



Myotonic Dystrophy FOUNDATION



Report *to our* Community 2025







Community, Care, *and a* Cure.

Our Mission

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

Our Vision

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

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.

Dear Myotonic Dystrophy Foundation Family,

What a year 2025 has been—filled with record-breaking milestones, deepening connections, and significant progress toward our mission of Community, Care, and a Cure!

The MDF Conference in Indianapolis was our largest gathering to date, welcoming more than 630 attendees from 34 U.S. states and 10 countries. In July, our second annual Myotonic Dystrophy In Motion Awareness Month inspired hundreds of community members around the world to prioritize movement and wellness, and our JOA Camp in August brought together adults living with juvenile-onset myotonic dystrophy (DM) for five unforgettable days of friendship and connection. Resources from our online library were downloaded over 100,000 times as we added new publications and translations to better serve our community in multiple languages.

On the research front, we are now closer than ever to a treatment for DM. With 11 drugs now in Phase I, II, or III clinical trials, and more than 25 in the broader pipeline, the momentum is extraordinary. We continued to work closely with clinical research coordinators this year to recruit participants and share trial and study details with DM families—a sincere thank you to everyone who has signed up to participate in this critical research. To further advance DM science and move us closer to a cure, MDF invested more than $1.5 million in research programs and grants in 2025, and our advocacy efforts garnered continued US Congressional support and secured Department of Defense research funding eligibility for the 9th consecutive year.

Together, we are making remarkable progress, and with each step, we move closer to a future where myotonic dystrophy no longer defines the lives of those we love.

Tanya Stevenson, EdD, MPH
Chief Executive Officer

Jeremy Kelly
Chair, Board of Directors

Our Year in Numbers

$1.5 Million+
MDF Research Programs & Grants

2,900+
Myotonic Dystrophy Family Registry participants

5,298
Letters to U.S. Congress

2,394
Support Group participants

800+
Warmline interactions

638
MDF Conference attendees

$4.3 Million
Revenue raised by MDF in 2025

Community

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

2025 MDF Conference

May 1 - 4, 2025 | Indianapolis, Indiana

The 2025 MDF Conference was the Foundation's largest gathering to date, with more than 600 attendees from 34 U.S. states and 10 countries. With 72 sessions spanning clinical care, research, advocacy, and wellness, the conference offered meaningful learning and connection opportunities for people living with DM, caregivers, clinicians, and researchers alike. Four outstanding community members were honored with annual awards recognizing their exceptional service and advocacy.

Evening events—including a Thursday welcome reception, a Friday murder mystery masquerade dinner, and a Saturday dinner and dance party—fostered the informal bonds that make this gathering so special for our community.

New in 2025, Sunday Morning Workshops offered attendees one-on-one sessions with experts on topics including genetic counseling, family planning, adaptive devices, insurance navigation, and caregiver resources.

638
Total attendees (in-person and virtual)

281
In-person community members

252
In-person professionals

92
Speakers

20
Exhibitors

17
Years of MDF Conferences

EVALUATION RESULTS HIGHLIGHT CONFERENCE IMPACT

Of the 132 attendees who responded to our post-event survey:

100 % of community members and professionals would recommend the MDF conference

100 % of people with DM1 felt more empowered to make healthcare decisions

100 % of people with DM2 felt more informed about myotonic dystrophy

100 % of caregivers felt they could trust others at the conference

95 % of first-time attendees felt like they were part of a larger community

2025 MDF ABOVE AND BEYOND AWARD

Midori Senoo

Honored for her decade of leadership and service to the DM community in Japan. In 2015, Midori co-founded the DM Patients' Group of Japan (DM-Family) to connect hundreds of individuals with DM experts, essential resources, clinical trials, and hope. Midori continues to serve as DM-Family's Managing Director.

Learn more about our past and upcoming conferences and watch conference highlight reels

www.myotonic.org/conferences





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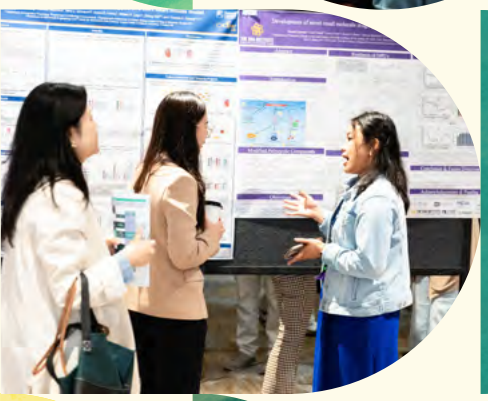







Myotonic Dystrophy
Family Registry

2025 MDF SUPPORT GROUP FACILITATOR WARRIOR AWARDS

Honorees are chosen by their fellow Support Group Facilitators (SGFs) for their commitment to the DM community and SGF peers.

Shaun Moore

Affected Men's Support Group Co-Facilitator

Cynthia "Cindy" Hubert

Washington State Regional Support Group Facilitator

Support Programs

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

2,394
Support Group Participants

312
Support Group Meetings

49
Support Group Facilitators

30
Topic-based and geography-based Support Groups

5
Facebook Groups

4
Support Group Languages*
**German, French, Spanish, English*

For more information about Support Groups:

www.myotonic.org/find-support

Support Groups and Facilitators

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 McPeek

DM2 Caregivers Virtual Support Group
Kim McPeek

Caregiver Virtual Support Group
Annette Rnjak and Ted Salwin

Caregivers of Children with CDM
Sarah Berman

Unaffected Caregivers Support Group*
Rose Albanese and Nathan Beucler
* *New in 2026*

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
Luke Desforges and Carolyn Valek

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

GEOGRAPHY-BASED

California
Rose Albanese

Canada
Alex LeBoeuf, Julie LeBoeuf, and Marie-Claude Sauvé

Chicago, IL
Rob Besecker and Ryan Vogels

Finger Lakes and Upstate New York
Emily Jones and Lois Schenk

Florida
Beth Feigenblatt, Kristen McClintock, and Mark Planco

Kansas City
John Cooley and Pat Gibson

Maryland
Caroline Easterling and Julian Easterling

Michigan
Suzanne Perkins

Mountain West Region
Kathie Thorsland

New England
Jeannine DeSoi and Bill Nuttall

New York City/New Jersey
Kathy Coletta and Guillermo Zubillaga

Southeast
Chuck Hunt and Mindy Kim

Ohio
Nathan Beucler and Carolyn Valek

Portland, OR
Mark Coplin

Texas
Peggy M. and Lynn S.

Virginia
Jodie Howell and Samantha Welsh

Washington State
Cindy Hubert

FACEBOOK GROUPS

DM1
Erin Beucler and Bill Nuttal

DM2
Kim Reynolds, Kelsey Freedman, Tom McPeek

Caregivers; Unaffected Male Caregivers
Nathan Beucler

JOA
Unmoderated

Our heartfelt gratitude to Jan Jaffe, Jonathan Friedman, and Barbara Ochoa, who retired from their Support Group Facilitator roles in 2025 after years of dedicated service to our community.

We hold Scott Virgo, Michigan Support Group Facilitator, in loving memory.

Meet our Support Group Facilitators at:

www.myotonic.org/sgfs

DM in Motion

The second annual Myotonic Dystrophy In Motion (MDIM) Awareness Month took place in July 2025.

251
Registrants

235+
Recording views

16
Countries represented

"I appreciate everyone's efforts to make this meaningful and useful to us all, at our level of ability."

— JANET, COMMUNITY PARTICIPANT

Learn about Myotonic Dystrophy In Motion Awareness Month, view the weekly webinars and Stump the Doctor session, and download our Exercising with DM Infographic.

www.myotonic.org/in-motion

WEEKLY *MOVEMENT MONDAY* PROGRAMMING

MDF hosted four weekly webinars for community members to learn and move with experts, rooted in the four pillars of movement for people living with DM:

Week 1:
Resistance Training

Week 2:
Balance and Fall Prevention

Week 3:
Aerobic and Cardio Training

Week 4:
Flexibility and Chair Yoga

MOVEMENT AND MEETING HAPPY HOURS

Following each *Movement Monday* session, participants were invited to join a social gathering to connect, share movement ideas, and motivate each other.

EXERCISE FOR MYOTONIC DYSTROPHY: STUMP THE DOCTOR BONUS SESSION

Dr. Andy Rohrwasser, MDF Chief Scientific Officer, hosted a special discussion on the role of exercise in myotonic dystrophy. He was joined by Movement Committee Member Donovan Lott, PhD, DPT (University of Florida) and cardiologist Matt Wheeler, MD (Stanford University) to answer community members' questions and share the latest research on how movement supports muscle health and long-term well-being in people with DM. This session was published into a podcast by the American Physical Therapy Association.

JOA Camp

MDF hosts a free camp experience exclusively for JOAs, adults living with juvenile onset myotonic dystrophy. Our 2025 camp was held in August at the Timber Point Outdoor Center in Illinois.

19
Camp activities: Rock climbing, zipline, fishing, karaoke, arts and crafts, archery, animal activities, swimming & more!

16
Campers from across North America between the ages of 18-48

5
Unforgettable days spent creating friendships

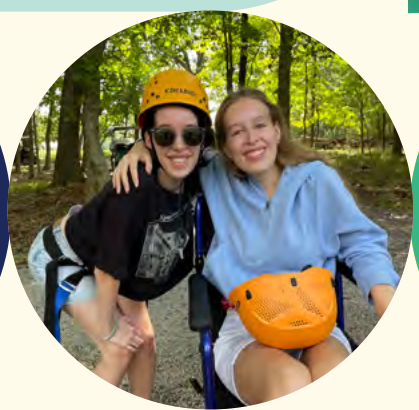
3
Travel scholarships fully covering the cost of traveling to camp

"My sons came home very proud and I can tell they both grew in different ways and got to experience the joy of meaningful connection. They talked about it nonstop for days."

— TODD, JOA PARENT

Learn more about our JOA Camp:

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.

110,000
Resource downloads from www.myotonic.org

14,300
Anesthesia Quick Reference Guide downloads

147
DM Toolkits distributed

48
Videos added to the MDF Digital Academy

ONE-ON-ONE SUPPORT: MDF WARMLINE

The MDF Warmline connects people living with DM—many newly diagnosed—and their family members with resources, support, and education. It provides a welcoming space to talk through concerns, get reliable information, and feel less alone during what can be an overwhelming time. The Warmline team helps guide individuals toward practical resources and a broader community of support.

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

800+
Warmline calls and email interactions

23
Total Ask-the-DM-Expert webinars

ASK-THE-DM-EXPERT

This virtual webinar series, launched in 2021, features DM clinicians and experts in diverse specialties who give recorded presentations and participate in live Q&A sessions with webinar attendees. View the webinars at: ***www.myotonic.org/ask-expert-series***

2025 Webinar Topics

- Mental Health & DM
- DM & Vision Challenges
- Genetics of DM
- Pediatric Endocrinology
- Life Hacks
- Palliative Care

Empowering the DM Community through Education

Access a wealth of materials and resources for individuals, families, and healthcare professionals on the MDF website. **Now available in multiple languages**

TOOLKITS & PUBLICATIONS

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

New Publications & Translations

- *Planning for Adulthood: A Guide for Families and Caretakers of Children with Congenital Myotonic Dystrophy* (published March 2026)
- *Donating Blood & Organs with Myotonic Dystrophy* (published January 2026)
- French-Canadian Language Medical Alert Card
- Spanish Language Medical Alert Card
- Spanish Language Community Anesthesia Guide
- Spanish Language Exercise Infographic

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 *recently expanded* map to help find or contribute information about medical professionals in your area.

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, including past webinars and conference presentations.

36
Total publications now available on website

288
Total videos now in MDF Digital Academy

Myotonic Dystrophy Explained

Launched in 2025, *Understanding Myotonic Dystrophy* is a vibrant series of short animations designed to boost awareness, deepen understanding, and empower people with DM, caregivers, and healthcare providers.

www.myotonic.org/understanding-DM

Episode 1: Understanding Myotonic Dystrophy: The Basics

Episode 2: Inheritance of Myotonic Dystrophy Type 1 (DM1)

Episode 3: Inheritance of Myotonic Dystrophy Type 2 (DM2

Toolkits & Publications

www.myotonic.org/toolkits-publications

Find a Doctor Map

www.myotonic.org/find-a-doctor-map

MDF Digital Academy

www.myotonic.org/digital-academy

Cure

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

MDF Research Investments

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

$1.5 Million+

Invested in Research Programs & Grants in 2025

$8 Million+

MDF investment in Research Fellowships & Grants since 2009

PILOT GRANT PROGRAM

This program drives innovation in the DM field by supporting researchers to explore new ideas, run early studies, and build collaborations.

2025	Since 2024
$250,000	$450,000
5 Pilot Grants	9 Pilot Grants

RESEARCH FELLOWS PROGRAM

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

2025	Since 2009
$375,000	$5,255,000
5 Research Fellowships	61 Research Fellowships

SHORT-TERM HIGH-PRIORITY GRANTS

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

2025	Since 2024
$100,000	$200,000
2 Short-Term High-Priority Grants	4 Short-Term High-Priority Grants

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 grants.

2025	Since 2023
$330,000	$1,280,000
2 Early Career Scholar Grants	7 Early Career Scholar Grants

SMALL GRANTS PROGRAM

Small grants provide funding 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

Advancing Drug Development

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

Bringing Together the Research Community

PROFESSIONAL TRACK OF THE MDF CONFERENCE

Dedicated programming for over 250 DM professionals included sessions covering cardiac care, clinical trial endpoints, and cutting-edge advancements in gene editing, diagnostics, and patient-centered research. Career workshops were offered to young investigators. The networking and poster showcase featured more than 70 posters on DM research.

PHARMA DAY

In Partnership with Euro-DyMA on May 1, 2025

The 5th Annual Pharma Day offered an opportunity for DM drug developers and research professionals to connect, learn, and network.

CARDIAC ENDPOINTS WORKSHOP

In Partnership with the DM Clinical Research Network on May 1, 2025

This half-day workshop included discussions focused on cardiology care standards, neuromuscular cardiology, electrophysiology and cardiac imaging.

NEWBORN SCREENING SYMPOSIUM

In Partnership with the RNA Institute on February 14, 2025

This first-ever symposium was organized to discuss how DM could be added to newborn screening. Newborn screening is one of the most successful public health services testing all babies shortly after birth for certain life-threatening diseases.

Connecting the Community with DM Drug Developers

MEET THE DM DRUG DEVELOPERS

This webinar series, launched in 2021, connects biotech and pharmaceutical partners with our community to share updates and to answer questions in a live format.

5
Meet the DM Drug Developers webinars in 2025

34
Webinars since 2021

20,000+
Total webinar views since 2021

INDUSTRY UPDATES

Dedicated sessions for the DM community on the drug development efforts in the DM field from biotechnology and pharmaceutical companies.

10
Industry update presentations at the 2025 MDF Conference

2025 Industry Partners

AMO PHARMA · arrakis THERAPEUTICS · Dyne THERAPEUTICS · Juvena THERAPEUTICS · NOVARTIS · sanofi · ARTHEx biotech · AVIDITY BIOSCIENCES A Novartis Company · GRITTGENE THERAPEUTICS · LUPIN NEUROSCIENCES · PepGen · VERTEX

View our Industry Updates sessions at:

www.myotonic.org/industry-updates

Watch or participate in Meet the DM Drug Developers webinars:

www.myotonic.org/meet-dm-drug-developers















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Hope



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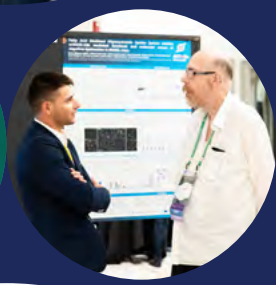
Myotonic Dystrophy Family Registry
Myotonic Dystrophy FOUNDATION
Myotonic Dystrophy IN MOTION


Myotonic Dystrophy FOUNDATION




Myotonic Dystrophy IN MOTION
Myotonic Dystrophy FOUNDATION

Supporting DM Clinical Studies & Trials

STUDY AND TRIAL RECRUITMENT

MDF actively collaborates with drug companies to share timely information about trials and studies, helping connect the community to new research opportunities and encouraging participation.

STUDY: BARRIERS TO CLINICAL TRIAL READINESS

The wide range of DM symptoms makes clinical trials hard to design. In 2025, MDF studied key issues related to clinical trial design, implementation, and outcomes with patients, caregivers, industry, and trial site leaders. Three main problems stood out: not enough reliable ways to measure what matters most to patients, limited long-term data and data sharing, and difficulty getting and keeping people in trials. Participants also said key symptoms—like fatigue, trouble thinking or GI issues—are often missed.

All study participants agreed that better teamwork could help. This includes working closer with the FDA, sharing data, better preparing trial sites, and improving trials designs by focusing on what matters most to patients. These changes could speed up new treatments and improve outcomes.

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.

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.

2900+
Participants in the Myotonic Dystrophy Family Registry

25+
DM drugs in the clinical pipeline

11
DM drugs in Phase I/II/III clinical trials

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

Questions?
Call us at
+1 415-800-7777

Study and Trial Resource Center

www.myotonic.org /study-trial-resource-center

Myotonic Dystrophy Clinical Research Network (DMCRN)

www.myotonic.org/dmcrn

Myotonic Dystrophy Drug Development Pipeline

COMPANY	PROGRAM / LEAD CANDIDATE	MODALITY	DM SUBTYPE	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	DRUG TARGET / MECHANISM
AMO Pharma Ltd	Tideglusib	Small Molecule	CDM / DM1	AMO PHARMA				Glycogen synthase kinase 3 beta (GSK3ß)
Avidity Biosciences	AOC 1001 del-desiran	Antibody Conjugated Oligonucleotide	DM1	AVIDITY BIOSCIENCES				DMPK
Lupin Neurosciences	Mexiletine	Small Molecule	DM1 / DM2	LUPIN				Sodium Channels
Dyne Therapeutics	Dyne 101 z-basivarsen	Antibody Fragment Conjugated Antisense Oligonucleotide	DM1	Dyne				DMPK
ARTHEx Biotech	ATX-01	Antisense microRNA Oligonucleotide	DM1	ARTHEx biotech				MBNL via miR-23b
Harmony Biosciences	Pitolisant	Small Molecule	DM1	HARMONY BIOSCIENCES				Histamine-3 receptor (H3R)
PepGen *(University of Oxford Spinout)*	PGN-EDODM1	Peptide Conjugated Antisense Oligonucleotide	DM1	PepGen				DMPK
Juvena Therapeutics	JUV-161	Stem Cell-Secreted Proteins	DM1	Juvena				—
Vertex	VX-670	Peptide Conjugated Oligonucleotide	DM1	VERTEX				DMPK
Sanofi	—	miRNA Technology in Adeno-Associated Virus	—	sanofi				DMPK
Sarepta	SRP-1003	Investigational RNA interference (RNAi) Therapeutic	DM1	SAREPTA THERAPEUTICS				r(CUG) of DMPK
ARTHEx Biotech	ATX-01	Antisense microRNA Oligonucleotide	CDM / DM2	ARTHEx biotech				MBNL via miR-23b
Arrakis Therapeutics	—	RNA-targeted Small Molecule (rSM)	DM1	arrakis				DMPK
Astellas Gene Therapies	AT466	Adeno-Associated Viral Antisense	DM1	astellas				DMPK
Design Therapeutics	DT818	Gene Targeting Chimera Small Molecule	DM1	DESIGN THERAPEUTICS				r(CUG) of DMPK
EditForce, Inc. EF-210	EF-210	Gene Transfer based Delivery of Pentatricopeptide Repeat Protein	DM1	EditForce				r(CUG) of DMPK
GrittGene Therapeutics	—	—	DM2	GRITTGENE THERAPEUTICS				—
Kinea Bio, Inc.	KNA-145; KNA-129	—	CDM / DM1	KINEA BIO				—
LoQus23	—	Small Molecule	DM1	LoQus23 THERAPEUTICS				MSH3
Modalis Therapeutics	MDL-202	AAV mediated CRISPR base DMPK silencing	DM1	MODALIS				DMPK
Novartis	—	—	DM1	NOVARTIS				—
Rgenta Therapeutics	RGT-DM1		DM1	Rgenta				r(CUG) of DMPK

Learn about different DM drugs in development:
www.myotonic.org/pipeline

As of April 2026

2025 Early Career Scholars

$330,000 INVESTMENT

Lukasz Jakub Sznajder, PhD
University of Nevada, Las Vegas, Nevada, U.S.

The project "Probing Expanded RNA Species and Their Role in DM2" will investigate whether RNA molecules containing retained CCUG repeats play a central role in DM2 disease mechanisms. The study also seeks to develop a cost-effective method to detect and measure intron retention. Using DM2 cell lines, patient tissues, and advanced molecular and bioinformatics tools, this research aims to clarify how DM2 develops and identify potential therapeutic strategies.

Scott Uhlrich, PhD
The University of Utah, Salt Lake City, Utah, U.S.

The project "Novel Digital Functional Outcome Measures for Myotonic Dystrophy Using Smartphone Video" will develop a video-based tool to measure movement in people with DM1. Using OpenCap software and smartphone cameras, the study will analyze everyday activities and apply machine learning to detect subtle movement changes. The team will also test whether reliable assessments can be performed at home with a single smartphone, potentially improving clinical trial sensitivity and accessibility.

2025 Research Fellows

$375,000 INVESTMENT

Louison Daussy, MSc
Institut National de la Santé et de la Recherche Médicale – DR Paris Centre Est, Paris, France

The project "DM1 Neuropathology: From Neuronal Morphology and Axonal Transport to the Reversion of Brain Disease" will investigate how DM1 affects brain cells. Using mouse models, the study will examine changes in neuron structure, function, and protein signaling. A novel experimental approach will test whether correcting gene expression in neurons can reverse brain-related symptoms, addressing a critical question in DM1 research.

Emily Davey, BS
University of Florida, Gainesville, Florida, U.S.

The project "Uncovering Regional and Cell-Type Specific Transcriptomic Signatures in the DM1 Brain" will map RNA changes across 11 brain regions in individuals with DM1. By analyzing RNA at the single-cell level, the study will determine which cell types are most affected and whether changes are localized or widespread. The result will be a detailed transcriptomic atlas to better understand the neurological basis of DM1 symptoms.

Diana Alejandra Madrid Fuentes, MSc
Wake Forest University Health Sciences, Winston-Salem, North Carolina, U.S.

The study "Validating Muscle MRI as a Biomarker of Disease Status in DM2" will evaluate whether quantitative MRI can serve as a reliable biomarker in DM2. By analyzing lower-extremity muscle images from patients and healthy controls using open-source AI tools, the team will measure fat content, muscle quality, and edema. These findings will be compared with functional outcomes such as strength and walking speed to assess clinical relevance.

Haneui Bae, PhD
University of Illinois Urbana-Champaign, Urbana, Illinois, U.S.

The project "Investigating the Molecular Mechanisms of Liver Dysfunctions in Myotonic Dystrophy" will explore how DM1 affects liver function. The study will examine disruptions in nutrient and drug metabolism to better understand disease mechanisms. Findings may guide therapeutic development and improve clinical management of metabolic complications in DM1.

Cécilia Légaré, PhD
Research Foundation of SUNY – University at Albany, Albany, New York, U.S.

The project "Identification of a Transcriptomic Signature in Myotonic Dystrophy Type 1" builds on a 12-week training program that improved strength and function in people with DM1. The team will now study molecular responses in female patients and examine long-term changes over three years. The study will also explore saliva-based biomarkers to identify non-invasive indicators of disease status and treatment response.

2025 Pilot Grant Recipients

$250,000 INVESTMENT

Kate Eichinger, PhD
University of Rochester, Rochester, New York, U.S.

The study "Wearable Sensors to Monitor Gait and Balance in Individuals with Myotonic Dystrophy" will use wearable technology to collect detailed data on walking and balance in adults with DM1. By identifying sensitive measures of functional change, the project aims to improve disease monitoring and support more effective clinical care and research.

Juan Manuel Fernandez, PhD
Institute for Bioengineering of Catalonia, Barcelona, Spain

The project "Biomimetic Muscle Models for In Vitro Functional Analysis and Drug Assessment in DM2 (BMM-2)" will develop a "muscle-on-a-chip" platform using 3D muscle tissue derived from DM2 patient cells. Engineered to replicate key disease features, the system enables functional testing, biomarker discovery, and real-time drug screening, with the goal of accelerating therapeutic development.

David Housman, PhD and
Christopher Ng, ScD
Massachusetts Institute of Technology (MIT), Cambridge, Massachusetts, U.S.

The project "A Targeted DNA Repair Enzyme Therapy for Myotonic Dystrophy" will develop an AAV-based gene therapy to deliver a DNA repair enzyme into muscle cells to stabilize CTG repeat expansions in DM1. The team will evaluate delivery, effectiveness, and safety in patient-derived cells and mouse models, with the long-term goal of advancing toward clinical trials.

Stephanie Tomé, PhD
Sorbonne Université–Inserm UMRS974, Paris, France

The study "Redefining the Genotype-Phenotype Paradigm in Myotonic Dystrophy Type 2" will use advanced genome sequencing to analyze the size and composition of the repeat region in DM2. By examining genetic data from a large patient cohort, the researchers aim to clarify how specific genetic features relate to symptoms, improving diagnosis and prognosis.

Arianna Tucci, MD, PhD
Queen Mary University of London, London, UK

The study "Myotonic Dystrophy Type 2: Using Genomics to Understand Frequency and Expressivity of the Disease" will analyze large-scale genomic datasets, including the UK Biobank, to estimate how frequently the DM2 mutation occurs across populations and identify potentially misdiagnosed cases. Using long-read DNA sequencing, the team will also study genetic variation in greater detail to improve prevalence estimates, diagnosis, and future therapeutic development.

Short-Term, High-Priority Grants

$100,000 INVESTMENT

Katarzyna Taylor, PhD
Adam Mickiewicz University, Poznan, Poland

The project "Identification of Novel Molecular Mechanisms of MBNL1 Expression as Potential DM Therapeutic Targets" will identify regulatory elements that control MBNL1 expression, a key protein involved in DM. By expanding understanding of how MBNL1 is regulated, this work aims to uncover new therapeutic targets and support the development of personalized or combination treatment strategies.

Samuel Carrell, MD, PhD
Virginia Commonwealth University, Richmond, Virginia, U.S.

The project "Identifying Genetic Sources of Disease Variability in Myotonic Dystrophy Type 1" will examine whether additional genes influence disease severity in DM1 and DM2. Using patient-derived muscle progenitor cells, the team will systematically inactivate individual genes and assess their effects on cellular function. Identifying genes that worsen or protect against disease may reveal new pathways for therapeutic development.

Advocacy

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

International DM Awareness

MDF is proud to be a founding member of the Global Alliance for Myotonic Dystrophy Awareness. Established in 2021, The Global Alliance now includes more than 60 international nonprofit organizations, academic and research institutions, biotechnology and pharmaceutical companies, and patient advocacy groups.

International Myotonic Dystrophy Awareness Day

Global Alliance partners mobilize to illuminate the DM community by lighting monuments and landmarks green all over the world on **September 15th** to increase DM visibility across communities and social media.

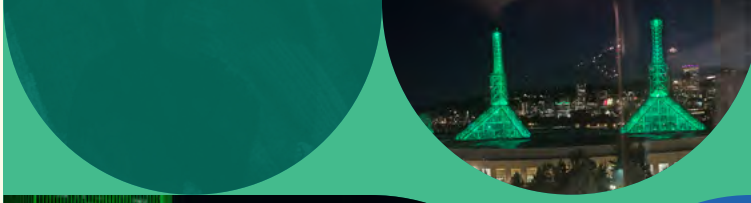
GET INVOLVED!

- 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

2025 KAYLA VITTEK MEMORIAL AWARD FOR OUTSTANDING COMMUNITY ADVOCATE

Beth Feigenblatt

MDF community leader, honored for her advocacy work in Florida and nationally.



INTERNATIONAL Myotonic Dystrophy Awareness Day SEPTEMBER 15

Meet the Global Alliance members and learn more about International Myotonic Dystrophy Awareness Day—including tips, suggestions, and resources for **how to celebrate on September 15th**

www.myotonic.org/international-dm-day

U.S. Congressional Advocacy

The Myotonic Dystrophy Foundation advocates for federal funding for DM research.

PEER REVIEWED MEDICAL RESEARCH PROGRAM (PRMRP)

MDF Congressional advocacy secured federal funding eligibility for DM from the Department of Defense's Peer Reviewed Medical Research Program (PRMRP) for the 9th year in a row. PRMRP funding has been instrumental in enhancing our understanding of both DM1 and DM2 and advancing breakthrough research and therapy development.

CONGRESSIONALLY DIRECTED MEDICAL RESEARCH PROGRAM (CDMRP)

Advocacy campaigns continued to urge Congress to increase federal funding for myotonic dystrophy, requesting $10 million for DM research as part of the Fiscal Year 2026 Congressionally Directed Medical Research Program. Thanks to the support of our grassroots advocates, 11 U.S. Senators and Representatives signed on to letters of support for CDMRP funding. Although Congress did not include DM research in the 2026 Fiscal Year budget, we are proud of the work we accomplished together and will try again!

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
Florida

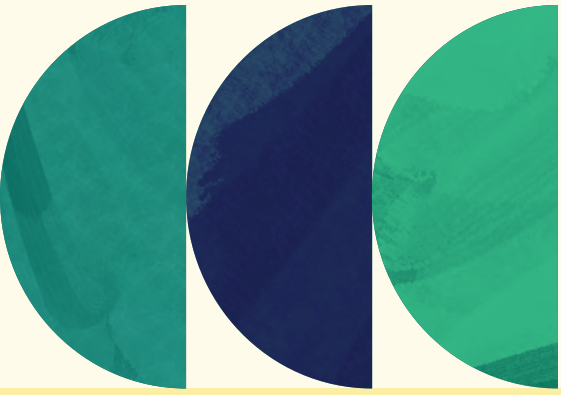
Lisa Harvey-Duren
California

Charles Hunt
Georgia

Emily Jones
New York

Eric Wang
Florida

Leo Zabezhinsky
Minnesota



5,298
Advocacy campaign messages to Congress through Voter Voice platform

990
New advocates participated in advocacy campaigns

2025 MDF LEGISLATIVE ADVOCACY AWARD

U.S. Senator Chris Van Hollen (D-MD)

Honored for advocacy in support of MDF's request for $10 million in DM research as part of the Congressionally Directed Medical Research Program (CDMRP).

Fundraising

A record-breaking year!

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

$4.3 Million
Total contributions

$2 Million
Gala

$408,000
Corporate sponsorships

$170,000
Fundraising campaigns hosted by community members

DIY Fundraising

Community members raised awareness about DM and funds for MDF through a variety of DIY (Do-It-Yourself) fundraising campaigns, ranging from endurance events and golf tournaments to in-person gatherings and social media fundraisers.

SPECIAL THANKS TO OUR DIY FUNDRAISERS, INCLUDING

- **Patrick Cornell** for his record-breaking cross-country motorcycle ride
- **Alita & Mike Ditkowsky and Diane & David Zack** for their St. James Brewery (NY) fundraiser
- **Jon Gulch and Nathan Beucler** for the 14th annual Andrew Gulch Memorial Golf Outing
- **Kelsey Hannah** for her custom t-shirt fundraiser honoring and supporting the Alviani family
- **Karen Papin** for her Miles for JD running campaign
- **Marc Steele** and his team for running 196 miles in the 2025 Ragnar Relay Race
- **Tommy Lancaster** for shaving off 5 years of hair (63 cm) in The Dreaded Shave *(January 2026)*

2025 MDF CONFERENCE

Our MDF Conference raised funds through sponsorships from industry partners, ticket sales, donations, and our exciting Mystery Box Challenge drawing—all of which help offset the costs of bringing our community together for this important event.

$437,425
Total Raised

$278,000
Sponsorships

$144,425
Tickets

$15,000
Donations and Mystery Box Challenge

Pat Cornell "Vroom Old Man" 125k+ Ride for DM

Record-Breaking Cross-Country Motorcycle Journey Raise Awareness and Funds for MDF

With his family deeply affected by myotonic dystrophy, Patrick "Pat" Cornell—a Navy veteran, 9/11 first responder, and retired firefighter—wanted to do something meaningful for the community. Already a seasoned long-distance rider, Pat found his answer on the open road. Known to his followers as "Vroom Old Man," he set out on his 2025 Indian motorcycle to ride 125,000+ miles in 125+ days and raise $125,000 for the Myotonic Dystrophy Foundation—an unprecedented challenge that became a rallying point for riders, rare disease advocates, and DM families across the country.

Pat didn't stop at the finish line. He pushed on to 146,000 miles across 146 days, smashing multiple records along the way. His fundraising goal still within reach, he hit the road again in 2026, counting the miles already ridden toward a new goal of 200,000 miles on one motorcycle in one year.

His determination has drawn national attention to DM and inspired a wave of new supporters to learn about—and give to—the cause.

Thank you, Pat, and all of your generous supporters!

FUNDRAISING ACHIEVEMENTS

$125,000+
Raised

1,025+
Donations

900+
Unique donors

860+
Donors new to MDF

MOTORCYCLE WORLD RECORDS

146,000 Miles in 146 Days
Iron Butt Association Record

Most consecutive 1,000 miles ridden on a motorcycle (72)
Guiness World Record eligible
Iron Butt Association Certified

Longest continuous ride within 1 country by a male rider without going home – 147k+ miles
Guiness World Record eligible

Learn more about Pat's Ride:

https://give.myotonic.org/pat125

2025 MDF Gala

Reaching New Heights • September 18, 2025 • Tribeca Rooftop, New York, NY

OUR ANNUAL GALA SET A NEW RECORD!

$2 Million
raised

360
Guests attended

42
Sponsors

2025 GALA COMMITTEE

Thank you to our 2025 Gala Committee, who organized and executed this successful fundraising event!

Martha Montag Brown
Co-Chair

Erica Kelly
Co-Chair

Leslie Lynch
Co-Chair

Robert Campagna

Suzanne Desforges

Elizabeth Florence

Courtney Harrington

Alex Malinovich

THANK YOU

We are beyond grateful to our event attendees, sponsors, donors, and volunteers for making our 2025 Gala the most successful yet!

Thank you to all of the families and research partners who shared their stories for our three Gala videos, which were filmed at the 2025 MDF Conference in Indianapolis, IN. The videos helped guests better understand the lived experience of DM and the critical role MDF plays in advancing community, care, and a cure for individuals and families.

Join us for the
2026 Gala
on October 10th
in San Francisco!

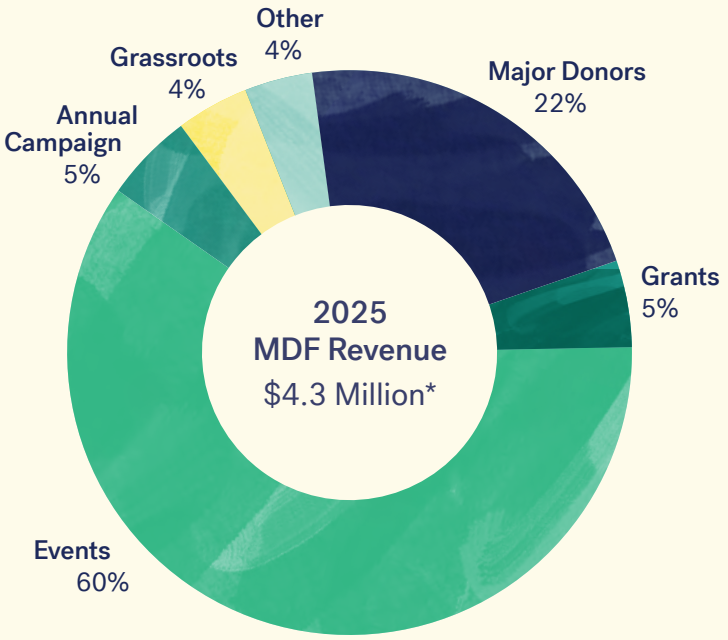

380
378
374
227
315
148
147
291
200
459
310


252
307

2025 MDF Financials

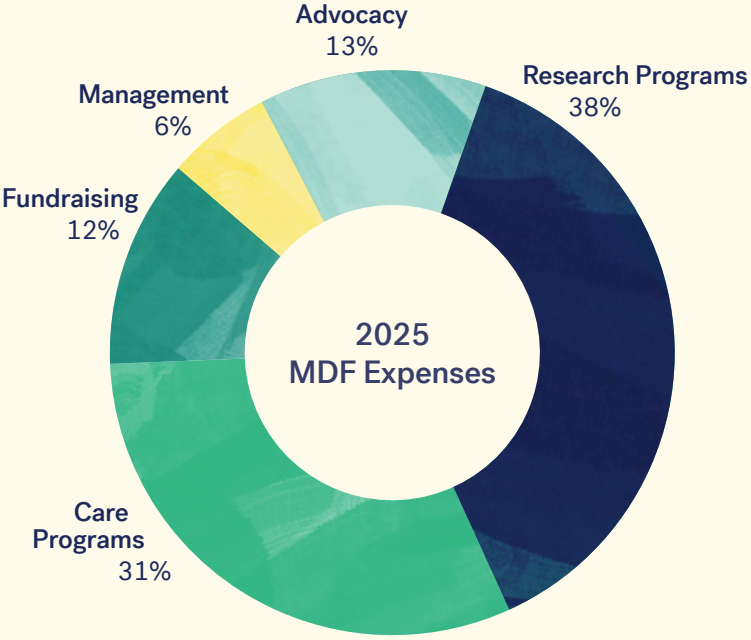
$39 Million+

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



75% of donations are from individual donors.

**Based on unaudited financial statements*



82% to Research, Care, and Advocacy programs in 2025.

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

↑

Invest
in Our
Mission →

Donate Online!

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.

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.

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

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.

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

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.

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

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 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.

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.

Over **30 biopharmaceutical companies are leading promising research** which may result in new treatments for myotonic dystrophy, and, one day, a cure.

Learn more and find citations at:

www.myotonic.org/at-a-glance

Read about MDF's impact at:

www.myotonic.org/impact

Myotonic Dystrophy FOUNDATION

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Oakland, CA 94612

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