

# 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

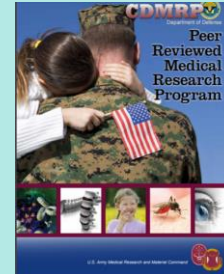
# Myotonic Dystrophy Global Alliance 15 Countries & 57 Partners



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

[www.myotonic.org/international-dm-day](http://www.myotonic.org/international-dm-day)

# A Decade of MDF Advocacy Advances



2014

2015

2016

2017

2018

2019

2020

2021

2022

- Kayla Vittek and her mom Lisa Harvey congressional testimony in support of the MD-CARE Act

- \$9M NIH funding

- 1<sup>st</sup> MDF Annual Meeting in Washington, D.C. features US Senate briefing on DM Research Funding

- \$9M NIH funding

- MDF hosts 1<sup>st</sup> ever DM Patient-Focused Drug Development “PFDD” meeting, with FDA to stress urgency for patient centered DM treatments

- \$9M NIH funding

- 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

- \$11M NIH funding

- U.S. Senate adds DM to Peer Reviewed Medical Research Program (PRMRP)

- \$3.1M PRMRP

- \$13M NIH funding

- Tim Haylon testifies before the House Appropriations Committee urging more DM research at NIH

- \$2.4 M PRMRP

- \$12M NIH Funding

- 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

- Sen. Kaine introduces Sen. Res 336, declaring 9/15 International Myotonic Dystrophy Awareness Day.

- \$300K PRMRP

- \$11M NIH Funding

- 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

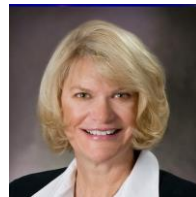
**OBJECTIVE:** Secure U.S. Senate Support for DM Research Eligibility in Fiscal Year 2024  
Department of Defense Peer Reviewed Medical Research Program (PRMRP)

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

## Senate Champions:



Sen. Tim Kaine  
Virginia (D)



Sen. Cynthia Lummis  
Wyoming (R)



Sen. Amy Klobuchar  
Minnesota (D)



Sen. Dick Durbin  
Illinois (D)



Sen. Susan Collins  
Maine (R)

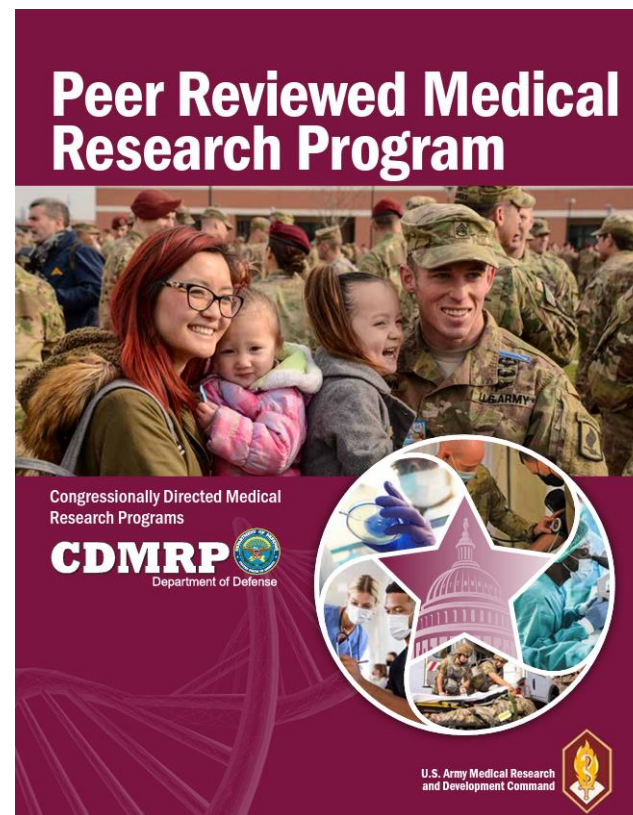


Sen. Dianne Feinstein  
California (D)



# 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 PRMRP Eligibility Process

- Each spring a US Senator must intentionally add diseases/disorders to the PRMRP funding eligibility list
- Without Senate advocacy, myotonic dystrophy will not be eligible to receive research funding through PRMRP

# Peer Reviewed Medical Research Program Myotonic Dystrophy Research

Fiscal Year 2018	Fiscal Year 2019	Fiscal Year 2020	Fiscal Year 2021	Fiscal Year 2022	Total
\$3.1 Million	\$2.4 Million	\$2.3 Million	\$300,000	\$8.8 Million	\$16.9 Million



Source: <https://cdmrp.army.mil/search.aspx>



# National Institutes of Health Myotonic Dystrophy Research

Research/Disease Areas (Dollars in millions and rounded)	2014	2015	2016	2017	2018	2019	2020	2021	2022 Estimated	2023 Estimated
Myotonic Dystrophy	\$9	\$9	\$9	\$11	\$13	\$12	\$24	\$11	\$12	\$11

<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

CONGRESS.GOV    Advanced Searches    Browse    Search Tools    Support    Sign In

Legislation    Congressional Record    Committees    Members


This Member    Examples: education, "post office"    Search Within

Home > Members > Senator Chris Van Hollen    Citation    Subscribe    Share/Save    Site Feedback

### Senator Chris Van Hollen (1959 - )

In Congress 2003 - Present | [Get alerts](#)

**MEMBER**    Hide Overview



Courtesy U.S. Senate Historical Office  
[Read biography](#)

**Website** <https://www.vanhollen.senate.gov>

**Contact** 110 Hart Senate Office Building Washington, DC 20510  
(202) 224-4654  
[Contact](#)

**Party** Democratic

**Senate** Maryland 115th-117th (2017-Present)

**House** Maryland, District 8 108th-114th (2003-2017)

**More on This Member**  
[See This Member's Remarks in the Congressional Record](#)  
[View Member Committee Assignments \(Senate.gov\)](#)

[www.vanhollen.senate.gov](https://www.vanhollen.senate.gov)





# Getting To Know Your Senator's Website





# Sending MDF Emails on Senator's Website

**CHRIS VAN HOLLEN**  
U.S. SENATOR FOR MARYLAND

ABOUT CONSTITUENT SERVICES ISSUES NEWS CONTACT INVESTING IN MD

Contact

## EMAIL SENATOR VAN HOLLEN

Share Your Opinion On Legislation

Find Assistance, Resources & More

Please use the following form to contact me with your comments and concerns. Please include your full name and address to receive a written response.

Your Name

Prefix

--

First Name\* Last Name\*

Required Required

Your Contact Information

Street Address\* Street Address (2)

Required



# Request A Meeting on Senator's Website

The screenshot shows the 'SCHEDULING REQUESTS' page on the website of Chris Van Hollen, U.S. Senator for Maryland. The page has a dark blue header with the senator's name and title on the left, and navigation links (ABOUT, CONSTITUENT SERVICES, ISSUES, NEWS, CONTACT, INVESTING IN MD) and social media icons on the right. Below the header, the page is titled 'SCHEDULING REQUESTS' and includes a paragraph explaining that meetings are primarily held virtually due to COVID-19. There are four buttons: 'Washington, DC Request' (dark blue), 'Maryland Request' (medium blue), 'Schedule A Meeting' (dark blue), and 'Schedule An Event Or Speaking Engagement' (medium blue). Below these buttons is a section titled 'Schedule a Meeting in DC' with a sub-section 'Your Information'. This section contains three input fields: 'First Name\*' (with 'Required' text inside), 'Last Name\*' (with 'Required' text inside), and 'Organization\*'. The 'Organization\*' field is currently empty.

CHRIS VAN HOLLEN  
U.S. SENATOR FOR MARYLAND

ABOUT CONSTITUENT SERVICES ISSUES NEWS CONTACT INVESTING IN MD

Contact

## 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\* Last Name\*

Required 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 7<sup>th</sup> year in a row as part of the fiscal year 2024 Department of Defense (DoD) Peer-Reviewed Medical Research Program (PRMRP). To date PRMRP has funded \$16 million in new myotonic dystrophy 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 1 in 2,100 people or over 150,000 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 on 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 simple tasks like putting on 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 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. Americans entering military service with undiagnosed myotonic dystrophy oftentimes have mild symptoms which grow more serious as they grow older. These cognitive impairments, daytime sleepiness and muscle problems are often viewed as a lack of military discipline rather than symptoms of a serious disease. It leads to discharge and a loss of veterans' benefits. Only afterwards, are these 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,

# Call to Action

- Email, Call, or Meet with Your Senators and Their Staff
  - [www.myotonic.org/myotonic-dystrophy-advocacy](http://www.myotonic.org/myotonic-dystrophy-advocacy)
- Participate in a DM Research Studies & Clinical Trials
  - [www.myotonic.org/study-trial-resource-center](http://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



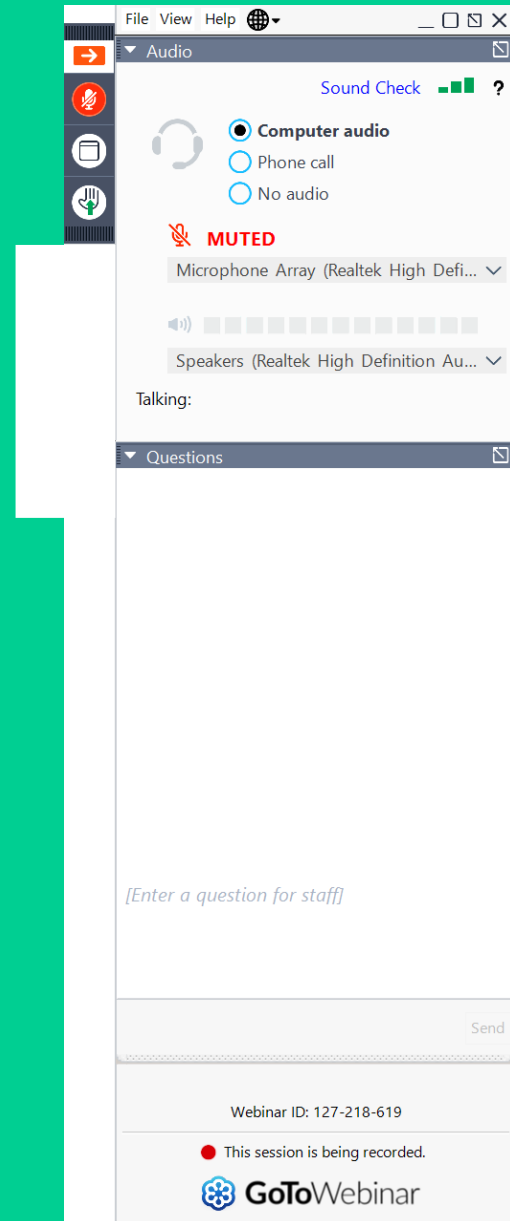
# Ask Questions Live!

## 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!



# Today's Program is Being Recorded

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

[www.myotonic.org/digital-academy](http://www.myotonic.org/digital-academy)

The screenshot shows the Myotonic Dystrophy Foundation Digital Academy website. At the top, there is a navigation bar with the Myotonic Dystrophy Foundation logo, a search bar, and social media icons for Facebook, Instagram, Twitter, LinkedIn, YouTube, and News, along with a 'Donate!' button. Below the navigation bar, the page is titled 'Digital Academy'. There are three video thumbnails: 'Pain and DM2' featuring two men, 'DM Heroes' with the text 'A person living with myotonic dystrophy is a HERO', and 'Heart Health - 2019 Myotonic Annual Conference' featuring a man at a podium. Each thumbnail has a 'Watch Video' button. Below the thumbnails is a search bar labeled 'Search the Digital Academy' and a 'Search' button. Underneath is a 'Video Categories' section with six icons and labels: 'Newly Diagnosed' (stethoscope icon), 'Clinical Trials & Drug Approval' (flask icon), 'Community Voices & Inspiration' (speech bubbles icon), 'Congenital & Childhood Onset' (child icon), 'Genetics & Disease Overview' (DNA helix icon), and 'Disease Management' (person at desk icon).



# Join the Myotonic Dystrophy Advocacy Movement to Fund Research!

Contact: Kevin Brennan

[kbrennan@bluebird-strategies.com](mailto:kbrennan@bluebird-strategies.com)

