Myotonic Dystrophy FOUNDATION

Report to Our Community

2023















Community, Care, and a Cure.

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.

Our Vision

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

Our Values

Community
Collaboration
Empathy
Knowledge
Hope
Urgency

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.

Dear Myotonic Dystrophy Foundation Family,

We are thrilled to share 2023 highlights of the Myotonic Dystrophy Foundation's (MDF) work toward our mission of Community, Care, and a Cure. It was an inspirational and active year full of significant research advancements, knowledge sharing, advocacy, community connection, and optimism.

Our 15th Annual Conference in Washington, DC, united over **600** DM families, clinicians, regulators, researchers, and industry partners from **18** countries and **37** U.S. states. This gathering was more than a conference; it was a powerful demonstration of our collective resolve to drive progress. Advocacy Day before the conference was particularly impactful, with **130** DM community members holding over **100** meetings with policymakers on Capitol Hill. It was this passion and determination that helped myotonic dystrophy, with over **$23 million** in grants secured to date from the Department of Defense, remain eligible for this federal funding for the 7th consecutive year.

Thousands of people utilized MDF's life-saving resources this year, with more than **500** people participating in support groups offered in **3** languages, reaching participants in over **10** countries. As the MDF Community surpassed **30,000** people, our Myotonic Dystrophy Family Registry activity also increased **10**-fold—with **13%** growth of participants—further proof that our community is dedicated to finding a cure for myotonic dystrophy!

Thanks to generous donors, MDF awarded **$1 million** in 2023 to **5** Research Fellows and **3** Early Career Research Grantees—all of which help advance our understanding of DM and move us closer to treatments and a cure!

This year has also been a testament to the strength and unity of our global network. The DM community has stepped up on **4** continents, enrolling in **8** clinical trials, helping move us closer than ever to seeing a drug come to market! September 15th celebrated this feat, as our 3rd Annual International Myotonic Dystrophy Awareness Day sparked an inspiring collaboration of **57** partners worldwide. This global effort not only raised crucial awareness but also reinforced our shared commitment to improving the lives of those affected by myotonic dystrophy. To better meet the needs of our growing international DM community, German and Spanish language hubs were added to the MDF website.

The November Gala on the Big Island of Hawaii raised **$1.1 million**, exemplifying the dedication of our supporters and their belief in our mission. Each of our 2023 accomplishments brings us closer to a future where myotonic dystrophy is no longer a barrier to living a full and vibrant life. Together, we are making significant strides in executing our strategic plan, and are more hopeful than ever for what lies ahead.

With deepest gratitude,

Tanya Stevenson, EdD, MPH
Chief Executive Officer

Jeremy Kelly
Chair, Board of Directors

Community

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

Our Annual Conference

- MDF hosted its 15th Annual Conference in Washington, D.C., in September 2023.
- The conference kicked off with our Advocacy Day on Capitol Hill *(see page 17 for details)* and featured 2 full days of educational, networking, and community panel sessions.

NEW! Our Conference Networking Buddies system paired DM Professionals with people living with DM and their caregivers, offering an exciting and new way for our community to get to know one another. We had 130 Buddy Pairs!

NEW! Our conference featured a Wellness Room, which offered quiet spaces and an array of activities designed to relax, rejuvenate, and restore, including Mindfulness Moments and Fireside Chats with experts.

- Attendees marveled at the Friday dinner performance by Magician and Mentalist David Gerard.
- At the conference's closing dinner, community member Jackson Cooley bravely took to the stage to make a surprise marriage proposal to Iberia Dunning (she said "Yes!"). These two first met and became friends at the MDF annual conference 5 years prior!

617
In-person and virtual attendees

37
U.S. States represented

225
Professionals in attendance

35
Travel scholarships provided

151
First-time attendees

18
Countries represented

56
Sessions

15
Updates from DM drug developers

1
Marriage proposal

Margaret Bowler

MDF LIFETIME ACHIEVEMENT AWARD

Mrs. Margaret Bowler of Nottingham, England was honored with the MDF Lifetime Achievement Award 35 years after launching the world's first myotonic dystrophy patient advocacy organization: Myotonic Dystrophy Support Group UK! She has dedicated her life to raising awareness about DM amongst the general public, clinicians, and researchers, and is an inspiration for advocacy work around the globe. Thank you, Margaret, for moving the field forward!

Learn more about our upcoming and past MDF Annual Conferences: www.myotonic.org/our-annual-conferences

Save the date for the 2025 MDF Annual Conference in Indianapolis, Indiana from May 2nd to May 4th!

MDF Partners with the University of Kansas Medical Center

In August 2023, MDF worked with the University of Kansas Medical Center to host a DM Day and a Medical School Roadshow.

- The Kansas City DM Day brought together MDF, clinicians and researchers, and the DM patient and family community. The one-day event offered a wonderful opportunity to hear research updates first-hand, learn about current DM disease management strategies from local DM health experts, and network with other community members.
- The Medical School Roadshow was designed to help educate students about DM before they graduate and begin clinical work, ultimately to improve clinical care and shorten the time to a diagnosis.

2024 MDF Regional Conferences

In 2024, MDF will host 6 Regional Conferences across the United States. These one-day conferences aim to connect local communities, bring DM resources to the DM community, and provide attendees with the opportunity to connect more closely with local researchers and clinicians.

2024 Regional Conference Tour Dates

Gainesville, Florida
March 23rd

Boston, Massachusetts
April 6th

Iowa City, Iowa
April 20th

Houston, Texas
May 4th

Los Angeles, California
May 18th

Seattle, Washington
June 1st

Support Programs

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

- Support programs included monthly in-person and virtual Support Group meetings, moderated Facebook Groups and Live Chats, and a virtual Friday Happy Hour.
- Thanks to our incredible volunteer Support Group Facilitators, Support Groups were provided in 3 languages: German, Spanish, and English.
- We are grateful for our amazing Facebook Group Moderators: Erin Beucler, Nathan Beucler, Tom McPeek, Bill Nuttall, Kimberly Reynolds, and Samantha Welsh!

"As a participant in MDF's Support Groups, it's really nice to be able to connect with people while sitting in my recliner since I'm not as able to go out and about as I used to [due to physical DM symptoms]."

— SHAUN MOORE, DM1 COMMUNITY MEMBER

265+
Support activities

36
Support Group Facilitators

5
Facebook Groups

175+
Support Group meetings

27
Topic-based and geography-based Support Groups

3
New Support Groups founded in 2023

2023 MDF SUPPORT GROUP FACILITATOR WARRIOR AWARDS

We were thrilled to present these awards publicly for the first time at our 2023 Annual Conference! The honorees are chosen by their fellow Support Group Facilitators (SGFs) for their commitment to the DM community and their SGF peers.

Ann Woodbury

Ann was selected for the Warrior Award for her unwavering support of the community through her work with Juvenile-onset Adults (JOAs) and caregivers of JOAs.

Kristen McClintock

As a new SGF in 2023, Kristen was selected for the Warrior Award for her commitment to the community as both the facilitator for the Florida Support Group and co-facilitator for the weekly Happy Hour.

For more information about Support Groups:
www.myotonic.org/find-support

Meet our SGFs at:
www.myotonic.org/sgfs

Support Groups and Facilitators

TOPIC-BASED

Affected Men's Support Group*
Shaun Moore*, Ryan Vogels*, and Jim Dolan*

Affected Women's Support Group*
Haley Martinelli and Jeannine DeSoi

DM2 Virtual Support Group
Tom McPeek and Haley Martinelli

DM2 Caregivers Virtual Support Group
Kim McPeek

Caregiver Virtual Support Group
Ted Salwin and Annette Rnjak*

Caregivers of Children with CDM
Sarah Berman and Loraine Dressler

Telefonische Gesprächsgruppe DM1 / DM2
Bernhard Rogg and Anke Klein

Grupo de Soporte Virtual en Español de MDF
David Kugler and Araceli Mera

Juvenile-onset Adult (JOA) Warriors
Ann Woodbury and Carolyn Valek

Juvenile-onset Adult (JOA) Caregivers
Ann Woodbury

Adult Facebook Chat
Mindy Kim and Bill Nuttall

DM Virtual Happy Hour
Mindy Kim and Kristen McClintock

* *New as of 2024*

GEOGRAPHY-BASED

Atlanta, GA
Chuck Hunt

Canada
Julie LeBoeuf and Alex LeBoeuf

Chicago
Rob Besecker and Ryan Vogels*

Finger Lakes and Upstate New York
Emily Jones and Lois Schenk*

Florida
Kristen McClintock and Beth Feigenblatt*

Kanas City (In-Person)
John Cooley and Pat Gibson

Massachusetts
Jeannine DeSoi and Bill Nuttall

Michigan (Hybrid In-Person/Virtual)
Suzanne Perkins and Scott Virgo

Mountain West Region
Kathie Thorsland* and Kay Hayes

New York City/New Jersey
Glenda Winson, Janis Jaffe, and Guillermo Zubillaga

North & South Carolina
Mindy Kim

Ohio
Carolyn Valek and Nathan Beucler*

Portland, OR (In-Person/Virtual)
Mark Coplin

Southern California
Rose Albanese*, Barbara Ochoa*, and Loraine Dressler

Virginia
Samantha Welsh and Jodie Howell

Washington State
Cindy Hubert and Jonathan Freedman

Care

The Myotonic Dystrophy Foundation provides resources and advocates for care.

- MDF empowers the DM community through education. We expanded our materials and resources for individuals, families, and clinicians, all accessible on our website.
- We published brand new clinical care recommendations on pregnancy and DM.
- Our team began work on new publications for individuals living with DM and their caregivers, including a mental health handbook and a heart guide *(both published in 2024 and now available on our website)*.
- We published Spanish translations of clinical care recommendations, our exercise guide, and MDF's life-saving anesthesia guidelines.
- Our Spanish and German language hubs on our website, which list our resources available in each language, reached approximately 4,000 users.
- MDF's Warmline connected patients—many newly diagnosed—and their family members with resources, support, clinical care referrals, and education.

33,000
Resource downloads from our website

300+
Warmline calls and email interactions

200
DM Toolkits distributed

26
Publications available on website

9
Languages of MDF resources

8
New Spanish translations of publications

"As a patient/person living with DM, I am able to refer to the Mental Health Handbook with the knowledge that it will answer many (if not most) of my general questions about my mental health in relationship with my DM."

— RUTH SHELDON, MPH, MSW, DM1 COMMUNITY MEMBER

¡Buenos días!
Visit our Spanish language hub:
www.myotonic.org/espanol

Guten Tag!
Visit our German language hub:
www.myotonic.org/deutsch

Educational Resources

Ask-the-DM-Expert
Virtual webinar series with experts in GI, brain, heart, speech and swallowing, mental health, lungs, and other specialties related to DM
www.myotonic.org/ask-expert-series

Find-a-Doctor Map
Community-driven map of medical professionals who have experience working with DM patients
www.myotonic.org/find-a-doctor-map

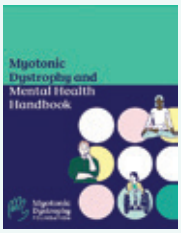
MDF Digital Academy
Largest DM-focused digital library in the world, housing educational and inspirational videos by DM experts
www.myotonic.org/digital-academy

Toolkits & Publications
Resources that guide health care providers and families in the care and management of DM
www.myotonic.org/toolkits-publications

Find Support from MDF!

Contact us at +1 415-800-7777 or info@myotonic.org

New Resources



Myotonic Dystrophy and Mental Health Handbook

This handbook hopes to give the reader an overview of possible mental health issues that may affect people living with DM and provide potential resources.

www.myotonic.org/mentalhealth

Myotonic Dystrophy and the Heart: A Community Guide

This guide aims to help people living with DM understand heart health risks and how they are managed.

www.myotonic.org/heart-guide

Clinical Recommendations for People of Pregnancy Potential with Myotonic Dystrophy

This resource is designed to provide clinicians with an overview of risks and care recommendations for individuals living with DM who are pregnant or considering pregnancy.

www.myotonic.org/pregnancy

Cure

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

Advancements in DM Research

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

- MDF's pivotal role in DM research and drug development includes:
  - » Support of clinical trials and design
  - » Helping researchers and drug developers understand the lived experience of people with DM
  - » Helping FDA and other regulatory agencies understand patient experience and priorities
  - » Expanding DM clinical study and trial infrastructure by contributing funding and support to the Myotonic Dystrophy Clinical Research Network (DMCRN), comprising more than 20 medical centers in the U.S., Europe, Japan, and New Zealand
- DM drug therapies in clinical trials doubled in 2023. In 2022, 4 treatments were in Phase I/II clinical trials. In 2023, the field expanded to 8 treatments and continues to grow.
- For the third year, we hosted our Meet the DM Drug Developers webinar series, which provided 7 biotechnology and pharmaceutical partners the opportunity to sit down with our community, share their progress, and answer questions live.
- We connected individuals living with DM with information about research studies and trials through our virtual Study & Trial Resource Center and the Myotonic Dystrophy Family Registry (MDFR).
- The MDFR continued to grow as one of the largest DM patient registries in the world. The MDFR is an invaluable tool that helps advance our understanding of DM.

8
DM drugs in Phase I/II/III clinical trials

100%
Increase in DM drugs in clinical trials since 2022

7
Meet the DM Drug Developers webinars

2600+
Participants in the Myotonic Dystrophy Family Registry (MDFR)

13%
Growth in MDFR participants in 2023 alone

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

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

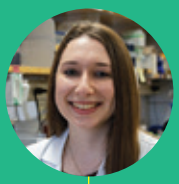
Watch or participate in Meet the DM Drug Developers webinars:
www.myotonic.org/meet-dm-drug-developers

MDF Research Investments

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

NEW! 2023 marked the first year of funding for MDF's Early Career Research Grant program. Through this program, MDF seeks to retain early career scholars who are passionate about research in the myotonic dystrophy field. Recognizing the critical need to recruit and retain clinical researchers early in their career, MDF prioritizes funding clinical researchers and physician-scientists.

- Our MDF Research Fellowship Program, created in 2009, provided two-year pre- and postdoctoral research fellowships to support new and innovative studies relevant to myotonic dystrophy.



"I have grown tremendously as a scientist since my MDF fellowship began in 2023. With the support of MDF, I've navigated the research process with my own project: developing a research plan, conducting research, analyzing data, and sharing it at conferences. I feel so inspired by the patient community, which includes some of my own family, and how they value and engage with research. I am grateful for MDF's support, and I am excited to continue doing research that is important to the DM community."

— EMMA SHEA, MDF DOCTORAL RESEARCH FELLOW, UNIVERSITY OF FLORIDA

$5 Million
MDF investment in Research Fellowships since 2009

52
Number of Research Fellows Funded since 2009

$570,000
for 3 Early Career Research Grants in 2023

$475,000
for 5 Research Fellowships in 2023

Learn more about MDF's research funding program:
www.myotonic.org/grants


Myotonic Dystrophy IN MOTION
Myotonic Dystrophy FOUNDATION
Myotonic Dystrophy Family Registry


17


2024 Review of MDF Research
Fellows and
Early Career Program
Applications


Ice Cream Flavors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Dystrophy
FOUNDATION
Myotonic
Dystrophy
Family Registry

2023 MDF Early Career Researchers
$570,000 Investment

Dylan Farnsworth, PhD

Senior Research Scientist, The RNA Institute, University at Albany, New York, U.S.

Dr. Farnsworth's study aims to understand how myotonic dystrophy affects different cell types in the body by using zebrafish as a model organism. His team will identify which genes are disrupted in specific cells, and how these disruptions contribute to symptoms like digestive issues and sleep disturbances. Since drug testing in zebrafish is very rapid and accessible, they will then test existing drugs in zebrafish to see if they can alleviate these symptoms, potentially leading to new DM treatments.

Matteo Garibaldi, MD, PhD

Assistant Professor, Sapienza University of Rome, Italy

Dr. Garibaldi is investigating muscle involvement in DM1 using muscle MRI and transcriptome analysis to understand the biological processes occurring in affected muscles before fat replacement. By identifying early disease markers and potential therapeutic targets, the research aims to improve treatment timing and intervention strategies for DM1 patients.

Melissa Hale, PhD

Assistant Professor, Virginia Commonwealth University, Richmond, Virginia, U.S.

Dr. Hale is exploring why muscle symptoms in children with congenital myotonic dystrophy (CDM) often improve in early childhood, unlike in adults with DM1, where muscle symptoms decline with age. By analyzing muscle biopsies from individuals living with CDM across various ages, Dr. Hale aims to understand the role of muscle stem cells in this improvement and identify potential new treatments for the most severely affected patients.

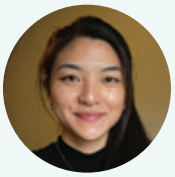
2023 MDF Research Fellows
$475,000 Investment

Mackenzie Davenport, PhD
University of Florida, Gainesville, Florida, U.S.

Dr. Davenport is investigating the exceptional muscle regeneration abilities of the African spiny mouse, which could provide valuable insights for treating muscle wasting in DM in humans. She aims to understand how muscle regeneration failure contributes to DM and to use findings from the spiny mouse to develop potential therapies for DM patients.

Julie Fortin, PhD
Groupe de recherche interdisciplinaire sur les maladies neuromusculaires (GRIMN), Jonquière, Québec, Canada

Dr. Fortin aims to better understand and measure the experiences of fatigue, daytime sleepiness, and apathy in individuals with DM1 through patient-reported outcomes. By developing questionnaires based on the perspectives of people living with DM1, she seeks to improve healthcare for DM1 patients and enhance the effectiveness of clinical trials targeting these debilitating symptoms.

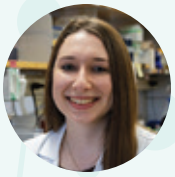


Tatiana Koike, PhD
Université de Montreal, Quebec, Canada

There is a strong therapeutic potential to develop strategies targeting defective muscle stem cells for the treatment of DM1. Dr. Koike and her team will test drugs that could eliminate these defective cells, potentially restoring muscle function. Their approach, which repurposes existing FDA-approved drugs, offers hope for improving the quality of life for DM1 patients with limited treatment options.

Jiss Louis, PhD
The RNA Institute, University at Albany, New York, U.S.

Dr. Louis and her team are researching a natural compound identified by the Berglund lab that reduces toxic RNA in DM1 models and plans to test its effectiveness in DM2 patient cells, with the goal of developing a safe therapy for DM2, for which there are currently no FDA-approved treatments.



Emma Shea
University of Florida, Gainesville, Florida, U.S.

Emma Shea's research aims to develop a CRISPR-Cas9 gene editing therapy to shrink the expanded DNA sequence causing DM. Unlike current treatments that target downstream molecules, this therapy seeks to directly address the root cause, potentially alleviating symptoms and slowing disease progression. By using a modified CRISPR-Cas9 system to make precise cuts near the repeats, the project aims to enhance specificity and induce contraction of the expanded DNA sequence.

Advocacy

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

Advocacy Initiatives

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

- MDF Congressional advocacy secured federal research funding eligibility for DM from the Department of Defense's Peer Reviewed Medical Research Program (PRMRP) for the 7th year in a row.
- Advocacy efforts continued for Congressional support for the Repeat Expansion Disease Initiative (REDI), which enables the National Institutes of Health (NIH) to allocate new federal funding for research on repeat expansion diseases like myotonic dystrophy.
- We hosted a Rare Disease Advocacy Webinar in February focused on MDF research funding legislative priorities (PRMRP, REDI) and taught MDF Advocates how to contact Congress urging support for our efforts.
- For Myotonic Dystrophy Advocacy Week (April 17-21), we launched a social media campaign urging and training MDF Advocates to email, call, and visit Congress in support of PRMRP and REDI funding requests.
- With rare disease partners, MDF advocated for passage of the following legislation: Newborn Screening Saves Lives Reauthorization Act; STAT (Speeding Therapy Access Today) Act; and the Orphan Drug Tax Credit Act.

NATIONAL ADVOCACY COMMITTEE

MDF's NAC was founded in 2022 to help grow the number of DM advocates. We are grateful to our NAC Members for their advocacy leadership!

Rob Besecker
Illinois

Belen Esparis
Pennsylvania

Martha Montag Brown
California

Charles "Chuck" Hunt
Georgia

Rebecca Coplin
Oregon

Emily Jones
New York

Loraine Dressler
California

Eric Wang
Florida

$6 Million
PRMRP funding in 2023

$9 Million
NIH myotonic dystrophy research funding in 2023

Rebecca Coplin

5TH ANNUAL KAYLA VITTEK MEMORIAL AWARD FOR OUTSTANDING COMMUNITY ADVOCATE

We are honored to present the Kayla Vittek Memorial Award annually to MDF community members who have made a significant contribution to raising awareness about DM, as well as advocating for critically needed research and resources. After her family's diagnosis in 2018, Rebecca Coplin has advocated for stronger policies and more funding for DM research at both state and federal levels. She began volunteering for MDF in 2020, reviewing resources and participating in the Portland Support Group, serving on a biotech's patient advisory council, serving as a DM grant reviewer for the U.S. Department of Defense's Peer Reviewed Medical Research Program, joining MDF's National Advocacy Committee, and serving as the Oregon State Captain for Advocacy Day on Capitol Hill, which she also helped organize! Thank you, Rebecca!

Advocacy Day

MDF hosted its Advocacy Day on Capitol Hill on September 7, 2023.

- 130 MDF Advocates from 23 U.S. states and 5 other countries visited Congress in Washington, D.C., to ask for more federal research funding for myotonic dystrophy.
- MDF Advocates completed more than 100 Congressional meetings, spreading awareness of the seriousness of myotonic dystrophy and the urgent need for treatments and a cure.
- We sponsored a 100-person lunchtime Congressional briefing to discuss the importance of increased funding for DM research, which was attended by MDF community members, industry, and leading federal researchers and agencies, including:
  - » **U.S. Senator Amy Klobuchar (D-MN)**
    Rare Disease Congressional Caucus Co-Chair
  - » **Lindsey A. Criswell, MD, MPH, DSc**
    National Institute for Arthritis, Musculoskeletal, and Skin Diseases (NIAMS) Director
  - » **U.S. Army Colonel Sarah Goldman, PhD**
    Congressionally Directed Medical Research Program (CDMRP) Director, Department of Defense
  - » **Chuck Hunt**
    MDF National Advocacy Committee, DM1 Community Member
  - » **Kath Gallagher**
    Avidity Biosciences, SVP, Global Program Head-DM1

Senator Tim Kaine (D-VA)
Senator Cynthia Lummis (R-WY)

2023 MDF CONGRESSIONAL LEADERSHIP AWARDS

Honored for their exceptional efforts in raising awareness for DM, providing hope, and supporting our community.

"I know that we have a long way to go, but I'm inspired by the dedication and resilience of the myotonic dystrophy community. Together, I believe we can make progress in finding better treatments and eventually a cure for this disease." — SENATOR TIM KAINE

Learn more about Advocacy Day 2023 on Capitol Hill:
www.myotonic.org/advocacy-day

Learn more about MDF's advocacy initiatives:
www.myotonic.org/advocate

International Myotonic Dystrophy 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 59 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:
  - **Awareness and education among clinical care teams:** Work with medical professionals to increase their understanding of DM to improve the quality of care that individuals with DM receive, reduce the time to diagnosis, and ultimately improve their quality of life.
  - **Clinical trial readiness for participants:** Improve access to information about trials and support for those who wish to participate and work to ensure that clinical trials are designed to be inclusive, accessible, and accommodating for all individuals with DM.
- 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

Celebrate on September 15th!

For tips, suggestions, and resources visit:
www.myotonic.org/international-dm-day

The Global Alliance for Myotonic Dystrophy Awareness

Algeria | Australia | Belgium | Canada | Canada | Denmark | Europe

France | Germany | Greece | Italy | Italy | Japan | Netherlands

Netherlands | New Zealand | Spain | Spain | Spain | Spain | Switzerland | Switzerland

United Kingdom | United Kingdom | United Kingdom | United Kingdom | United Kingdom | United Kingdom

United Kingdom | United Kingdom | USA | USA | USA | USA

USA | USA | USA | USA | USA | USA

USA | USA | USA | USA | USA | USA

USA | USA | USA | USA | USA | USA | USA

USA | USA | USA | USA | USA | Worldwide

Fundraising

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

- Individual and institutional contributions to MDF totaled more than $2.6 million in 2023!
- Our Annual Conference raised more than $200,000 from 15 sponsors, including industry partners and family foundations.
- Our dedicated community members hosted a total of 193 social media fundraisers to support MDF, raising nearly $46,000 from 929 individual gifts.
- The 13th annual Andrew Gulch Memorial Golf Outing held in the Toledo, Ohio area hosted 230 participants and raised approximately $11,000 for MDF! This special event is in memory of the Gulch family's son, Andrew, who was born with myotonic dystrophy and passed away in 2001. We are grateful for the continued partnership with event organizers Jon Gulch and Nathan Beucler.

Groovy Garibaldi

RELAY TEAM SWIMS ACROSS THE ENGLISH CHANNEL

The Groovy Garibaldi was a team of four women from the San Diego, CA area who love open water swimming for the stunning views from the water, the encounters with sea life, the therapeutic effects, and the community. The team captain, Michella, successfully completed a solo swim of the English Channel in June 2021, and wanted to do it again "for fun" with friends. Two of the team members work in a patient-focused biotechnology company revolutionizing a new class of target RNA therapeutics, focusing on rare neuromuscular diseases, including myotonic dystrophy type 1. The four decided to swim across the English Channel and dedicate the swim to MDF. Thank you to Rachel Johns, Anneke Raney, Michella Thomas, and Cyndi Cavanagh for making it from England to France and for your amazing fundraising efforts, which raised more than $11,000 from 86 supporters for MDF!

Interested in hosting your own grassroots fundraiser? Get started at: www.myotonic.org/grassroots

MDF Gala

- MDF hosted its 9th annual fundraising Gala on November 11th at the Mauna Kea Beach Resort on Hawaii's Big Island.
- Thanks to our event attendees, sponsors, donors, and volunteers, we raised $1.1 million to improve quality of life for individuals and families living with DM and accelerate research toward treatments and a cure.
- Thank you to all who shared your personal stories for our Gala video, which helped educate our attendees about DM and inspire them to support our mission.

2023 Gala Committee

Thank you to our volunteer 2023 Gala Committee, who organized and executed such a successful fundraising event!

Alison Woods
Co-Chair

Martha Montag Brown
Co-Chair

Erica Kelly
Co-Chair

Elizabeth Florence

Leslie Lynch

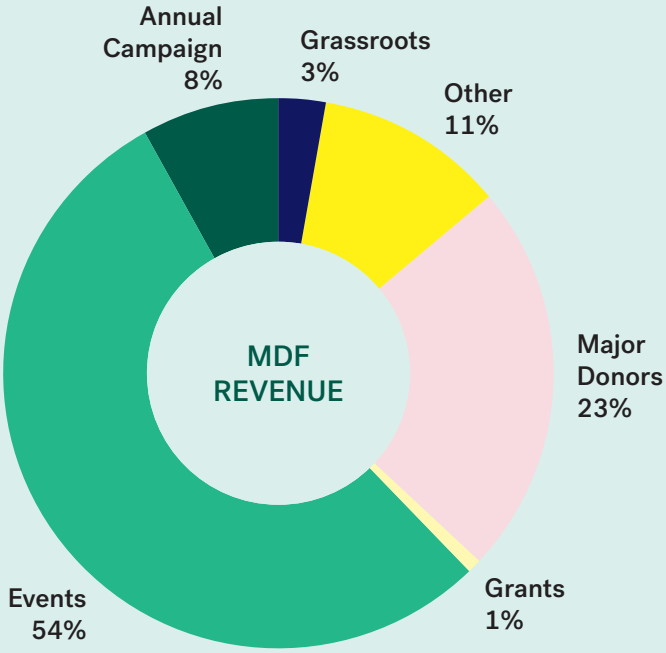
Join us for the 2024 Gala on September 14th in Hollywood, CA!

www.myotonic.org/gala

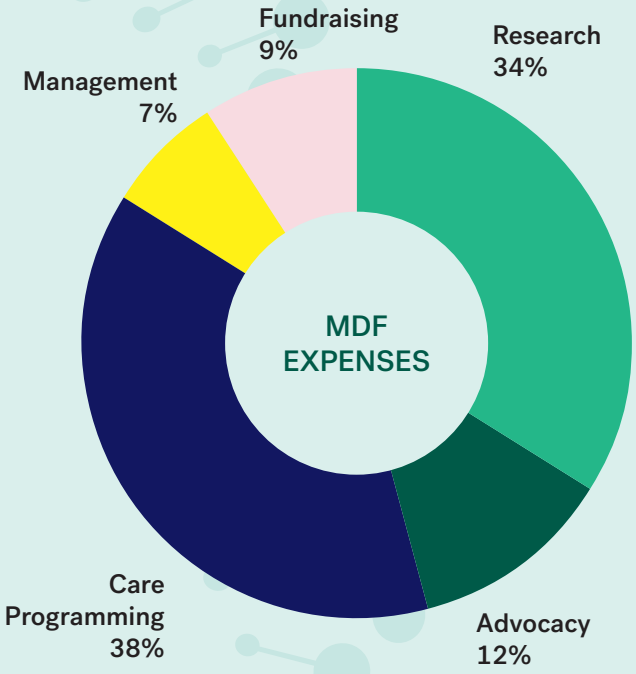
MDF Financials

2023 Audited Financials

$2,648,540 Revenue



90% of donations are from individual donors.



84% of MDF expenses went toward Research, Care, and Advocacy programs in 2023.

Invest in Our Mission

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
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Please contact us for more information

$32 Million

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

Donate Online!
www.myotonic.org/donate

Questions?
Call us at +1 415-800-7777 or email development@myotonic.org

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 **40 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/myotonic-dystrophy-glance

Read about MDF's impact at:
www.myotonic.org/our-impact

Myotonic Dystrophy FOUNDATION

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