2023 Rare Disease Day

Raising Our Voice To Increase Federal DM Research Funding & Find A Cure

February 28, 2023
What Is Rare Disease Day?

• On February 28, 2008, a global movement of rare disease advocates was launched to advance social opportunity, healthcare, and access to diagnosis and therapies for people living with a rare disease

• Aims to change and improve the lives of the 300 million people across the world living with a rare disease

• Includes individuals, families, caregivers, healthcare professionals, researchers, clinicians, policy makers, industry representatives working to raise awareness and take action
Myotonic Dystrophy Global Alliance
15 Countries & 57 Partners

- Nonprofits
- Researchers & Academia
- Hospitals & Clinics
- Schools
- Biotech/Pharma

www.myotonic.org/international-dm-day
### A Decade of MDF Advocacy Advances

<table>
<thead>
<tr>
<th>Year</th>
<th>Events</th>
</tr>
</thead>
</table>
| 2014 | • Kayla Vittek and her mom Lisa Harvey congressional testimony in support of the MD-CARE Act  
$9M NIH funding |
| 2015 | • 1st MDF Annual Meeting in Washington, D.C. features US Senate briefing on DM Research Funding  
$9M NIH funding |
| 2016 | • MDF hosts 1st ever DM Patient-Focused Drug Development “PFDD” meeting, with FDA to stress urgency for patient centered DM treatments  
$9M NIH funding |
| 2017 | • Social Security adds Congenital DM to Compassionate Allowance Program, enabling individuals to quickly qualify for disability benefits including health insurance coverage  
Myotonic Dystrophy PFDD Voice of the Patient report released  
$9M NIH funding |
| 2018 | • U.S. Senate adds DM to Peer Reviewed Medical Research Program (PRMRP)  
$3.1M PRMRP  
$13M NIH funding |
| 2019 | • Tim Haylon testifies before the House Appropriations Committee urging more DM research at NIH  
$2.4 M PRMRP  
$12M NIH Funding |
| 2020 | • Congress includes provision in annual spending bill urging increased federal funding for DM research citing need “to develop the first ever FDA approved for this inherited genetic disorder.”  
$2.3M PRMRP  
$13M NIH funding |
$300K PRMRP  
$11M NIH Funding |
| 2022 | • Senate passes International DM Awareness Day resolution.  
• Congress launches NIH Repeat Expansion Disorder Initiative REDI  
• Record $8.8 M in DM PRMRP Funding; $12M NIH Funding |
MDF Research Advocacy Priority

OBJECTIVE: Secure U.S. Senate Support for DM Research Eligibility in Fiscal Year 2024 Department of Defense Peer Reviewed Medical Research Program (PRMRP)

- Conditions/Diseases Must Be Reapproved Every Year
- DM Eligible 6 Years In A Row
- PRMRP Has Funded $17 Million in DM Research

Senate Champions:

Congressionally Directed Medical Research Program

• Established by Congress in 1992
• Early Focus on Breast Cancer Research
• Expanded Focus on “Warfighter”
• 35 Research Programs
  • Peer Reviewed Medical Research Program (PRMRP)
Myotonic Dystrophy PRM RP Eligibility Process

• Each spring a US Senator must intentionally add diseases/disorders to the PRM RP funding eligibility list

• Without Senate advocacy, myotonic dystrophy will not be eligible to receive research funding through PRM RP
Peer Reviewed Medical Research Program
Myotonic Dystrophy Research

<table>
<thead>
<tr>
<th>Fiscal Year 2018</th>
<th>Fiscal Year 2019</th>
<th>Fiscal Year 2020</th>
<th>Fiscal Year 2021</th>
<th>Fiscal Year 2022</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>$3.1 Million</td>
<td>$2.4 Million</td>
<td>$2.3 Million</td>
<td>$300,000</td>
<td>$8.8 Million</td>
<td>$16.9 Million</td>
</tr>
</tbody>
</table>

Source: https://cdmrp.army.mil/search.aspx
## National Institutes of Health

### Myotonic Dystrophy Research

<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Myotonic Dystrophy</td>
<td>$9</td>
<td>$9</td>
<td>$9</td>
<td>$11</td>
<td>$13</td>
<td>$12</td>
<td>$24</td>
<td>$11</td>
<td>$12</td>
<td>$11</td>
</tr>
</tbody>
</table>

[https://report.nih.gov/funding/categorical-spending/](https://report.nih.gov/funding/categorical-spending/)
Congressional Budget Process
Budget Process Timeline

- State of the Union Address
- President’s Budget Released (Early March)
- Congressional Budget Resolution
- House & Senate Appropriations Hearings & Mark-Ups
- House – Senate Conference
- New Fiscal Year, October 1st
We Want You To Be A Myotonic Dystrophy Foundation Advocate
Rare Disease Day Call to Action

• Contact Your U.S. Senators

• Participate in DM Research Studies and Clinical Trials

• Sign-Up for Myotonic Dystrophy Family Registry

• Be a Self-Advocate
How to Start A Relationship With Your U.S. Senator

www.vanhollen.senate.gov
Getting To Know Your Senator’s Website
Sending MDF Emails on Senator’s Website
Request A Meeting on Senator’s Website

SCHEDULING REQUESTS
Thank you for reaching out to schedule a meeting or invite me, and my staff, to an event. Due to ongoing COVID-19 and Capitol Hill campus restrictions, meetings are primarily held virtually over video conference or by telephone. Please fill out the appropriate form below to submit your request with as much information as you can provide. Someone from my office will be in contact with you as soon as possible.

Washington, DC Request
Maryland Request
Schedule A Meeting
Schedule An Event Or Speaking Engagement

Schedule a Meeting in DC
Your Information
First Name* Required
Last Name* Required
Organization*
What Do I Say? It’s Only 4 Steps!

1. Name/Hometown
2. Personal Story
3. Please Include DM As Eligible Condition in FY24 DoD PRMRP
4. Thank You and I Look Forward to Your Reply

February 28, 2023

The Honorable Jane Doe
1234 Senate Office Building
Washington, D.C. 20510

Dear Senator,

As a Myotonic Dystrophy Foundation advocate from your home state, I am writing to ask for your support to maintain eligibility for myotonic dystrophy research awards for the 2024 fiscal year in accordance with the Department of Defense (DoD) Peer-Reviewed Medical Research Program (PRMRP) eligibility guidelines.

DoD PRMRP has funded $16 million in new myotonic dystrophy research research which has helped advance our understanding of this rare genetic disorder. I was diagnosed with myotonic dystrophy, care for a family member living with myotonic dystrophy, or am a friend of a person living with myotonic dystrophy and I would appreciate your support.

Myotonic dystrophy is a multi-systemic inherited genetic disease that affects as many as 3 in 3,100 people or over 120,000 individuals in the United States. It impacts adults and children as well as veterans and active-duty military personnel. While there is limited prevalence data for this rare genetic disorder, the Myotonic Dystrophy Foundation has worked with many veterans who were undiagnosed during their service and unfairly discharged because the disease prevented them from carrying out their duties. Some tasks like buttoning up protective gear like a gas mask or attaching dangerous munitions to aircraft.

We believe new research funding will help us better understand and diagnose myotonic dystrophy, and discover new treatments and a cure which will benefit civilians, active-duty military personnel, and veterans.

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 slowed muscle problems, heart function abnormalities, breathing difficulties, ataxia, acne, speech and swallowing difficulties, and disfigurement, cognitive impairments, excessive drooling, and dry eyes or diabetes. Some veterans with undiagnosed myotonic dystrophy sometimes have myotonic dystrophy and develop end stage myotonic dystrophy and develop symptoms which grow more serious as they grow older.

Cognitive impairments, daytime sleepiness and muscle problems are often viewed as a lack of military discipline rather than symptoms of a serious disease. It lead to discharge and a loss of veterans benefits. Afterward, are those veterans diagnosed and begin treatment.

Myotonic dystrophy also 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. I would deeply appreciate your support of our request and look forward to your reply.

Sincerely,

[Logo] Myotonic Dystrophy Foundation
Call to Action

- Email, Call, or Meet with Your Senators and Their Staff
  - www.myotonic.org/myotonic-dystrophy-advocacy

- Participate in a DM Research Studies & Clinical Trials
  - www.myotonic.org/study-trial-resource-center

- Join DM Family Registry
  - https://myotonicregistry.patientcrossroads.org/
International Myotonic Dystrophy Advocacy

• The Problem to Be Solved
  • Improve Medical Care, Increase Awareness, Improve Disability Benefits?
• Find Out Who Represents You
  • Legislative Representative(s)
• Draft Your Proposal
• Call, Email, or Request A Meeting
• Invite Families, Physicians, Others to Join
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!

Myotonic Dystrophy Foundation
Today’s Program is Being Recorded

Please refer to the MDF Digital Academy to view today’s recording at:

www.myotonic.org/digital-academy
Join the Myotonic Dystrophy Advocacy Movement to Fund Research!

Contact: Kevin Brennan
kbrennan@bluebird-strategies.com