrophy type 1 (DM1) is a disease caused by a CTG repeat expansion in the
DMPK gene on chromosome 19. The expansion causes a protein called myotonin pero
kinase (dysferlin) to be produced at high levels in muscles, leading to muscle weakness and
dystrophy. The disease is characterized by muscle weakness, fasciitis, and other
manifestations. The disease is progressive and can cause significant morbidity and
mortality.

While U.S.-based researchers at the University of Florida, University of Rochester, Vanderbilt University, Emory University, and other institutions are conducting important research, Myotonic Dystrophy Foundation and our Scientific Advisory Committee urge Congress and the NIH to establish a new trans-
it national research initiative (REDI) within the Office of the NIH Director to fund new researchers and accelerate scientific discovery in this important new field.

For example, we believe new federal research investments in the area of redi and next
gen sequencing, high-throughput high-resolution imaging, high-throughput DNA synthesis techniques, and other areas of biological research will help us understand how the expanding RNA causes a variety of symptoms in virtually every tissue of the body, including heart, brain, and smooth muscle, and how the primary mutations lead to the disease.

We believe that new investments in studies of DM pathogenesis and RNA regulation will accelerate efforts to identify treatments and eventually cure for DM and other related diseases.

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

Request

The Myotonic Dystrophy Foundation and its supporters believe that the Department of
Defense should continue to support research in myotonic dystrophy. This program
provides important funding for research that is not always supported by other agencies.

The CDMRP program has a long history of supporting research in myotonic
 dystrophy, and it has been a pivotal source of funding for many important studies.

We urge Congress and the NIH to establish a new research initiative (REDI) within
the Office of the NIH Director to fund new researchers and accelerate scientific
discovery in this important new field.
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”
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
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!
Thank You!