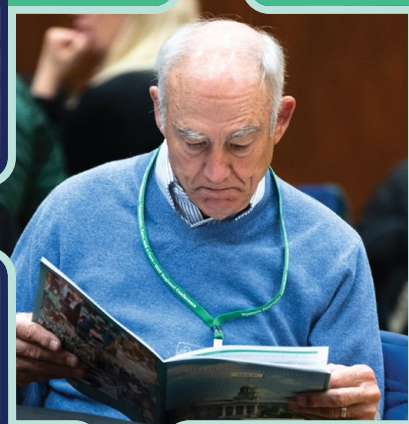


# Report to Our Community 2024



Myotonic  
Dystrophy  
FOUNDATION







# Community, Care *and* a Cure.

## Our Year in Numbers

**\$1 Million+**

Grants &  
Fellowships

**2,900+**

Myotonic Dystrophy  
Family Registry participants

**2,700+**

Letters to  
Congress

**2,500+**

Support Program  
participants

**737**

Regional Conference  
attendees

**11**

New publications  
and translations

## Dear Myotonic Dystrophy Foundation Family,

As we look back on 2024, we are filled with immense pride and hope, reflecting on the remarkable strides we've made toward our shared vision of Community, Care, and a Cure for myotonic dystrophy (DM). This year, thanks to our generous donor community, we reached an incredible milestone with over \$1,000,000 invested in research grants and fellowships, including our first-ever short-term high-priority grants and a new pilot grant program, accelerating critical advancements in the search for a cure. With nine drugs currently in clinical trials, we are closer than ever to the long-awaited approval of the first treatment for DM.

Our advocacy efforts reached new heights, as the DM community sent over 2,700 messages to U.S. Congress through our new Voter Voice platform, demanding greater federal research funding. In support of connecting families to clinical care and research opportunities, we shifted our conference structure to hold six regional conferences across the U.S., engaging over 730 individuals. Our support programming flourished as well, with our multilingual Support Groups and Facilitators offering over 230 meetings, providing a safe space for more than 2,500 participants to share, learn, and grow together.

We developed 11 new topic-based resources to further educate and empower our families, and in July we launched Myotonic Dystrophy In Motion Awareness Month to inspire community members around the world to prioritize exercise and wellness. **Together, we are making incredible progress, and with each step, we move closer to a future where myotonic dystrophy no longer has the power to define lives.**

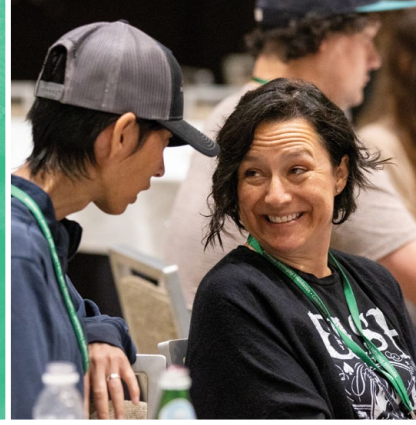
With deepest gratitude,

**Tanya Stevenson, EdD, MPH**  
Chief Executive Officer

**Jeremy Kelly**  
Chair, Board of Directors

# Our Vision

We envision a world with **treatments** and a **cure** for **myotonic dystrophy**.



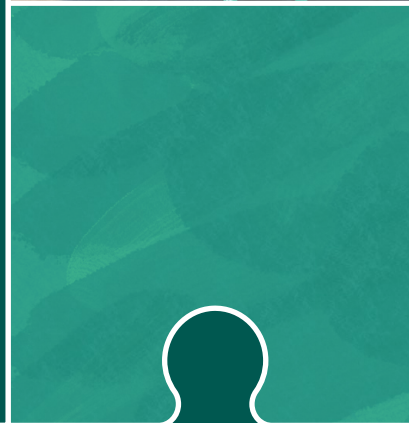
# Our Mission

The mission of the Myotonic Dystrophy Foundation is **Community, Care, and a Cure.**

- We support and connect the myotonic dystrophy **Community.**
- We provide resources and advocate for **Care.**
- We accelerate research toward treatments and a **Cure.**

# Our Values

Community  
Collaboration  
Empathy  
Knowledge  
Hope  
Urgency



Founded in 2007, the Myotonic Dystrophy Foundation (MDF) is the leading global advocacy organization helping patients and families navigate life with myotonic dystrophy (DM). MDF is usually the first resource contacted by newly diagnosed patients, their families, social workers, and clinicians looking for support.



# Community

The Myotonic Dystrophy Foundation supports and connects the myotonic dystrophy community.



## MDF Regional Conferences

For the first time, MDF hosted six Regional Conferences across the United States. These conferences were one-day in-person events to connect local DM communities and provide attendees with the opportunity to connect more easily with local researchers and clinicians. We are grateful to our partners in each location who helped make these conferences possible.

[2024 Regional Conference Tour →](#)



### Gainesville, Florida

MARCH 23RD  
In partnership with Center for NeuroGenetics, University of Florida

**UF** | College of Medicine  
UNIVERSITY of FLORIDA



**182**  
Attendees



## By the Numbers

**737**

Attendees

**24**

Travel scholarships

**346**

First-time attendees

**9**

Industry sponsors

**93**

Sessions

**3**

Lab tours



### Houston, Texas

MAY 4TH  
In partnership with Houston Methodist

HOUSTON  
**Methodist**  
CONTINUING CARE HOSPITAL

**81**

Attendees





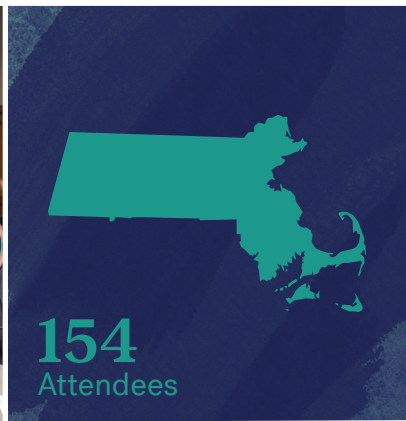
*"Meeting and talking with the researchers, seeing and hearing about the process of their work, and seeing the facility was an incredible experience. It was the most meaningful experience in my life with DM..."*

- SEATTLE, WASHINGTON LAB TOUR ATTENDEE



Learn more about our past conferences and watch highlight reels from each conference

[www.myotonic.org/our-annual-conferences](http://www.myotonic.org/our-annual-conferences)



Iowa City,  
Iowa

APRIL 20TH  
In partnership with  
University of Iowa



106  
Attendees



Boston,  
Massachusetts

APRIL 6TH  
In partnership with Massachusetts General Hospital



104  
Attendees



110  
Attendees



Seattle,  
Washington

JUNE 1ST  
In partnership with  
Seattle Wellstone  
Muscular Dystrophy  
Specialized Research  
Center and University  
of Washington



Los Angeles,  
California

MAY 18TH  
In partnership with UCLA Health





# Support Programs

MDF's support programs, led by trained community volunteers, create safe and welcoming spaces to build community, learn, and share.

## NEW SUPPORT GROUPS

### → Affected Men's Support Group & Affected Women's Support Group

*Both of these groups launched in summer 2024 with a record number of registrations — over 70 community members each!*

### → Groupe International de Soutien des Francophones & Maryland Support Group

*Our first French-language Support Group and our Maryland Support Group formed in 2024, with their first meetings in January 2025.*



*"I want to be there for people who have [DM] or who have kids with this disease. When I first was diagnosed there were a lot of people who helped my family through the process and I want to do that for others."*

— BARBARA OCHOA,  
SUPPORT GROUP FACILITATOR

## By the Numbers

2,095

Support Group participants

518

DM Virtual Happy Hour participants

238

Support Group meetings

51

Support Group Facilitators

29

Support Groups

4

New Support Groups founded

4

Support Group Languages\*

\*German, French, Spanish, English



For more information  
about Support Groups:

[www.myotonic.org/find-support](http://www.myotonic.org/find-support)



Meet our Support Group  
Facilitators at:

[www.myotonic.org/sgfs](http://www.myotonic.org/sgfs)



# Support Groups and Facilitators



## GEOGRAPHY-BASED

### Atlanta, GA

Chuck Hunt

### Canada

Alex LeBoeuf and Julie LeBoeuf

### Chicago, IL

Rob Besecker and Ryan Vogels

### Finger Lakes and Upstate New York

Emily Jones and Lois Schenk

### Florida

Beth Feigenblatt and Kristen McClintock

### Kansas City

John Cooley and Pat Gibson

### Maryland

Caroline Easterling and Julian Easterling

### Michigan

Suzanne Perkins and Scott Virgo

### Mountain West Region

Kay Hayes and Kathie Thorsland

### New England

Jeannine DeSoi and Bill Nuttall

### New York City/New Jersey

Janis Jaffe, Glenda Winson, and Guillermo Zubillaga

### North & South Carolina

Mindy Kim

### Ohio

Nathan Beucler and Carolyn Valek

### Portland, OR

Mark Coplin

### Southern California

Rose Albanese and Barbara Ochoa

### Texas

Peggy M. and Lynn S.

### Virginia

Jodie Howell and Samantha Welsh

### Washington State

Jonathan Freedman and Cindy Hubert

## TOPIC-BASED

### Affected Men's Support Group

Jim Dolan, Shaun Moore, and Ryan Vogels

### Affected Women's Support Group

Jeannine DeSoi and Haley Martinelli

### DM2 Virtual Support Group

Haley Martinelli and Tom McPeck

### DM2 Caregivers Virtual Support Group

Kim McPeck

### Caregiver Virtual Support Group

Annette Rnjak and Ted Salwin

### Caregivers of Children with CDM

Sarah Berman

### Telefonische Gesprächsgruppe DM1 / DM2

Anke Klein and Bernhard Rogg

### Groupe International de Soutien des Francophones

Sarah Berman, Julie LeBoeuf, and Marie-Claude Sauvé

### Grupo de Soporte Virtual en Español de MDF

David Kugler and Araceli Mera

### Juvenile-onset Adult (JOA) Warriors

Carolyn Valek and Ann Woodbury

### Juvenile-onset Adult (JOA) Caregivers

Kyle Dunson, Peggy Melton, and Ann Woodbury

### Adult Facebook Chat

Mindy Kim and Bill Nuttall

### DM Virtual Happy Hour

Mindy Kim and Kristen McClintock





# DM In Motion

Myotonic Dystrophy In Motion (MDIM) Awareness Month is designed to provide community members with opportunities for physical activity, education, and community connection.

- MDF hosted its first MDIM Awareness Month in July 2024.
- Throughout the month, MDF hosted four webinars designed for all activity and comfort levels, which followed weekly themes.
- Events engaged community members in educational and inspirational sessions about the importance of exercise, ways to exercise safely at home, and how to connect with nature and ourselves while moving.
- Our In Motion Buddy System offered a chance for participants to connect and collaborate 1:1 with others in the DM community about their experiences and ideas on exercise and movement.
- MDF created the Exercising with Myotonic Dystrophy Infographic that outlines ideas for finding motivation, monitoring exercise, and adding movement to daily life.

*“I was so elated!... We are motivated and will stay on our feet and stay in touch long after MDIM Awareness Month has ended.”*

— MDIM BUDDY PARTICIPANT ON THEIR BUDDY ASSIGNMENT



Download our Exercising with DM Infographic

[www.myotonic.org/exercise-info](http://www.myotonic.org/exercise-info)



Learn about MDIM Awareness Month and view the weekly webinars:

[www.myotonic.org/in-motion](http://www.myotonic.org/in-motion)

## WEBINAR SERIES

### **Week 1: Come As You Are!** ***Stump the Doctor & Community Panel***

Burning questions and insightful discussions about exercise and mobility with a knowledgeable panel.

### **Week 2: Little Things Count** ***Exercises for Everyday Life***

The importance of a balanced movement practice and simple movements that can enhance your overall well-being.

### **Week 3: The Natural World** ***The Benefits of Nature & Breathwork***

An exploration of the connection between nature, wellness, and breathwork. The transformative power of mindfulness and breathwork practices to enhance daily movement routines.

### **Week 4: Let's Keep It Going!** ***Virtual Zumba Class***

An exhilarating Zumba class designed to celebrate the achievements of Myotonic Dystrophy In Motion Awareness Month.

## By the Numbers

**430+**

Recording views

**90**

Webinar participants

**130**

Community participants

**32**

U.S. States represented

**83%**

Survey respondents found the sessions valuable

**14**

Countries represented



# JOA Camp

MDF hosts a free camp experience exclusively for JOAs, adults living with juvenile onset myotonic dystrophy.

- MDF was thrilled to again host our JOA Camp in August 2024!
- Nine campers joined us for the 5-day, 4-night quintessential summer experience.
- JOA Camp is held at Easterseals' Timber Pointe Outdoor Center, a 170-acre facility located on Lake Bloomington in Hudson, Illinois.



*“This was one of the best summers of my life. I made many new friends and memories and connected with old friends. I look forward to going back next summer!”*

— CHRISTINE BADE, JOA CAMPER

## CAMP ACTIVITIES

- Canoe, kayak, and paddleboard
- Zipline and giant swing
- Archery practice
- Pontoon boat ride
- Marshmallow roasting
- Singing and dancing
- Feeding goats
- And more!



Learn more about our JOA Camp:

[www.myotonic.org/camp-joa](http://www.myotonic.org/camp-joa)



# Care

The Myotonic Dystrophy Foundation provides resources and advocates for care.

For those who are diagnosed with DM, finding access to appropriate care from clinicians and healthcare professionals can be particularly challenging. Medical professionals are often unfamiliar with the disease because they see cases so infrequently, and the complicated and variable nature of the disease makes supporting patients and accessing clear treatment guidelines more difficult.



## WARMLINE

The MDF Warmline connects patients—many newly diagnosed—and their family members with resources, support, and education.

Need Support? Contact us at  
+1 (415) 800-7777 or [info@myotonic.org](mailto:info@myotonic.org)

## By the Numbers

**33,000+**

Resource downloads  
from our website

**500+**

Warmline calls and  
email interactions

**30**

Videos added to the  
MDF Digital Academy

**8**

Languages of  
MDF resources

**11**

New publications  
and translations

*[The Ask-the-DM-Expert webinar provided] a very clear and complete presentation, explanations, and PowerPoint slides. Clear answers to commonly raised questions, provided by a recognized and experienced professional. This was a great presentation and webinar!"*

— GENETIC TESTING & COUNSELING FOR DM WEBINAR ATTENDEE



¡Buenos días!  
Visit our Spanish language hub:  
[www.myotonic.org/espanol](http://www.myotonic.org/espanol)



Guten Tag!  
Visit our German language hub:  
[www.myotonic.org/deutsch](http://www.myotonic.org/deutsch)

# Empowering the DM Community through Education

Access a wealth of materials and resources for individuals, families, and clinicians on the MDF website.



[www.myotonic.org/toolkits-publications](http://www.myotonic.org/toolkits-publications)

## FIND-A-DOCTOR MAP

This community-driven map helps affected individuals, families, and caregivers connect with medical professionals who have experience working with DM patients. Visit the map to help find or contribute information about medical professionals in your area.  
[www.myotonic.org/find-a-doctor-map](http://www.myotonic.org/find-a-doctor-map)

## MDF DIGITAL ACADEMY

The largest DM-focused digital library in the world houses more than 200 hours of educational and inspirational videos by DM experts.  
[www.myotonic.org/digital-academy](http://www.myotonic.org/digital-academy)

## ASK-THE-DM-EXPERT

This virtual webinar series features DM clinicians and experts in diverse specialties who give recorded presentations and participate in live Q&A sessions with webinar attendees.  
[www.myotonic.org/ask-expert-series](http://www.myotonic.org/ask-expert-series)

### 2024 Ask-the-DM-Expert Webinars

- DM & the Heart
- Genetic Testing & Counseling for DM
- GI Issues & DM
- Reproductive Health & DM

## TOOLKITS & PUBLICATIONS

MDF resources help guide health care providers and families in the care and management of DM.

### New Publications

- Exercising with Myotonic Dystrophy Infographic (English)
- MDF Medical Alert Card (French-Canadian)
- Myotonic Dystrophy and Mental Health Handbook (English and Spanish)
- Myotonic Dystrophy and the Heart: A Community Guide (English and Spanish)
- Practical Advice for Anesthesia for Individuals with Myotonic Dystrophy and Their Families (English)
- Nutrition Guide (Spanish)
- Role of Physical Therapy (Spanish)

### New English Translations

MDF facilitated translation of two seminal works about myotonic dystrophy from German into English, both originally published more than a century ago.

- Dr. Hans Steinert's initial descriptions of patients with myotonic dystrophy (1908)
- Dr. Bruno Fleischer's "On Myotonic Dystrophy with Cataracts: A Hereditary, Familial Degeneration" (1918)



## Understanding DM

MDF is excited to introduce *Understanding Myotonic Dystrophy*, a series of short educational animations designed to help individuals living with DM and their families better understand the condition.

### Watch & Learn



[www.myotonic.org/understanding-DM](http://www.myotonic.org/understanding-DM)



# Cure

The Myotonic Dystrophy Foundation accelerates research toward treatments and a cure.



## Advancements in DM Research

Since its founding in 2007, MDF has helped dramatically change the DM research and drug development landscape.

MDF plays a key role in DM research and drug development by investing in research, researchers, and initiatives to attract more industry, foundation, and government funding. We help researchers, drug companies, and regulators to understand the needs of people living with DM. We also connect individuals with DM to information about research studies and trials.

[www.myotonicregistry.org](http://www.myotonicregistry.org)



### Myotonic Dystrophy Family Registry (MDFR)

#### Your Participation Makes a Difference!

Sharing information with the MDFR, through surveys and medical reports, can play a critical role in accelerating progress towards finding effective treatments and a cure.

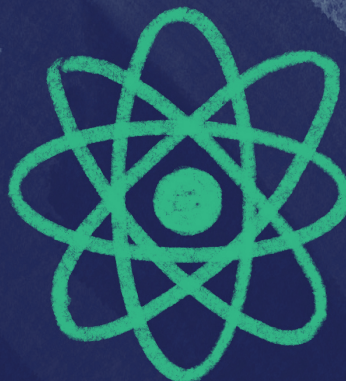
#### Login now:

[www.myotonicregistry.org](http://www.myotonicregistry.org)

#### Questions?

Call us at +1 415-800-7777

# Supporting DM Clinical Studies & Trials



## Myotonic Dystrophy Clinical Research Network (DMCRN)

MDF expands clinical study and trial infrastructure by contributing funding and support to the DMCRN, a network of more than 20 medical centers in the U.S., Europe, Japan, and New Zealand.

[www.myotonic.org/dmcrn](http://www.myotonic.org/dmcrn)

## Study and Trial Resource Center

This resource provides details on studies and trials, including the clinical trial process, participant guidelines, and a list of current clinical studies and trials.

[www.myotonic.org/study-trial-resource-center](http://www.myotonic.org/study-trial-resource-center)

## By the Numbers

**2,900+**

Participants in the Myotonic Dystrophy Family Registry

**25+**

DM drugs in clinical pipeline

**9**

DM drugs in Phase I/II/III clinical trials

**7**

Meet the DM Drug Developers webinars

**6**

Industry update sessions at Regional Conferences

## CONNECTING THE COMMUNITY WITH DM DRUG DEVELOPERS

MDF's "Meet the DM Drug Developers" webinar series, hosted since 2021, connects biotech and pharmaceutical partners with our community to share updates and to answer questions in a live format. The series has had more than 17,000 views since its launch!

In 2024, we featured the following partners in the series:



Learn about different DM drugs in development:

[www.myotonic.org/pipeline](http://www.myotonic.org/pipeline)



Watch or participate in Meet the DM Drug Developers webinars:

[www.myotonic.org/meet-dm-drug-developers](http://www.myotonic.org/meet-dm-drug-developers)



# MDF Research Investments

MDF is investing in the next generation of DM researchers and thought leaders.

## By the Numbers

**\$1.7 Million**

Invested in Research Programs  
in 2024

**\$380,000**

for 2 Early Career  
Scholar Grants

**\$220,000**

for 4 Doctoral  
Research Fellowships

**\$200,000**

for 4 Pilot Grants

**\$100,000**

For 2 Short-Term  
High-Priority Grants

**56**

Number of Research Fellows  
Funded since 2009

**\$7 Million+**

MDF investment in Research  
Fellowships & Grants since 2009

### Research Fellows Program

Created in 2009, our flagship program provides two-year pre- and postdoctoral research fellowships to support new and innovative studies relevant to myotonic dystrophy.

### Early Career Scholars Program

This program was launched in 2023 to help retain early career scholars who are passionate about research in the DM field. MDF prioritizes funding clinical researchers and physician-scientists with these two-year grant awards.

### Pilot Grant Program

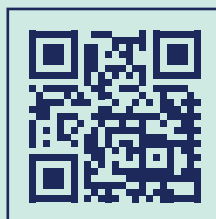
This new program supports innovative DM research to gather preliminary data on novel ideas.

### Short-Term High-Priority Grants

These grants are awarded to researchers for one-year projects focused on high-priority topics, as identified by MDF's Board of Directors.

### Small Grants Program

Created in response to a need to expand access and boost research efforts in DM, this program provides grants for research journal open access fees (up to \$5,000) and scientific conference presentation and travel expenses (up to \$2,500).



Learn more about MDF's  
research funding program:

[www.myotonic.org/grants](http://www.myotonic.org/grants)







## 2024 Early Career Scholars

**\$380,000 INVESTMENT**

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### **Johanna Hamel, MD**

*Assistant Professor | University of Rochester, New York, U.S.*

Dr. Johanna Hamel's study, "Remote Assessments in Myotonic Dystrophy," explores how the length of the CTG repeat mutation influences the timing and severity of DM1 symptoms. The research uses video conferencing and toolkits to reach diverse participants, aiming to uncover additional genetic factors and improve patient registries for future clinical trials.



### **Tahereh Kamali, PhD**

*Postdoctoral Research Fellow | Stanford University School of Medicine, California, U.S.*

Dr. Tahereh Kamali's study, "Utilizing Generative AI to Expand Clinical Data for DM Studies and Treatment Efficacy Planning," seeks to enhance an AI model she developed that identifies changes in the central nervous system related to DM. By generating synthetic data and using real-world patient data, the project aims to improve diagnosis accuracy and advance personalized treatments for DM patients.

## 2024 Research Fellows

**\$220,000 INVESTMENT**

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### **Betty Bekele**

*Emory University, Atlanta, Georgia, U.S.*

Betty Bekele's study, "Altered Inhibitory Neurotransmission in Mouse Models of Myotonic Dystrophy Type 1," explores how DM1 affects GABA, a brain chemical crucial for sleep and anesthesia sedation. By studying changes in GABAA receptor subunits, the research aims to understand DM1's impact on the brain and test therapies, including flumazenil, to improve sleep and anesthesia-related complications in DM1 patients.



### **Sakura Hamazaki**

*University of Rochester, New York, U.S.*

Sakura Hamazaki's study, "Impact of Calcium Entry through Cav1.1 in Myotonic Dystrophy Myopathy," investigates how altered ion channels, particularly Cav1.1, contribute to muscle weakness in DM1. Using mouse models, the study aims to correct the function of these channels and validate them as therapeutic targets, with potential implications for developing or repurposing treatments for DM1.



### **Alexandra L. Marrero Quinones**

*Virginia Commonwealth University, Richmond, Virginia, U.S.*

Alexandra L. Marrero Quinones' study, "Evaluation of MSH3 as a Genetic Modifier of Trinucleotide Repeat Instability in Myotonic Dystrophy," investigates the role of DNA repair genes, particularly MSH3, in the rapid expansion of CTG repeats in congenital DM1. By analyzing blood samples from families with rapid repeat expansion, the study aims to uncover how MSH3 variants contribute to repeat instability, offering insights into disease progression and potential new therapeutic targets for DM1.



### **Cameron Niazi**

*University of Florida, Gainesville, Florida, U.S.*

Cameron Niazi's project, "Leveraging CRISPR/Cas-based Epigenetic Modifications for the Treatment of Myotonic Dystrophy Type 1," explores a novel CRISPR/Cas approach to silence the faulty DM1 gene without cutting the DNA, reducing the risks of off-target effects. By turning off the gene responsible for producing toxic RNA, this project aims to provide a safer alternative to traditional gene editing, offering a promising new therapeutic strategy for DM1.

# 2024 Pilot Grant Recipients

**\$200,000 INVESTMENT**

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**Joel R. Chamberlain, PhD**

*Research Associate Professor | University of Washington, Seattle, Washington, U.S.*

Dr. Joel Chamberlain is exploring a novel approach to treat DM1 by using natural cell-derived vesicles to deliver drugs that can destroy toxic RNA structures in muscles in her project, "Efficacy Testing of Cell-Derived Nanovesicle Delivery of Small Interfering RNAs for Treatment of DM1." If successful, this could lead to a new, non-invasive treatment that targets the root cause of DM1.



**Paloma Gonzalez Perez, MD, PhD**

*Neuromuscular Attending | Massachusetts General Hospital, Boston, Massachusetts, U.S.*

In her project, "Investigating Benefits of a Physical Therapist-Guided Exercise Program in Myotonic Dystrophy Type 2," Dr. Paloma Gonzalez Perez is testing the effectiveness of a simple, guided exercise program designed to improve motor function and reduce pain in DM2 patients. Her study will examine the long-term benefits of exercise under physical therapist supervision, with the goal of making this program accessible to more DM2 patients.



**Emma Matthews, FRCP**

*Reader of Neurology and Consultant Neurologist | St George's, University of London, U.K.*

Dr. Emma Matthews aims to uncover why DM1 patients are more prone to abnormal lipid profiles (dyslipidemia) in her project, "Exploring Transcriptional Dysregulation of Lipid Metabolism Genes in DM1." By comparing lipid metabolism genes in DM1 patients with and without dyslipidemia, her study could lead to better treatment guidelines and new therapies for managing this condition.



**Belinda Pinto, PhD**

*Research Assistant Scientist | University of Florida, Gainesville, Florida, U.S.*

In her project, "Investigating the Contribution of Circadian Disruption to Hypersomnolence in Myotonic Dystrophy," Dr. Belinda Pinto is studying how disruptions in the circadian system contribute to excessive daytime sleepiness in DM1 patients. Using animal models, she seeks to understand the molecular causes of hypersomnia and pave the way for future therapeutic developments.

## Short-Term High-Priority Project Awardees

**\$100,000 INVESTMENT**

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**Kristina Kelly, DPT**

*Assistant Research Professor | University of Missouri-Columbia, Missouri, U.S.*

Dr. Kristina Kelly's study, "Neural Mechanisms of Motor Fatigability in Myotonic Dystrophy Type 1," explores how the nervous system contributes to motor fatigability in individuals with DM1. By comparing nervous system activity before and after exercise between DM1 patients and healthy controls, the study aims to uncover the biology behind fatigue and develop better strategies for managing it.



**Lukasz Sznajder, PhD, MSc**

*Assistant Professor | University of Nevada, Las Vegas, U.S.*

Dr. Lukasz J. Sznajder's study, "Delineating Pathogenic RNA Species in Myotonic Dystrophy Type 2," aims to validate the hypothesis that mRNA with retained CCUG repeats plays a central role in DM2's molecular changes. By using DM2-derived cell lines, tissues, and advanced bioinformatics, the study seeks to identify key pathogenic molecules and develop potential preventative therapies, ultimately advancing understanding and treatment of DM2.



# Advocacy

The Myotonic Dystrophy Foundation advocates for improved care and a cure.

## By the Numbers

2,754

Advocacy campaign  
messages to Congress

1,159

New advocates participated  
in grassroots campaigns

16

Senators and Representatives  
signed letter of support for  
CDMRP funding of DM research

## Advocacy Initiatives

MDF drives key initiatives for improved care and accelerated research while raising DM visibility with stakeholders in U.S. Congress and other policymakers, federal and state agencies, regulatory agencies, medical professionals, and the media.

### MDF Empowers Grassroots Advocates

MDF empowered thousands of our community members to engage in grassroots advocacy through a new platform called Voter Voice. Community members who visit our custom webpage can email preloaded advocacy messages to their Senators and Representatives. In 2024, MDF launched three advocacy campaigns with Voter Voice, generating 2,754 messages to Congress.

### Congressionally Directed Medical Research Program (CDMRP) Advocacy

Our 2024 advocacy campaigns urged Congress to increase federal funding for myotonic dystrophy, specifically requesting \$10 million for DM research as part of the Fiscal Year 2025 Congressionally Directed Medical Research Program (CDMRP). Thanks to the support of our grassroots advocates, 16 U.S. Senators and Representatives signed on to letters of support for CDMRP funding. Although Congress did not include DM research in their 2025 Fiscal Year budget, we are proud of the work that we accomplished together and will try again!

### State Captain Recruitment

In December 2024, MDF launched a recruitment campaign to have U.S. State Advocacy Captains in all 50 states. State Advocacy Captains will 1) coordinate meetings with their state congressional delegations several times a year in support of MDF's research advocacy objectives; and 2) recruit and mobilize grassroots advocates in their state to build awareness and secure more federal funding for DM research.





## 2024 MDF LEGISLATIVE ADVOCACY AWARDS

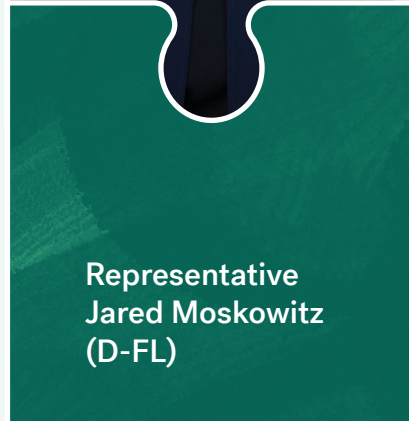
Representative Jen Kiggans (R-VA)

Representative Jared Moskowitz (D-FL)

Honored for leading the first ever congressional letter to the House Appropriations Committee in support of MDF's request for \$10 million in DM research as part of the Congressionally Directed Medical Research Program (CDMRP). It was signed by 11 members of the U.S. House of Representatives and 5 members of the U.S. Senate!



Representative  
Jen Kiggans  
(R-VA)



Representative  
Jared Moskowitz  
(D-FL)



## PEER REVIEW MEDICAL RESEARCH PROGRAM (PRMRP)

MDF Congressional advocacy secured continued federal research funding eligibility for DM from the Department of Defense's Peer Reviewed Medical Research Program (PRMRP) for the eighth year in a row.

Over the past seven years, PRMRP has allocated over \$23 million to DM research, significantly enhancing our understanding of both DM1 and DM2. PRMRP funding has been instrumental in advancing breakthrough research and therapy development. PRMRP-funded studies have uncovered key insights into DM, leading to promising treatment strategies. Funding has supported research on gene editing and molecular therapies, giving hope to the DM community.



## NATIONAL ADVOCACY COMMITTEE (NAC)

MDF's NAC was founded in 2022 to lead new advocacy campaigns and motivate the DM community to advocate for increased federal research funding. We are grateful to our NAC members for their advocacy leadership!

Rob Besecker  
Illinois

David Brand\*  
Virginia

Martha Montag Brown  
California

Rebecca Coplin  
Oregon

Belen Esparis  
Pennsylvania

Lisa Harvey-Duren\*  
West Virginia

Charles Hunt  
Georgia

Emily Jones  
New York

Eric Wang  
Florida

Leo Zabezhinsky\*  
Minnesota

*\*New in 2025*



Let your voice be heard!

Learn more about MDF's  
advocacy initiatives  
and take action:

[www.myotonic.org/advocate](http://www.myotonic.org/advocate)



# International DM Awareness

The Global Alliance for Myotonic Dystrophy Awareness celebrates International Myotonic Dystrophy Awareness Day on **September 15th**.



- MDF is proud to be a founding member of the Global Alliance for Myotonic Dystrophy Awareness. Established in 2021, the Global Alliance now includes over **60** international nonprofit organizations, academic and research institutions, biotechnology and pharmaceutical companies, patient advocacy groups, and others working together to raise myotonic dystrophy awareness.
- Global Alliance members directed their energies toward two primary areas of focus throughout the year: 1) Awareness and education among clinical care teams, and 2) Clinical trial readiness for participants.
- Partners mobilized to illuminate the DM community by lighting monuments and landmarks green all over the world on **September 15th**. This initiative increased DM visibility across communities and social media.



## Get Involved!

- Light up your local landmarks, monuments, and important buildings in green
- Share the International Myotonic Dystrophy Awareness Day logo
- Social Media Campaign: During the month of September, post DM facts and your experiences on social media to help people understand more about the disease
- Window Sign Campaign: Ask neighbors and shops in your community to display an International Myotonic Dystrophy Awareness Day window sign



How will you celebrate on  
**September 15th?**

For tips, suggestions, and resources:  
[www.myotonic.org/international-dm-day](http://www.myotonic.org/international-dm-day)

# The Global Alliance for Myotonic Dystrophy Awareness





# Fundraising

Our donors make it possible for the Myotonic Dystrophy Foundation to advance our mission of Community, Care, and a Cure.

## Changing the Future of Myotonic Dystrophy

MDF's Change the Future of Myotonic Dystrophy end-of-year fundraising campaign featured a series of four new impactful videos, profiling families affected by DM. We are grateful to all of the individuals who participated in the interviews, with special thanks to Luke Desforges, Ben Reynolds, and Kim Reynolds for sharing your experience living with DM.



Check out our Change the Future of Myotonic Dystrophy video series to learn more about the impact of your support!

[www.myotonic.org/impact](http://www.myotonic.org/impact)

## \$ Raised in 2024

### \$3.2 Million

Total Contributions

### \$1.6 Million

Gala

### \$380,000

Corporate Sponsorships

### \$70,000

Fundraising campaigns hosted by community members





# DIY Fundraising

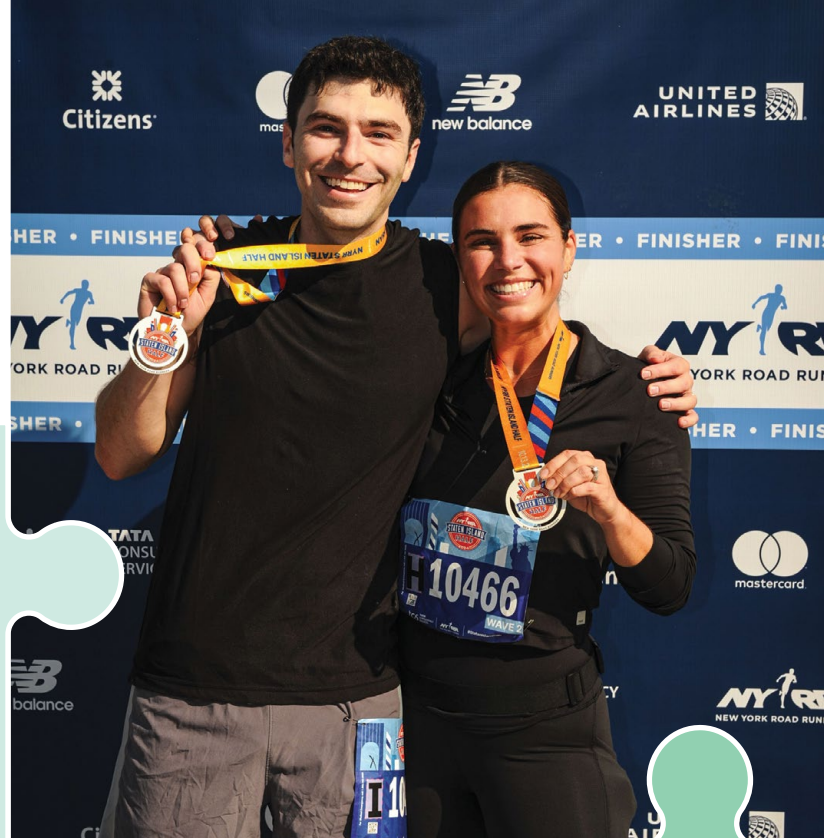
Community members raised awareness about DM, and funds for MDF, through a total of 130 different fundraising campaigns, ranging from endurance events and golf tournaments to in-person donation drives and social media fundraisers.

## Casey & Max's Half-Marathon Challenge

Running a half-marathon had been on Casey and Max Segal's bucket list for years. In spring 2024, they decided to make their dream a reality by signing up for a race on Staten Island, scheduled six months later, and began their training together. With no experience running more than two miles at a time, the challenge ahead was daunting.

When Casey's father passed away unexpectedly in May, their grief fueled their determination to reach their goal. Casey's father had lived with DM, which her 26-year-old brother also inherited. To give their challenge even more significance, Casey and Max turned their half-marathon into a fundraiser for MDF, honoring both her father and brother.

Not only did they complete their first half-marathon, they also raised nearly \$20,000 for MDF. Thank you, Casey and Max, and all of your generous supporters!



*"I am so hopeful for the future and what MDF can accomplish with the right funds and team supporting their mission."*

— CASEY SEGAL, DONOR & DIY FUNDRAISER



Andrew Gulch Memorial Golf Outing  
[www.mytonic.org/agm](http://www.mytonic.org/agm)



## New Fundraising Platform and Toolkit

MDF created a new DIY fundraising platform and toolkit to empower our community members to create their own unique fundraisers and manage their campaign webpages.



Interested in fundraising for MDF?  
Learn more here:

[www.mytonic.org/diy](http://www.mytonic.org/diy)



# 2024 MDF Gala

Our annual Gala raised a record-setting \$1.6 Million!



## GALA HIGHLIGHTS

- Our 10th annual fundraising Gala was held on September 14th at the Taglyan Complex in Hollywood, California.
- Critical funds raised at the event improve quality of life for individuals and families living with DM and accelerate research towards treatments and a cure.
- We are beyond grateful to our event attendees, sponsors, donors, and volunteers for making the Gala so incredibly successful!
- Thank you to all the families and research partners who shared their stories for our four Gala videos. These compelling videos helped guests better understand the lived experience of DM, offered hope for the future, and inspired people to give generously in support of our mission.



## 2024 Gala Committee

Thank you to our 2024 Gala Committee, who organized and executed this successful fundraising event!

**Peter Desforges**

**Suzanne Desforges**

**Elizabeth Florence**

**Whitney Gates**

**Alex Kapp**

**Leslie Lynch**

**Laurie Rodli**

**Benjamin Youngblood**

**Martha Montag Brown**  
Co-Chair

**Erica Kelly**  
Co-Chair



Join us for the 2025 Gala  
on September 18th in NYC!

[www.myotonic.org/gala](http://www.myotonic.org/gala)



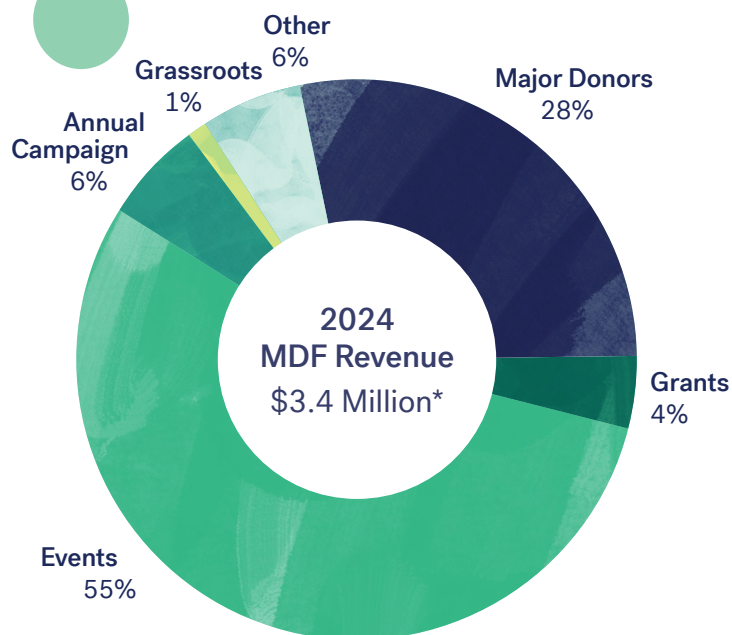




# MDF Financials

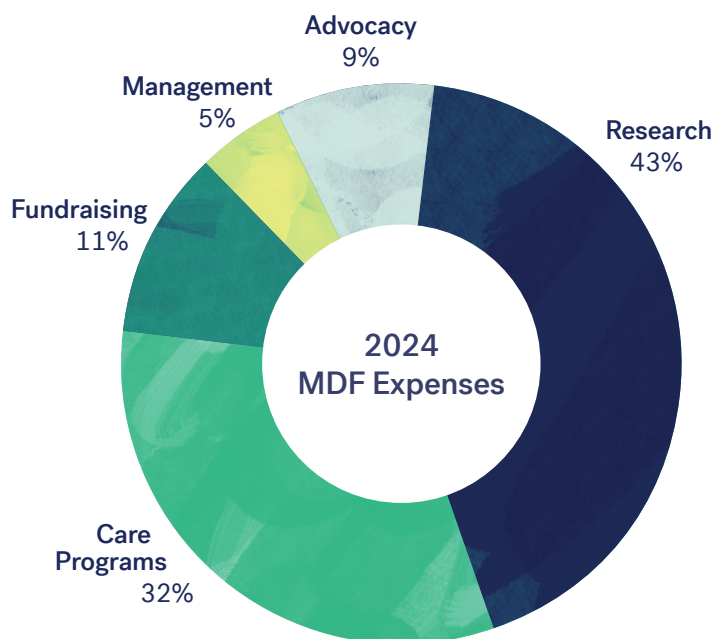
## \$35 Million+

Amount raised by MDF since 2007 to support its mission of Community, Care, and a Cure



~80% of donations are from individual donors.

\*Based on unaudited financial statements



84% to Research, Care, and Advocacy programs in 2024.

### Mail-in Donation

Please make checks payable to

**Myotonic Dystrophy Foundation**  
663 13th Street, Suite 100  
Oakland, CA 94612

### Online Donation

One-time and recurring gifts

### More Ways to Give

- » Stock
- » Donor Advised Funds
- » IRA Qualified Charitable Contributions
- » Workplace & Corporate Matching Gift Programs

### QUESTIONS?

Call us at  
+1 415-800-7777

or email  
[development@myotonic.org](mailto:development@myotonic.org)

↑  
Invest  
in Our  
Mission →



Donate Online!

[www.myotonic.org/donate](http://www.myotonic.org/donate)



# Myotonic Dystrophy at a Glance



Myotonic dystrophy is a rare, multi-systemic, inherited disease that may affect as many as **1 in 2,100 people**, or over 3 million individuals across the world.



**Myotonic dystrophy is inherited** — people living with myotonic dystrophy have a 50% chance of passing on the mutated gene to their children.



Millions of people are living with DM globally, yet **millions of people do not know they have the disease** and are in need of care.



**People living with myotonic dystrophy experience varied and complex symptoms**, from skeletal muscle problems, to heart, breathing, digestive, hormonal, speech and swallowing, diabetic, immune, excessive daytime sleepiness, early cataracts and vision challenges, and cognitive difficulties.



Myotonic dystrophy is **the most common form of adult muscular dystrophy** and considered the most variable of all known conditions.



**Myotonic dystrophy does not always look the same.** The different body systems affected, the severity of symptoms, and the age of onset of those symptoms vary greatly between individuals, even in the same family.



**Myotonic dystrophy is commonly referred to as DM**, an abbreviation of the Latin name used by doctors and researchers worldwide: dystrophia myotonica. Other names for DM include myotonic muscular dystrophy (MMD), Steinert's Disease for DM1, and proximal myotonic myopathy (PROMM) for DM2.



**Delays in diagnosing myotonic dystrophy are common.** Despite the availability of simple genetic tests, a lack of familiarity with the disease on the part of healthcare providers can allow misdiagnoses to persist for decades.



Myotonic dystrophy symptoms usually become more severe with each generation, yet there **is currently no cure and there are no approved treatments**.



Over **40 biopharmaceutical companies are leading promising research** which may result in new treatments for myotonic dystrophy, and, one day, a cure.



**Mutations prevent genes from carrying out their functions properly**, which can impact multiple body systems. Myotonic dystrophy type 1 is caused by a mutation in the DMPK gene, while myotonic dystrophy type 2 is caused by a mutation in the CNBP gene.



Learn more and find citations at:  
[www.myotonic.org/at-a-glance](http://www.myotonic.org/at-a-glance)



Read about MDF's impact at:  
[www.myotonic.org/impact](http://www.myotonic.org/impact)





663 13th Street, Suite 100  
Oakland, CA 94612

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**Richard Moxley III, MD**  
*(Emeritus Member)*

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