

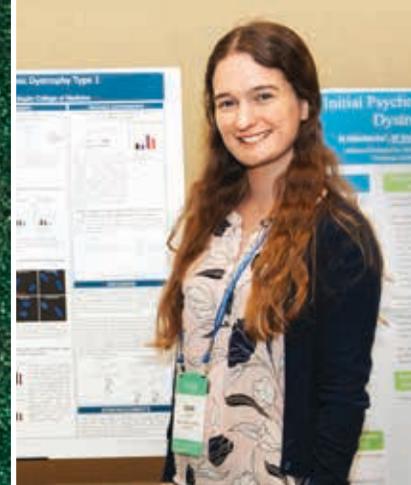


**Myotonic  
Dystrophy**  
FOUNDATION



# Report to the Community 2022





Access the Digital  
2022 Annual Report at:  
[www.myotonic.org/  
Annual-Report-2022](http://www.myotonic.org/Annual-Report-2022)



The Myotonic Dystrophy Foundation (MDF) is a 501(c)(3) nonprofit public charity focusing on Community, Care, and a Cure for people living with myotonic dystrophy (DM).

Every contribution you make helps us deliver essential support and information to patients, families and the medical community. We fund and support important research efforts, raise awareness, and advocate with policy makers.

All donations are tax-deductible to the full extent allowed by law. You will receive a tax receipt from MDF acknowledging your donation either online or by mail.

The Myotonic Dystrophy Foundation's IRS tax EIN is: 20-5014628.

For more information, please contact us.

**663 Thirteenth Street, Suite 100, Oakland, California 94612**  
**415.800.7777 | [info@myotonic.org](mailto:info@myotonic.org) | [www.myotonic.org](http://www.myotonic.org)**



# Report to the Community 2022

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## Community. Care. Cure.

Since 2007, the Myotonic Dystrophy Foundation (MDF) has been the leading global advocate 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.

As the world's largest patient organization focused solely on myotonic dystrophy, MDF is often the only resource international patients can find. To date, MDF has supported the DM community in over 139 countries.

In addition to providing community education and support, MDF is committed to advancing DM research and advocating for legislation and infrastructure initiatives to better understand DM, accelerate drug development, reduce time to diagnosis, and improve quality of care.

Currently tens of thousands of people living with myotonic dystrophy, their families & friends, make up the Myotonic Dystrophy Foundation community.

COMMUNITY  
COLLABORATION  
EMPATHY  
KNOWLEDGE  
HOPE  
URGENCY



## 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.



## Letter From MDF's CEO & Board Chair

Dear Myotonic Dystrophy Foundation Family,

We hope you are all enjoying a beautiful summer with friends and family!

The last year has been incredible for our community and we are thrilled to share some of the remarkable progress we have made together. Despite the distances that separated us during the pandemic, our community demonstrated extraordinary resilience and emerged even stronger in 2022. As we began to gather in person again, we found that our connections to each other and to our shared mission remained as robust as ever. The hopeful and confident spirit of our community propelled MDF to achieve unprecedented breakthroughs in all aspects of our mission – from advancing new initiatives and resources, to growing support groups and international alliances, to making strides in research towards treatments for myotonic dystrophy (DM) – all the while expanding our team nearly twofold.

We take great pride in the success of our in-person events held throughout the year, including our Annual Conference in San Diego that hosted over 600 people from 39 US states and 14 different countries, and our record-breaking fundraising gala in New York City that raised over \$1.35 million in critical funds. Our continued investment in research yielded significant results as we funded an unprecedented eight MDF Research Fellowships, supported our pharmaceutical and biotechnology partners during multiple clinical trials, and successfully advocated for myotonic dystrophy to be included as an eligible condition to receive research funding through the Peer Reviewed Medical Research Program (PRMRP) for a 6th year in a row. We were overjoyed to see the Department of Defense's PRMRP award a groundbreaking \$8.8 million in research funds to DM professionals in 2022 alone!

We are excited to have also expanded our global reach in 2022, with over 57 members of the Global Alliance for Myotonic Dystrophy Awareness celebrating the second annual International Myotonic Dystrophy Awareness Day on September 15th! MDF also launched eight new support groups, including a virtual Spanish-language support group that now serves people in more than five countries.

As new trials and studies launch over the next year, MDF remains committed to enhancing our impact more than ever before and we remain dedicated to improving the lives of individuals and families living with DM.

Thank you for your ongoing support and for making 2022 a year of community. We hope you enjoy this annual report.

Sincerely,



Tanya Stevenson, EdD, MPH  
Chief Executive Officer



Jeremy Kelly  
Chair, Board of Directors

## 2022 Board of Directors

Jeremy Kelly  
Board Chair & Lifetime Trustee

Martha Montag Brown  
Vice-Chair

Elizabeth Florence, Esq.  
Secretary

David Herbert  
Treasurer

David Berman, MBA  
John W. Day, MD, PhD  
Belen Esparis, MD  
John Fitzpatrick  
Haley Martinelli, Esq.  
Thomas (Tom) McPeck  
Joel Revill  
Charles Thornton, MD

Tanya Stevenson, EdD, MPH  
Chief Executive Officer

## 2022 Scientific Advisory Committee

Charles Thornton, MD  
University of Rochester, SAC Chair

Tetsuo Ashizawa, MD  
Houston Methodist Neurological Institute

Guillaume Bassez, MD, PhD  
Institut de Myologie

Andy Berglund, PhD  
RNA Institute, University at Albany

Kathie Bishop, PhD  
Acadia Pharmaceuticals

Thomas A. Cooper, MD  
Baylor College of Medicine

John W. Day, MD, PhD  
Stanford University

Nicholas E. Johnson, MD, MSci., FAAN  
Virginia Commonwealth University

Douglas Kerr, MD, PhD, MBA  
Generation Bio

Darren Monckton, PhD  
University of Glasgow

Laura Ranum, PhD  
University of Florida

Jacinda Sampson, MD, PhD  
Stanford University

Eric Wang, PhD  
University of Florida

Richard Moxley III, MD  
University of Rochester  
Emeritus Member

# 2022 MDF Annual Conference

## MDF Annual Conferences Through the Years

- 2023 - Washington, DC
- 2022 - San Diego, CA
- 2021 - Virtual
- 2020 - Virtual
- 2019 - Philadelphia, PA
- 2018 - Nashville, TN
- 2017 - San Francisco, CA
- 2016 - Washington, DC
- 2015 - Washington, DC
- 2014 - Washington, DC
- 2013 - Houston, TX
- 2012 - San Francisco, CA
- 2011 - Clearwater, FL
- 2010 - Rochester, MN
- 2009 - Los Angeles, CA

## How many did you attend?

Watch the 2022 MDF Annual Conference sessions at:



[www.myotonic.org/2022-mdf-annual-conference](http://www.myotonic.org/2022-mdf-annual-conference)

**Reunited And It Feels So Good!** For the first time since 2019, our incredible community was able to reunite in-person at the MDF Annual Conference in San Diego, CA. As our first truly hybrid event, we offered remote attendees from around the globe the chance to virtually participate in live sessions as if they were in the room! After two years of online-only conferences, finally meeting in-person made this a “family reunion” to remember.

### Who was there?

- **625** community members and DM professionals both in-person and virtually
- **184** First Time Conference Attendees
- **42** educational, networking, and community panel sessions
- Attendees from **39** US States and **14** countries

## 2022 Conference Highlights

### Movement Moments

Community leaders from the MDF Movement Committee led participants in Movement Moments focused on endurance, strength, mobility, and balance. These activities emphasized the power of movement in every person’s life and provided small examples of ways to move safely, joyfully, and together.

### Avidity Biosciences Lab Tour

Over 40 members of the DM community visited and toured the labs at Avidity’s La Jolla campus to learn more about their drug development process and progress towards a treatment for DM1.

### JOA Program

Members of the Juvenile-onset Adult (JOA) community had their very own track, which took place in the comfy and cozy JOA lounge. Sessions focused on fun and friendship were created just for them.

### Award Winners

**Emily Jones** - *4th Annual Kayla Vittek Memorial Award for Outstanding Community Advocate*, presented by Lisa Harvey-Duren

**Luke Bolt** - *Above & Beyond Award*, presented by MDF’s CEO, Tanya Stevenson, EdD, MPH

**Tetsuo “Tee” Ashizawa, MD** - *Lifetime Achievement Award*, presented by Larry Lord and Darren Monckton, PhD



## Join us in Washington, DC!

The 2023 MDF Annual Conference will take place at the Renaissance Washington DC Downtown Hotel on Sept. 7th, 8th & 9th. Learn more and register at:

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



## Movement Moments



## Avidity Biosciences Lab Tour



## JOA Program



## Award Winners



Emily Jones, Lisa Harvey-Duren



Luke Bolt, Dr. Tanya Stevenson



Dr. Tetsuo "Tee" Ashizawa

# Empowering the DM Community through Education



## Life-Saving Anesthesia Guidelines

One of MDF's most-utilized resources is our downloadable Anesthesia Guidelines, which educates clinical care teams on how to mitigate the severe and life-threatening reactions to anesthesia by DM patients.

Access the English language Anesthesia Guidelines here:



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

*"When my son went into emergency heart surgery, his primary care physician wasn't available to prepare the anesthesiologists for the special needs my son had. Thankfully we found the Anesthesia Guide on the MDF website and were able to get it into the hands of the anesthesiologist before the surgery. I don't know what would have happened if we didn't have that information easily available, but I'm so grateful we did. The surgery went smoothly!"*

~ Community member

Thanks to our international partners, the DM Anesthesia Guidelines are now available in German, Japanese and Spanish.

Access all Guidelines here:



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



## Ask-the-Expert

MDF's virtual series with DM experts is available online. Experts in GI, brain, heart, speech and swallowing, mental health, lungs, and other specialties related to DM share information and answer community questions. View the complete archives at: [www.myotonic.org/ask-expert-series](http://www.myotonic.org/ask-expert-series)



## Find-a-Doctor Map

Finding medical professionals who understand myotonic dystrophy is one of the most important things you can do to help manage the unique symptoms of DM. With your help, MDF has compiled a database of medical professionals who have experience working with DM patients. Use the Find-a-Doctor Map to help you find medical professional in your area at: [www.myotonic.org/find-a-doctor-map](http://www.myotonic.org/find-a-doctor-map)



## Toolkits & Publications

MDF has convened world experts in DM – specialists, researchers, and those living with the disease – to create resources that guide health care providers and families in the care and management of DM. Publications include the MDF Toolkit and Clinical Care Guidelines for DM1, DM2, congenital DM, anesthesia, cardiology, and more. View all toolkits and publications here: [www.myotonic.org/toolkits-publications](http://www.myotonic.org/toolkits-publications)



## MDF Digital Academy

View hours of educational and inspirational videos by DM experts whenever you would like. Videos are categorized by areas of interest, for example, clinical trials and drug approval, DM2, congenital and childhood onset, genetics, and more! [www.myotonic.org/digital-academy](http://www.myotonic.org/digital-academy)



**Need additional support?**

**Call our Warmline at 415.800.7777**



## International Resources



### Serving Our Community Around the World

MDF now offers Spanish and German language hubs on our website, which list our resources available in each language. Created in partnership with community leaders, there are publications, videos, and support groups offered for Spanish and German language speakers.

#### El Grupo de Soporte Virtual en Español de MDF

**Creadores y moderadores: David Kugler y Araceli Mera**



El Grupo de Soporte Virtual en Español de MDF fue inaugurado en 2022 por David, originario de Venezuela, y por Araceli, oriunda de Chile. Actualmente, miembros de más de cinco países hispanoparlantes participan de este grupo.

Visite nuestra página en español: [www.myotonic.org/espanol](http://www.myotonic.org/espanol)

#### Telefonische Gesprächsgruppe DM1/DM2

**Moderatoren: Anke Klein & Bernhard Rogg**



Telefonisches Treffen für Betroffene DM1/DM2 und/oder deren Angehörige. Teilnahme über Computer, Tablet, Smartphone oder Festnetzanschluss möglich.

Lesen Sie mehr über das Treffen der deutschsprachigen Selbsthilfegruppe: [www.myotonic.org/deutsch](http://www.myotonic.org/deutsch)

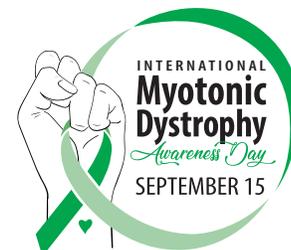
## Save The Dates!



**September 7th, 8th, 9th**  
**2023 MDF Annual Conference**  
[www.myotonic.org/conference](http://www.myotonic.org/conference)



**Friday, September 15th**  
**2023 International DM Awareness Day**  
[www.myotonic.org/international-dm-day](http://www.myotonic.org/international-dm-day)



**November 11th**  
**2023 MDF Gala**  
[www.myotonic.org/gala](http://www.myotonic.org/gala)



# MDF Research Investments



## Fund the Future of DM Research!

Help support the development of new DM researchers, the advancement of DM academic research and discoveries, and provide hope for the DM community by investing in our Fund-A-Fellow program!

Learn more and donate at: [www.myotonic.org/fellows](http://www.myotonic.org/fellows)



## Investing in the Next Generation of Researchers

Since 2009, MDF has invested nearly **\$4.5 million** in the next generation of DM researchers and thought leaders by providing two-year pre- and post-doctoral research fellowships to support new and innovative myotonic dystrophy studies. In 2022, MDF funded a record **8 new Research Fellowships**.

- MDF has funded **52** fellows since 2009!
- Nearly **90%** of MDF Fellows have continued in the field of DM research.

In 2022 we launched an all-new Early Career Research Grant program after recognizing a need to retain early career scholars in the field of myotonic dystrophy. These grants support new researchers with projects that focus on basic, translational and/or clinical research or care projects in myotonic dystrophy. In 2022 applications came in from all over the world and MDF awarded 3 grants which will begin in 2023. To date, nearly **\$7 million** has been spent on research grants and drug development acceleration efforts.



## Research Fellow Spotlight

### Dr. Kamyra Simone Edokpolor – 2021-2022 MDF Research Fellow

Dr. Kamyra Simone Edokpolor completed her MDF Research Fellowship at Emory University School of Medicine, Atlanta, Georgia, where she researched therapeutic strategies for CNS symptoms in DM1. In 2022, Kamyra published a paper on her findings, which identified a potential remedy for excessive daytime sleepiness and altered anesthetic responses in people with myotonic dystrophy, using a mouse model that suggests a link to the neurotransmitter GABA.

The findings may have implications for the development of new treatments. Dr. Edokpolor collaborated with former MDF fellows and partners including 2017 MDF Research Fellow Anwasha Banerjee and 2012 MDF Research Fellow and Current SAC Member Dr. Eric T. Wang.



Read the full research paper: *Altered Behavioral Responses Show GABA Sensitivity in Muscleblind-Like 2-Deficient Mice: Implications for CNS Symptoms in Myotonic Dystrophy.* eNeuro. 2022

## 2022 MDF Research Fellows



**Lily Cisco**

**Doctoral Student at the University of Rochester Medical Center, New York**

The overall goal of Lily's research is to better understand the mechanism of skeletal muscle weakness and degeneration in myotonic dystrophy and to determine if repurposing FDA-approved drugs holds therapeutic promise.



**Avery Engelbrecht**

**Doctoral Student at the University of Florida, Gainesville, Florida**

Avery's research is focused on the generation and characterization of a Myotonic Dystrophy Type 2 (DM2) BAC mouse model, and he is excited to be able to further explore the molecular, physiological, and behavioral features of DM2 in these mice.



**Jesus Frias**

**Doctoral Student at the The RNA Institute, University at Albany, New York**

Jesus' study tests the therapeutic potential of compounds to determine how these compounds work at the molecular level with the aim of developing more effective therapeutics for the disease and ultimately in addressing the need for treatments for DM.



**Christina Heil, PhD**

**Postdoctoral Research Associate at the University of Rochester Medical Center, New York**

Dr. Heil's study uses long-read DNA sequencing to study age-dependent repeat instability in DM1 mouse models, which will optimize long-read DNA sequencing as a tool in research and care, leading to better disease characterization, genetic counseling, and patient prognoses.



**Preeti Kumari, PhD**

**Postdoctoral Fellow at Massachusetts General Hospital, Boston, Massachusetts**

The goal of Dr. Kumari's study is to use cerebrospinal fluid (CSF) samples from myotonic dystrophy patients to identify markers of brain involvement that can be used to detect early changes in the progression of DM and determine whether new treatments are working.



**Larissa Nitschke, PhD**

**Postdoctoral Associate at the Baylor College of Medicine, Houston, Texas**

Dr. Nitschke's study aims to 1) determine the molecular details by which the MBNL2 protein is increased upon loss of MBNL1, 2) investigate the extent to which the compensatory mechanism counteracts DM1 disease and, 3) test if the mechanism can be extrapolated to improve DM1 symptoms.



**Zoe Scherzer**

**Doctoral student at the University of Florida, Gainesville, Florida**

Using a top-down approach to study causes of DM-associated sleep problems, Zoe's study will classify the exact profile of these issues starting at a large scale (whole body). They will then investigate individual components within the body (events happening within body cells).



**Xiaomeng (Belle) Xing**

**Doctoral student at the University of Nottingham, England, UK**

Xiaomeng's study aims to: work out how cells degrade the mutant expRNAs in order to inform future therapeutic approaches for DM; develop a reliable and accurate method to count mutant DMPK transcripts using new technology; measure the binding and dissociation kinetics of RNA-MBNL interactions using a new technique called KIN-CLIP.

# DM Research Advancements

## Connecting the Community with DM Drug Developers

Since 2021 MDF has hosted **Meet-the-DM-Drug-Developers** webinars with our biotechnology and pharmaceutical partners who are actively working on treatments and a cure for DM. We invite them to sit down with our community, share their progress, and answer questions live!

The following partners have shared updates on their ongoing studies and trials:



Since 2007, MDF has helped the DM research and drug development landscape dramatically change. While in 2007 there were only 2 pharmaceutical companies interested in developing drugs for DM and no viable treatments on the horizon, in 2022 we saw 4 DM treatments in Phase I/II clinical trials and over 40 other biotech and pharmaceutical companies and academic institutions in other various stages of development.

Thanks to what is now a thriving ecosystem of researchers and research institutions, biotech and pharmaceutical companies, individuals and families living with DM, and organizations like MDF to support them, there is hope for treatments and a cure for DM!

## MDF's Study & Trial Resource Center

Our community is involved in the first clinical trials of targeted therapies for myotonic dystrophy, and a number of other critical studies are underway. There are a number of ways our community can get involved in research. Learn more by visiting our Study & Trial Resource Center at [www.myotonic.org/study-trial-resource-center](http://www.myotonic.org/study-trial-resource-center)

## DM Clinical Research Network (DMCRN)

Since 2012, MDF has helped expand the DM clinical study and trial infrastructure by contributing funding and support to the Myotonic Dystrophy Clinical Research Network (DMCRN). The network is currently made up of 22 medical centers in the US, Europe, and New Zealand with significant proficiency in myotonic dystrophy clinical care and research. Participating sites in the Network share data, research results, and the rights to publish these results, dramatically lowering barriers to advancing DM science and research.

### Ground-breaking studies by the DMCRN include:

#### END-DM1

The *ESTABLISHING BIOMARKERS AND CLINICAL ENDPOINTS IN MYOTONIC DYSTROPHY TYPE 1: END-DM1* study seeks to fill in gaps in the foundation for successful DM trials and studies, including improving the understanding of DM1 prevalence and disease mechanisms while establishing reliable biomarkers.

#### ASPIRE-DM1

The overall goal of the *ASSESSING PEDIATRIC ENDPOINTS IN DM1: ASPIRE-DM1* study is to establish valid clinical endpoint assessments for children with congenital myotonic dystrophy and childhood myotonic dystrophy type 1 and develop biomarkers for the condition.



Watch or participate in  
Meet-the-DM Drug-Developers:  
[www.myotonic.org/meet-dm-drug-developers](http://www.myotonic.org/meet-dm-drug-developers)

# 2022 Drug Development Snapshot

Check out the 4 DM drugs in Phase I/II clinical trials in 2022!

COMPANY	PROGRAM	MODALITY	DM PATIENT	PRE-CLINICAL	PHASE 1	PHASE 2	PHASE 3
 AMO Pharma	AMO-02 (Tideglusib) for Childhood Onset DM1 - REACH CDM study	Small Molecule	CDM				
 Harmony Biosciences	Safety and Efficacy of Pitolisant on Excessive Daytime Sleepiness and Other Non-Muscular Symptoms in Patients With Myotonic Dystrophy Type 1	Small Molecule	DM1				
 Avidity Biosciences	Study of AOC 1001 in Adult Myotonic Dystrophy Type 1 (DM1) Patients (MARINA)	Antibody Oligonucleotide Conjugate	DM1				
 Dyne Therapeutics	Dyne 101 for Adult Myotonic Dystrophy Type 1 – ACHIEVE study	Antibody Oligonucleotide Conjugate	DM1				

View the DM Drug Development Pipeline here: <https://www.myotonic.org/pipeline>

## Myotonic Dystrophy Family Registry (MDFR)



**Celebrate the 10th anniversary of the Myotonic Dystrophy Family Registry and join today!**

**Join Today!**



If you have been affected by myotonic dystrophy as a patient or a caregiver, we need to hear from you! Join the MDFR and help us create the world's largest community committed to helping end DM.

By joining the MDFR you will:

- Provide critically needed information to researchers pursuing treatments and a cure for myotonic dystrophy
- Make it easier for MDF to connect you with researchers recruiting trial and study participants
- Be informed about the latest news on DM research
- Gain access to anonymous data on symptoms, demographics and other summary information



**It's easy!**

**Log in now: [www.myotonicregistry.org](http://www.myotonicregistry.org)**

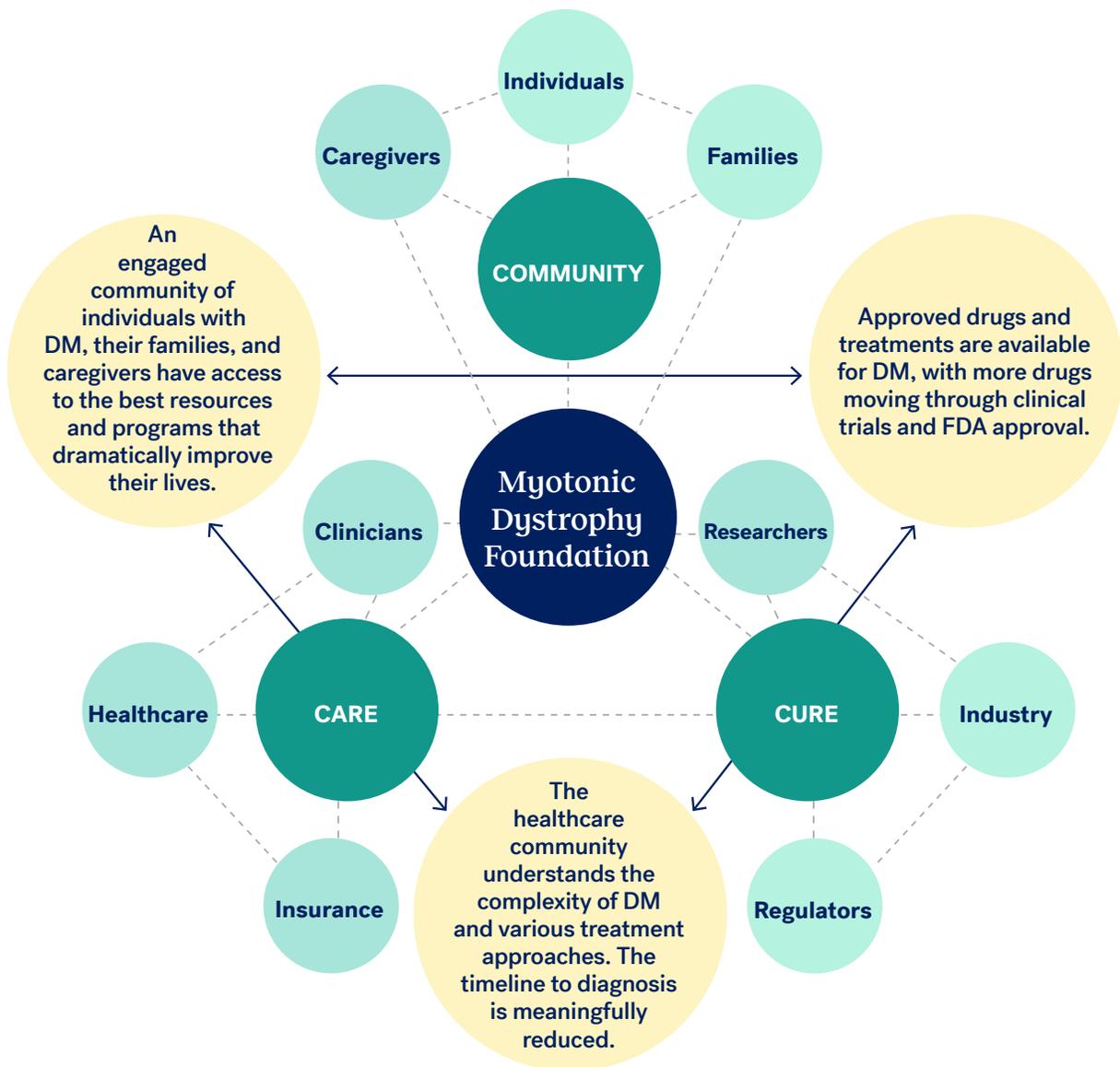
**Questions? Call us at 415-800-7777**

# MDF Theory of Change

**MDF's Theory of Change** connects our mission to our vision. It outlines the logic behind how the Foundation's mission and core activities will produce outputs and outcomes that will bring us closer to our envisioned future where no one is experiencing the negative effects of myotonic dystrophy. Please see the diagram below for a visual representation.

MDF takes a comprehensive and collaborative approach. Through direct services, education, and advocacy, we promote a vibrant and connected community, increase access to appropriate healthcare and crucial resources, and eliminate barriers to drug development. This approach leads to reduced time to diagnosis, improved clinical care, higher quality of life, advancements in research and approved drugs, and ultimately, a cure.

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



We support and connect the myotonic dystrophy community, provide resources and advocate for care, and accelerate research toward treatments and a cure.

# 2022 MDF Support Groups

**Growth in 2022:** Led by our **36** amazing volunteer Support Group Facilitators (SGFs), the MDF support programs create safe spaces to build community, learn, and share. Now, many groups meet virtually as well, allowing access and community connection from all over the world. Support Groups and their facilitators are very often a lifeline to those struggling with a recent diagnosis or coping with symptoms that can make everyday activities difficult and overwhelming. *Thank you to our SGFs for being heroes and pillars of our community!*

In 2022 alone, there were...

- **274** in-person, hybrid, and virtual Support Activities
- **15** new SGFs
- **8** new Support or Discussion Groups founded: Virginia, NYC area, Arizona, San Diego, CDM Families, DM2 Caregivers, International Spanish-Language, and Healthier Living!
- Support Groups in **3** languages: German, Spanish, English



## 2022 MDF Lifetime Achievement Awardee Spotlight MDF Support Group Program Founder: Leslie Krongold, EdD



Dr. Leslie Krongold was diagnosed with DM1 in her mid-30s and quickly became a community leader, taking over an MDA Support Group just six months after joining it. A natural at connecting and communicating, Leslie cultivated a community around her, as she led support groups, completed a doctorate, and educated others on self-management health behaviors.

After meeting MDF's founders and speaking at the 2nd MDF Annual Conference, in 2012 Leslie became MDF's Outreach Director. In her 4 years at the position, Leslie built MDF's Support Group program from the ground up, recruiting facilitators and creating a network of over 20 MDF Support Groups which has since grown to over 30 groups offered in three languages reaching over 10 countries. Today, MDF's Support Groups remain a deeply cherished program.

Over the years, Dr. Krongold continued to lead several MDF groups and advocate for important health-related topics within the community, until retiring from her many roles in 2022. Now based in Mendocino with her wife, Jessica, Leslie hosts her Glass Half Full podcast, has served as a federal research grant reviewer, facilitates two online support groups, launched the annual May Movement Challenge to promote adaptive and accessible exercise, and advocates to make community spaces more accessible. We are all fortunate to have benefited from Leslie's warmth, positivity, energy, and intellect. *Thank you for all that you've done for the DM Community, Dr. Krongold!*

For a complete list of Support Groups and Facilitators visit:



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



*Want to connect  
with others in the  
DM community?*

### Join an MDF Facebook Group!

- Myotonic Dystrophy Type 1
- Myotonic Dystrophy Type 2
- Unaffected Male Caregiver
- Caregivers
- Juvenile-onset Adults (JOA)

## 2022 MDF Support Groups

The Myotonic Dystrophy Foundation is the world's largest myotonic dystrophy patient advocacy organization, connecting people living with DM in over 139 countries around the world. Individuals diagnosed with DM and their families can sometimes feel overwhelmed, isolated, and in need of support. The MDF support programs, led by trained community volunteers, create safe spaces to network, learn, and share. We would like to specially thank our Support Group Facilitators for donating their time and energy to create these unique opportunities.

Find your support community below. Learn more about our SGFs at: [www.myotonic.org/sgfs](http://www.myotonic.org/sgfs)

## MDF Geography-based Support Groups & Facilitators

Arizona, USA



Teresa Cummings

Atlanta, GA, USA



Chuck Hunt

Canada  
Support Group



Alexandra LeBoeuf

Canada  
Support Group



Julie LeBoeuf

Chicago, IL, USA



Rob Besecker, MBA

Dallas, TX, USA



Sherry Morris

Florida, USA



Kristen McClintock

Kansas City  
Region, USA



John Cooley

Kansas City  
Region, USA



Patricia Gibson

Massachusetts, USA



Jeannine DeSoi

Massachusetts, USA



Bill Nuttall

Michigan, USA



Suzanne Perkin

Michigan, USA



Scott Virgo

Mountain West  
Region, USA



Kay Hayes

New York City Area



Janis Jaffe

New York City Area



Susan "Glenda" Winson

New York City Area



Guillermo Zubillaga

N. & S. Carolina, USA



Mindy Kim

Ohio, USA



Carolyn Valek

Portland, OR, USA



Mark Coplin

## MDF Support Groups (cont.)

Rochester, NY, USA



Emily Jones

San Diego, CA USA



Rashid Kassir

S. California, USA



Loraine Dressler

Virginia, USA



Jodie Howell, LPN, CCRP

Virginia, USA



Samantha Welsh

Washington State  
Region, USA



Jonathan Freedman

Washington State  
Region, USA



Cindy Hubert

*"Our family felt lost after our diagnosis... We didn't know anyone had DM outside of our family. But when we found a DM support group nearby, we knew we had to go, and that changed everything overnight. We walked in that room, and we knew for the first time we were not alone."*

~ Support Group attendee

## MDF Virtual Support Groups & Facilitators

Caregivers of Children  
Living with CDM



Sarah Berman

Caregivers of Children  
Living with CDM



Loraine Dressler

Caregiver Call-in  
Support Group



Ted Salwin

DM2 Caregivers  
Support Group



Kim McPeck

DM2 Virtual  
Support Group



Tom McPeck

DM2 Virtual Support  
Group



Haley Martinelli

Grupo de Soporte  
Virtual en Español



David Kugler

Grupo de Soporte  
Virtual en Español



Araceli Mera

Telefonische  
Gesprächsgruppe  
DM1 / DM2



Anke Klein

Telefonische  
Gesprächsgruppe  
DM1 / DM2



Bernhard Rogg

Juvenile-onset  
Adult (JOA) Warriors



Carolyn Valek

Juvenile-onset  
Adult (JOA) Warriors



Ann Woodbury

Adult Facebook Chat,  
DM Virtual Happy Hour



Mindy Kim

Adult Facebook Chat



Bill Nuttall



# Myotonic Dystrophy In Motion

*“Exercise can potentially benefit any part of the body, including muscles, heart, brain, and breathing. But, there are different types of exercise, and the trick is to work with a care team to do it safely and consistently, in ways that can maximize the benefits and minimize the risks of injury, and find a regimen that can be continued long term.”*

~ Charles Thornton, MD,  
Chair, MDF Scientific Advisory Committee

Though exercise does not cure myotonic dystrophy, it can help optimize function and maintain strength. To support our community, MDF launched Myotonic Dystrophy In Motion, a new initiative dedicated to helping the DM community build & maintain a life in motion. No matter where you are or what your ability level may be, we invite you to join the movement!

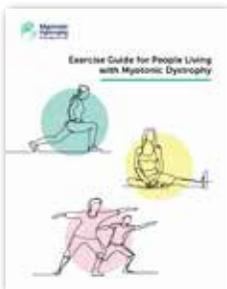
[www.myotonic.org/in-motion](http://www.myotonic.org/in-motion)

## Inspiring Us with Motion



After the incredible outpouring of support for Luke Bolt’s grassroots fundraiser at the 2019 Annual Seattle to Portland Bike Ride — and interest in an

MDF 2018 Hawaii Gala bike jersey he sported during the ride — he sought to keep up the momentum. With the support of Luke’s parents, Jodie and Steve, Team Bolt began dreaming up a line of MDF activewear to inspire others living with DM to get moving, which they sported on their Seattle to Portland ride in 2022. Luke’s ideas and determination helped inspire DM In Motion and earned him the Above & Beyond Award at the 2022 MDF Annual Conference!



## The Exercise Guide for People Living with Myotonic Dystrophy

This Guide explains the benefits of exercise for those living with DM and offers tips and recommendations for safe exercise and fitness. It was written for the DM community by two physical therapists who specialize in working with individuals with DM, Dr. Tina Duong and Dr. Katy Eichinger.

Access the Exercise Guide at:



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

**The MDF Movement Committee** was founded in 2022 to think of ways to inspire our community to move, including the 2022 Conference Movement Moments. Thank you to:

Nathan Beucler

Luke Bolt

Teresa Cummings

Tina Duong, PhD, PT

Kate Eichinger, PhD, PT, DPT, NCS

Lorrie Gallagher

Mindy Kim

Leslie Krongold, EdD

Ellen Shapiro



# 2022 Advocacy Highlights

MDF advocates for legislation, research, and infrastructure initiatives that advance our understanding of myotonic dystrophy, accelerate drug development, and improve diagnosis and care. We strive to raise the visibility of myotonic dystrophy and people living with DM with key stakeholders in Congress, federal and state agencies, medical professionals, and the media.



Learn more about MDF Advocacy Initiatives here: [www.myotonic.org/advocate](http://www.myotonic.org/advocate)

## National Advocacy Committee

In 2022, MDF founded its National Advocacy Committee (NAC) to help grow the number of DM advocates to educate federal agencies about the challenges faced by the DM community and the importance of funding and support for DM research. In preparation for our very first Advocacy Week, the NAC helped organize a training webinar on the “do’s and don’ts” of scheduling a meeting with members of Congress in the US.

Martha Montag Brown,  
California

Dr. Belen Esparis,  
Pennsylvania

Emily Jones,  
New York

Dr. Eric Wang,  
Florida

Rebecca Coplin,  
Oregon

Charles “Chuck” Hunt,  
Georgia

Mindy Kim,  
North Carolina

Rob Besecker,  
Illinois

## Peer Reviewed Medical Research Program (PRMRP)



In 2022, MDF’s advocacy efforts yielded exceptional results as myotonic dystrophy was designated an eligible research condition under the Department of Defense’s Peer Reviewed Medical Research Program (PRMRP) for the sixth consecutive year. This designation provided an opportunity for DM researchers to apply for federal grant funding. An impressive \$8.8 million in research grants was secured for the 2023 fiscal year, which more than doubled the total amount awarded through PRMRP for DM research to date.

Thank you to our MDF Advocates across the nation! This remarkable achievement is truly a testament of your dedication and efforts to engage your elected officials. Let’s do it again next year!

*Congratulations to Dr. Laura Ranum and Dr. Maurice Swanson from the University of Florida on your PRMRP research awards!*



Dr. Laura Ranum



Dr. Maurice Swanson



Learn how to apply for PRMRP funding here: [www.myotonic.org/prmrp-2023](http://www.myotonic.org/prmrp-2023)

## Repeat Expansion Disease Initiative (REDI)



In 2022, MDF successfully secured Congressional support for the Repeat Expansion Disease Initiative (REDI), which will enable the National Institutes of Health (NIH) to allocate new federal funding for research on repeat expansion diseases like myotonic dystrophy.

Congress identified DM as the most significant scientific opportunity among over 50 other genetic diseases also caused by repeat instability and toxic RNA, and asked the NIH to fund cutting edge science which will lead to new treatments and cures for this class of genetic disorders and related conditions. REDI is a significant new opportunity to increase NIH funding for DM research and related genetic conditions, accelerating discoveries and the DM drug development process!

# 2022 MDF Gala



## MDF Breaks Fundraising Record at the 2022 Gala

**On Thursday, October 20th, MDF raised a record \$1.35 million at our annual fundraising Gala at Sony Hall in Midtown Manhattan.** These funds help us accelerate DM research and provide critical programs and services for individuals and families living with myotonic dystrophy.

To our nearly 300 event attendees, our event sponsors, donors, planning committee, speakers, and volunteers—we are in awe of what we accomplished together that night, thanks to your incredible generosity and commitment to MDF’s mission. You made this event the smashing success that it was, and we can’t thank you enough!

We would like to extend a special thank you to:

- Our incredible 2022 Gala Co-Chairs and Committee of the 2022 MDF Gala, who organized and executed our most successful fundraising event in MDF history: Martha Montag Brown, Erica Kelly, Leslie Lynch, Molly Aube, Elizabeth Florence, Shannon Kates, and Alison Woods.
- Tsion Bensusan and Joanne Assor for hosting our community at this spectacular event at the one-of-kind Sony Hall in the heart of New York City.
- Dr. Eric Wang, Jeannine DeSoi, Zoe & Sarah Berman, and all those we interviewed for the Gala, for generously sharing your personal experiences and stories.

*“My diagnosis will never define me, it will mean I’m special and unique in my own way... The doctors said, ‘She’ll never walk, she’ll be wheelchair bound.’ So taking my first steps, my mom said I just fell into the chair and it was the biggest day of my life... They said I’ll never walk, well welcome to never.”*

~ Zoe Berman

Join us or donate to the 2023 Gala in Hawaii: [www.myotonic.org/gala](http://www.myotonic.org/gala)



# 2022 Fundraising Highlights



Brito family

## Grassroots Fundraiser Spotlight: Jesus Brito & World Bonsai Day

Jesus Brito, an award-winning Bonsai artist, and his wife Elena, are parents to 10-year-old Camila, who was born with congenital myotonic dystrophy. After Camila's diagnosis, MDF's resources helped the Britos navigate her care, improve her quality of life, and even defy many of the doctors' predictions.

At D&L Bonsai Nursery's 2022 World Bonsai Day in Ocklawaha, Florida, Jesus was the featured Bonsai Artist and he designated MDF as the beneficiary charity of the day. The all-day event included live bonsai demonstrations, exhibitors, food, and a raffle & auction with all proceeds going to MDF. We can't thank Jesus enough for dedicating his time and his incredible skillset to raise DM awareness and help fund MDF's mission! Start your own fundraiser at: [www.myotonic.org/grassroots](http://www.myotonic.org/grassroots)

## Donor Spotlight: Dr. Lois and Mr. Ellis Oppenheim

Happily married for 52 years with two children, Ellis is a retired NYC business man and Lois is a Medical Humanities professor. While they had suspected for several years that Ellis had undiagnosed health issues, doctors couldn't pinpoint the cause. In 2015, when Ellis' gait changed, they consulted a neurologist who diagnosed him with DM2 and sent them to Dr. Jacinda Sampson. She introduced them to the resources and community available through MDF.

It felt like it was only 5 minutes after the Oppenheims first called MDF that they had a toolkit in their hands and began learning and connecting. They've been involved with MDF ever since, participating in support groups, attending conferences and webinars, and raising awareness. Lois even met one of her best friends through an MDF Facebook group.

Proud of their annual gift to MDF, the Oppenheims strongly believe that the Foundation's programming — bolstered by MDF's strong leadership and Board of Directors — is worth every bit of time and money they've invested over the years. "The organization is exceedingly well run by people who truly understand DM and the DM community — and they keep our community at the center of their decisions." Lois and Ellis contribute to MDF each year because they know their investment will make a difference now and in the future, ensuring the next generation will have the support they need, if they need it, while helping to move us one step closer to a cure. *Thank you, Lois and Ellis, for helping change the future of myotonic dystrophy!*

*"When we want to make a charitable donation, we never think twice about where to make it. MDF is always first."*

~ Lois Oppenheim



Lois and Ellis Oppenheim

**Contribute to  
Community,  
Care, and  
a Cure!**



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



Myotonic Dystrophy Foundation  
663 Thirteenth St., Suite 100  
Oakland, CA 94612



415.800.7777

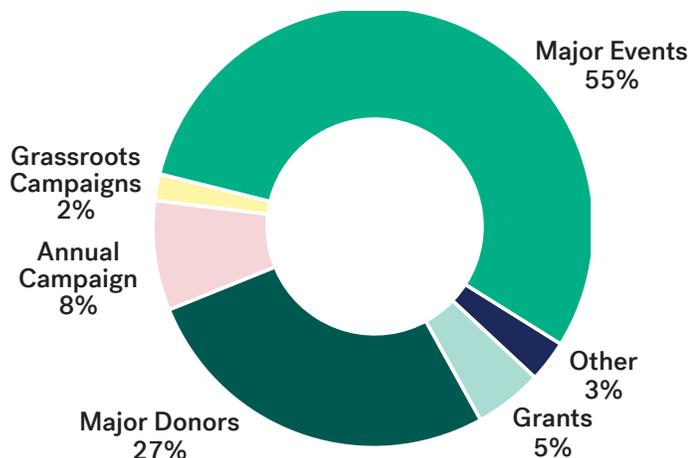
# 2022 Financials

## 2022 MDF Revenue By Source

Total \$2,882,564

**\$1.35 million**

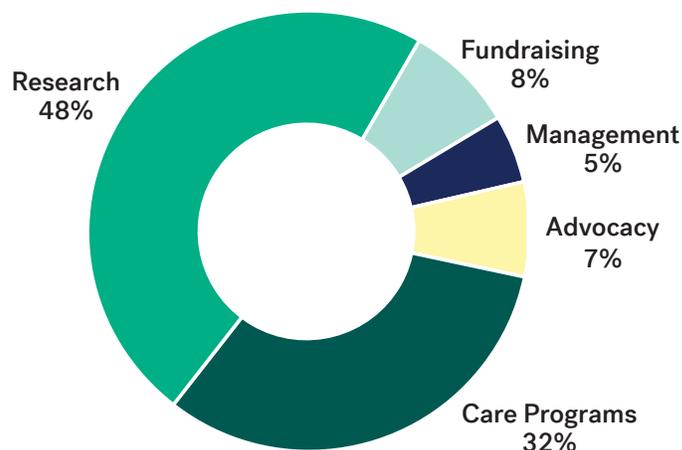
Record amount raised at our 2022 Gala in NYC



## 2022 MDF Expenses

Total \$2,602,081

**87%** of MDF expenses went toward Research, Care, and Advocacy programs in 2022



Find out more about MDF's impact at [www.myotonic.org/our-impact-0](http://www.myotonic.org/our-impact-0)

## MDF Strategic Plan

### A comprehensive, community-directed three-year plan

In January of 2021, MDF set out to create a comprehensive Strategic Plan to articulate a refreshed vision and mission for the organization, as well as set the goals, strategies, and tactics that will be prioritized over the next three years, 2021-2024. Now, MDF is working every day toward four primary goals: Community, Care, Cure and Organizational Strength.

#### Goal 1: COMMUNITY

Strengthen our community

#### Goal 2: CARE

Improve access to effective healthcare that meets the needs of affected individuals, families, and caregivers

#### Goal 3: CURE

Eliminate barriers to accelerate drug development

#### Goal 4: ORGANIZATIONAL STRENGTH

Build a strong, sustainable organization



Read the full Strategic Plan at [www.myotonic.org/strategicplan](http://www.myotonic.org/strategicplan)

## Meet Team MDF!

Kate Beck, *Director of Development*

Kevin Brennan, *Advocacy Consultant*

Mindy Buchanan, *Director of Programs*

Kleed Cumming, *Director of Communications & Technology*

Mindy Kim, *Registry Outreach Specialist*

Michael Knaapen, *Director of Programs (through Sept. 2022)*

Mounica Lakshmi Dugginapeddi, MPH, *Community Programs Coordinator*

Sofia Olmos, PhD, *Myotonic Dystrophy Family Registry Coordinator*

Andrew Olsen, *Operations & Data Specialist*

Emily Romney, MPA, *Special Projects Manager*

Ruth Sheldon, MPH, MSW, *Health Resource Consultant*

Nadine Skinner, PhD, MPA, *Research Coordinator*

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

# 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 doesn't 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](http://www.myotonic.org/myotonic-dystrophy-glance)

# International Myotonic Dystrophy Awareness Day



It is critical to raise awareness about myotonic dystrophy to improve the quality of life of people living with the disease. MDF helped lead a Global Alliance of 57 DM-focused organizations in celebrating the second ever **International Myotonic Dystrophy Awareness Day** on September 15th, 2022.

## Uniting Efforts in Two Shared Areas of Focus

Many members of the Global Alliance also joined together leading awareness efforts in two specific areas of focus to maximize impact in 2022:

1. **Awareness and education among clinical care teams:** Myotonic dystrophy awareness and education among clinicians is crucial to delivering timely diagnoses and the quality of health care that people living with DM require.
2. **Clinical trial readiness for participants:** With 4 drug treatments in clinical trials in 2022, clinical trial readiness for people living with myotonic dystrophy is more important than ever to the development of therapies and a cure.

## U.S. Senate Recognizes International DM Awareness Day

Led by US Senator Tim Kaine (D-VA) and co-sponsored by Sen. Lummis (R-WY), Sen. Klobuchar (D-MN), and Sen. Smith (D-MN), the US Senate passed Senate Resolution 772 officially designating September 15th as International Myotonic Dystrophy Awareness Day.

## MDF'S International Partners Light Monuments and Landmarks in Green All Over the World

On September 15th, 2022, landmarks, monuments, and buildings all over the world shone **green**, the official color of the Global Alliance for DM Awareness. Partners around the globe mobilized to illuminate the DM community.

*Images to the left: Council House, Perth, Australia; Biocruces Bizkaia, Spain; The Great North Run, UK; Empire State Plaza, Albany, N.Y., USA*

## Join Us In Raising Our Voices in Honor of International Myotonic Dystrophy Awareness Day this September 15th!

We encourage all of our community members to come together this DM Awareness Day to share your personal experiences with friends, family, classmates, colleagues and your local community. Here are a few ways you can raise visibility of DM:

- Light up your local monument in green
- Share the **DM-At-A-Glance** sheet at school or work
- Ask neighbors and shops in your community to display an **International Myotonic Dystrophy Awareness Day window sign**
- Share the **DM Toolkit** with healthcare providers in your community



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



# Global Alliance for DM Awareness

MDF is proud to be a founding member of the Global Alliance for Myotonic Dystrophy Awareness! Comprised of dozens of organizations and institutions around the world dedicated to helping raise awareness about DM, all members of the Global Alliance will be celebrating International Myotonic Dystrophy Awareness Day on September 15th. Learn more and join the Alliance here: <https://www.myotonic.org/international-dm-day>



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Belgium



Canada



Canada



Denmark



Europe



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Germany



Greece



Italy



Italy



Japan



Netherlands



Netherlands



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Worldwide



## Contribute to Community, Care, and a Cure!



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



Myotonic Dystrophy Foundation  
663 Thirteenth Street, Suite 100  
Oakland, CA 94612

*Please make checks payable to the  
Myotonic Dystrophy Foundation.*



415.800.7777



**Myotonic  
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