

# Myotonic Dystrophy Foundation

## 2021 in Review



*Together we can change the future  
of myotonic dystrophy*



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Eric Wang, PhD\*

\* *New in 2022!*

## Vision

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

## 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,

In this first MDF Annual Report, we reflect on the progress made during our 15 year history and are excited to share a snapshot of our 2021 achievements. Throughout two years of an often confusing and isolating pandemic, you helped MDF find new and creative ways to connect families living with DM to our support community and comprehensive resource library, increase funding for groundbreaking DM research, and improve DM awareness and care through clinical and policy maker education. With unrivaled resiliency our community simply refused to give up, found ways to participate in remote research and clinical care, and embraced new technology. Your spirit and collaborative leadership remain the inspiration MDF needs to keep breaking down barriers to progress in care and a cure.

As three new clinical trials launched this year, MDF proudly funded five DM Research Fellows with eight more anticipated to start in 2022 (more than any other year)! We hosted the 2nd Virtual MDF Conference with almost 700 participants from 21 countries! We supported and served over 15,000 individuals and families living with DM in 139 countries via support groups, Warmline phone calls, online resources, and symptom management education! In the last year we led the efforts to launch and steward a unified Global Alliance which grew to over 55 diverse DM-focused organizations, who together designated September 15th International Myotonic Dystrophy Awareness Day and advocated for improved DM awareness and care around the world. Over the twelve months of 2021 not only did MDF refuse to slow down but we grew stronger and even more hopeful.

On behalf of our Board of Directors and staff, we would like to thank you for supporting MDF and partnering with us to change the future of myotonic dystrophy. You make all the difference and we are grateful for your trust.

Sincerely,



Jeremy Kelly  
*Board Chair & Lifetime Trustee*



Tanya Stevenson, EdD, MPH  
*Chief Executive Officer*

# 2021-2024 Strategic Plan

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

IN JANUARY OF 2021, the Myotonic Dystrophy Foundation (MDF) set out to create a comprehensive Strategic Plan, directed by the DM community, to articulate a refreshed vision and mission for the organization, as well as set the primary goals, strategies, and tactics that will be prioritized over the next three years, 2021-2024.

To ensure the plan represented MDF's entire ecosystem, the planning process was guided by an 11-person Strategic Planning Steering Committee comprised of MDF's CEO, individuals and families affected by DM, board members, representatives of the scientific and research community, clinicians, and other organizational partners and advocates. MDF enlisted a third party social impact strategy firm, Third Plateau, to facilitate the process.

Over the course of nine months, MDF engaged in a three-phase planning process.

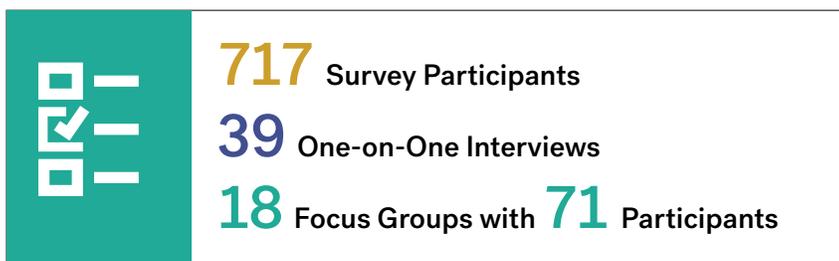
The first phase, or **Learning Phase**, included engaging over **800 participants** through interviews, focus groups, and a large community survey.

The second phase, **Ideation**, focused on defining MDF's desired future state, through analysis of the research findings and planning sessions.

The third and final phase, **Iterative Design**, focused on drafting and refining the plan, as well as defining a feasible implementation timeline, financial implications, and critical milestones and metrics. The full plan was approved by the Board of Directors in December 2021. Now, MDF is working every day toward four primary goals:

- **COMMUNITY:** strengthen our community
- **CARE:** improve access to effective healthcare that meets the needs of affected individuals, families, and caregivers
- **CURE:** eliminate barriers to accelerate drug development
- **ORGANIZATIONAL STRENGTH:** build a strong, sustainable organization

## Strategic Plan Community Participation



To learn more about the Strategic Plan, visit:  
[www.myotonic.org/strategicplan](http://www.myotonic.org/strategicplan)

## MDF Core Values

Our core values guide our approach to pursuing our vision and mission. The values are foundational to MDF's work and reflect the characteristics and behaviors to which we aspire, both as an organization and across our broader community.

collaboration  
EMPATHY community  
HOPE URGENCY  
knowledge

## Strategic Planning Steering Committee

Kevin Brennan  
*Bluebird Strategies, DM Advocacy*

Loraine Dressler  
*MDF Support Group Facilitator & Former Registered Nurse*

Belen Esparis, MD  
*Sleep Medicine Physician, University of Pennsylvania*

John Fitzpatrick  
*MDF Board Member & Strategic Planning Committee Chair*

Sarah Howe, MBA  
*Marigold Foundation, Canada*

Jeremy Kelly  
*Chair, MDF Board of Directors*

Leslie Krongold, EdD  
*MDF Support Group Facilitator*

Tom McPeck  
*MDF Board Member & DM2 Support Group Facilitator*

Tom Silk  
*Chair, MDF UK Board of Trustees*

Tanya Stevenson, EdD, MPH  
*MDF CEO*

Eric Wang, PhD  
*DM Researcher, University of Florida*

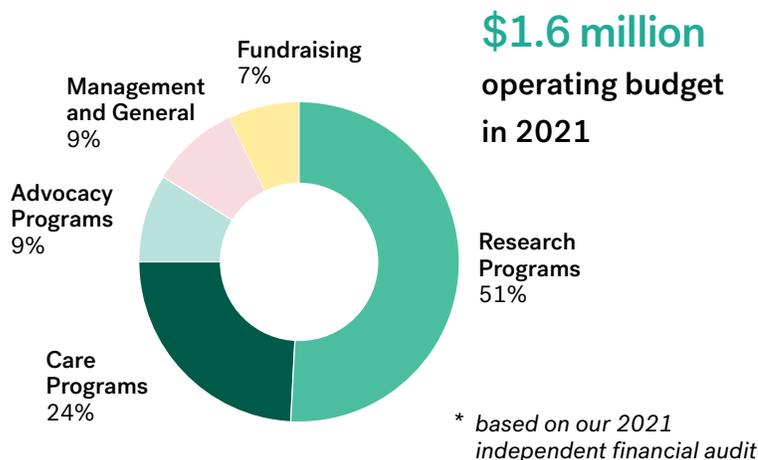
# Thriving During Uncertain Times

**DURING THE COVID-19 PANDEMIC** MDF worked side by side with our community to find new and creative ways to advance our mission in the face of continued uncertainty. We connected families living with DM to our support network and comprehensive resource library, and even created new programs to specifically suit the virtual format. We increased funding for groundbreaking DM research, and improved international DM awareness and care through clinical, community, and government advocacy. We saw unprecedented participation in programs such as the Annual Conference, Annual Fundraising Gala, Support Groups, and educational Webinars, proving the adaptability of our community and the continuing need for the support MDF provides.

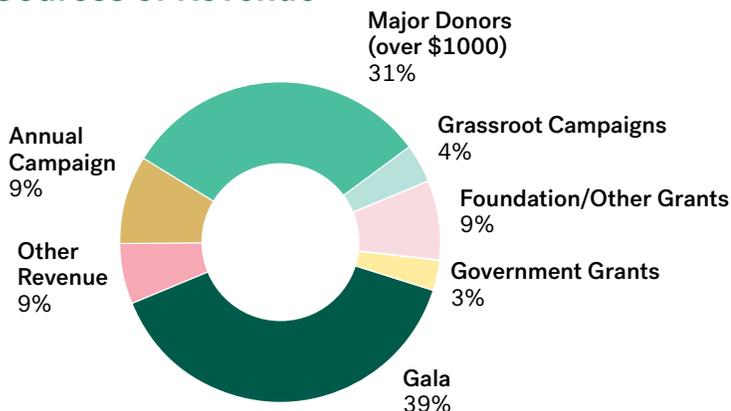
In 2021, **84%** of MDF expenses went toward **Research, Care, and Advocacy** programs.



## 2021 MDF Expenses\*



## 2021 MDF Sources of Revenue



### Did You Know?

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

# Achievements during the COVID-19 pandemic

**2**  
successful  
virtual Galas

Developed our  
**1st**  
community-driven  
Strategic Plan



Supported  
**3**  
new  
clinical trials  
for drugs in  
development



Launched **3**  
new Webinar  
series



**2**  
virtual Family  
Conferences



Launched the  
Global Alliance and  
International  
Myotonic Dystrophy  
Awareness Day  
(Sept 15th)



Initiated  
summer  
internship  
program

**1st** ever  
hybrid Family  
Conference

Launched  
**Myotonic Dystrophy  
In Motion**

Developed the  
Myotonic Dystrophy  
Research Map



Added  
**3**  
new staff  
members

**4**  
new SAC  
members

**3**  
new board  
members



Moved  
the office



Issued  
**8**  
guides on  
health  
insurance,  
employment  
access,  
exercise,  
and more



Created and  
distributed COVID-19  
Clinical Care  
Recommendations  
& FAQs

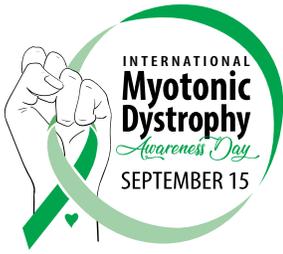


Organized  
**10**  
Community  
Meetings

Finances  
stronger  
than ever



Added **11**  
new Support  
Groups



## International Myotonic Dystrophy Awareness Day

In honor of Rare Disease Day 2021, MDF helped to assemble a Global Alliance of over 21 myotonic dystrophy-focused organizations worldwide to declare September 15th as International Myotonic Dystrophy Awareness Day. Since then, the Global Alliance has grown to 55 organizations on five continents representing all facets of the DM eco-system: Nonprofits, Researchers & Academia, Hospitals & Clinics, Schools, and Biotech/Pharma companies!

International Myotonic Dystrophy Awareness Day aims to garner the attention of the wider general public, policy makers, regulators, bio-pharmaceutical representatives, researchers, health care professionals, and anyone with an interest in changing the future of myotonic dystrophy.

Raising awareness of myotonic dystrophy will help improve service provision, basic research, drug development, and policymaking related to the disease. Increased funding for myotonic dystrophy research will improve health outcomes, reduce disability, and increase life expectancy for individuals living with the disease, and holds great promise for helping individuals with diseases with similar genetic bases, such as Fragile X syndrome and Huntington's disease.

Learn more and get involved at:

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

### Did You Know?

Myotonic dystrophy is referred to as DM, an abbreviation of the Latin name used by doctors and researchers worldwide, *dystrophia myotonica*.



## Advocate Spotlight

Alexandra LeBoeuf was the winner of the International Myotonic Dystrophy Awareness Day Logo Design Contest! Her design won the international competition with a majority of the hundreds of votes from over 22 countries. Since then, across the DM ecosystem around the world, the DM community has used this logo to raise awareness and help signify the day! For her advocacy and commitment to the DM community, she received the 2021 Kayla Vittek Memorial Award for Outstanding Community Advocate!

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## Global Alliance Highlights

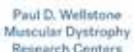
In 2021 MDF organized and facilitated monthly virtual meetings with the Global Alliance — connecting and engaging dozens of global DM-focused organizations in discussion about increasing myotonic dystrophy awareness.

- CureDM UK and partners in the UK lit up over 44 monuments and buildings in green on 9/15/2021!
- MDF created an awareness-raising social media campaign which was shared by dozens of other organizations and included in posts estimated to reach hundreds of thousands of people around the globe!
- MDF hosted a celebratory virtual program featuring the voices of our community, Global Alliance Members, and a special performance by singer-songwriter Eric Hutchinson!
- With the help of community advocates, MDF helped introduce US Senate Resolution 336, which designates September 15th International Myotonic Dystrophy Awareness Day. Similar proclamations were signed in Manitoba, Canada, and New York State!

# Meet the Global Alliance for Myotonic Dystrophy Awareness

Working together to change the future of myotonic dystrophy around the world.

Learn more and join the Alliance at: [www.myotonic.org/international-dm-day](http://www.myotonic.org/international-dm-day)

Algéria	Australia	Belgium	Canada	Denmark	
					
Europe	France	Germany	Greece	Italy	
					
Japan	Netherlands	New Zealand			
					
Spain		Switzerland			
					
United Kingdom					
					
					
USA					
					
					
					
					
					

# Community

*MDF supports and connects the myotonic dystrophy community to research, care, and each other.*

AS THE WORLD'S LARGEST myotonic dystrophy (DM) patient advocacy organization, which serves individuals in over 139 countries, helping foster community for those living with DM is one of our most important jobs. Programs such as our Support Groups, the Warmline, and our Annual Conference are a few of the ways we provide support and the opportunity for connection to our community.

*"I am so grateful for the support, leadership and information only MDF could provide. Thank you for making this meeting happen!"*

— Community Meeting attendee

## Did You Know?

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

## Support Groups

Each month MDF supports dozens of in-person meetings, virtual meetings, and Facebook groups, all run and moderated by trained MDF volunteers. The meetings and groups bring community members together to share their experiences, create lifelong friendships and reduce the isolation that often accompanies this disease.

In 2020 and 2021, many support group facilitators shifted to virtual formats and even created some new groups! Find support here:

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

### Support Groups In 2021

**200** hours of in-person and virtual support group meetings

**30** Support Group Facilitators

**22** regional and topic-based support groups

**5** Facebook Groups

Myotonic Dystrophy Type 1 (DM1)

Myotonic Dystrophy Type 2 (DM2)

Unaffected Male Caregiver Group

Caregivers Group

Juvenile Onset Adult

(JOA) Group

### Community Meetings

MDF also organized **10** Community Meetings across the US over the past two years. These virtual meetings were held to connect our DM community in geographic areas where there are no support groups or a group has not met regularly for several years. Community members learned about MDF's resources, connected with each other, and even came together to start a new support group!

## MDF Annual Conference

For the last **14** years, MDF has hosted the world's largest myotonic dystrophy-only conference in the world. We convene the entire DM ecosystem to provide opportunities to learn, share, grow, and inspire DM families, researchers, clinicians, industry partners, donors and allies. All under one roof! The MDF Annual conference provides families opportunities to connect with one another and allows researchers to learn about the realities of the disease directly from people living with the disease. It also offers the chance to engage with some of the most esteemed DM-researchers in the world and participate in their work in real-time. The conference is also where the DM community can learn about clinical studies and trials underway and ask questions of experts they don't otherwise have access to at home.

Nearly **700** attendees from **38** US States and **22** different countries participated in each of the virtual MDF conferences in 2021 and 2022. Over **20** conference sessions on different topics were offered to families and DM professionals.

## Support Group Facilitator Spotlight

# Mindy Kim

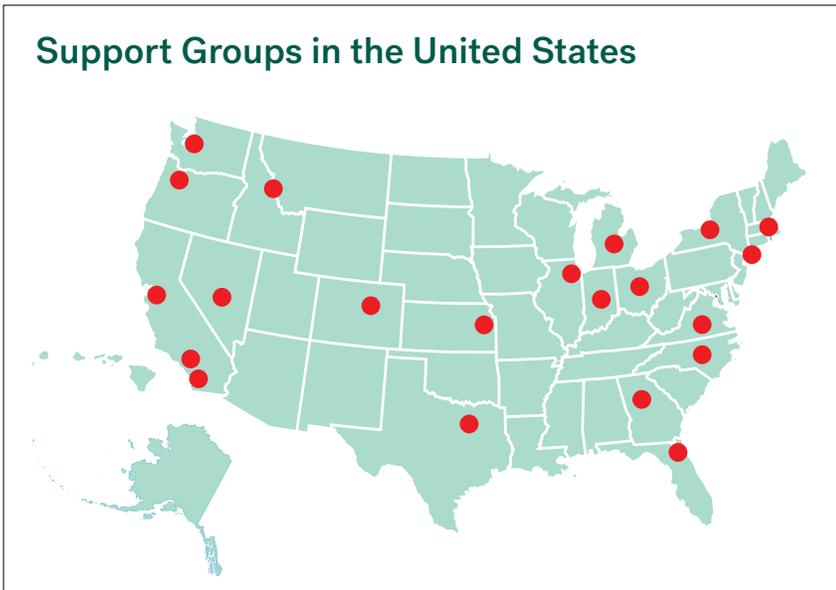
In January 2021, Support Group Facilitator, Mindy Kim (below right, with her parents), started hosting a weekly Friday “DM Community Happy Hour” over Zoom. It’s been a hit amongst the community, with the recurring event scheduled every Friday through 2022 and beyond!



**“My support group facilitator was the first person to truly understand what I was going through. I couldn’t have braved my diagnosis without them.”**

— Support Group Member

## Support Groups in the United States



### Support Group Facilitators

Our Support Group Facilitators are the leaders and pillars of our community. We can’t thank them enough for the countless hours, the lives they’ve changed for the better, and the incredible example of courage and positivity that they set!

- Rob Besecker, Chicago, IL, USA
- Teresa Buffone, Ottawa, Canada (retired 2021)
- John Cooley, Kansas City Region, USA
- Mark Coplin, Portland, OR, USA
- Teresa Cummings, AZ, USA\*
- Jeannine DeSoi, Boston, MA, USA\*
- Loraine Dressler, Orange County, CA, USA & Parents/Guardians of Children with DM
- Sarah Francheteau-Berman, Parents/Guardians of Children with DM\*
- Jonathan Freedman, Seattle, WA, USA
- Patricia Gibbs, Kansas City Region, USA
- Kay Hayes, Denver, CO, USA
- Jodie Howell, VA, USA\*
- Cindy Hubert, Seattle, WA, USA
- Chuck Hunt, Atlanta, GA, USA
- Janis Jaffe, New York City, NY, USA\*
- Emily Jones, Rochester, NY, USA
- Rashid Kassir, San Diego, CA, USA\*
- Mindy Kim, NC, USA & Facebook Moderator
- Anke Klein, International German Speakers
- Leslie Krongold, EdD, Adults 50+ living with DM1 & Healthy Living Discussion group
- Carrie Lahnovyvh, Rochester, NY, USA
- Alexandra LeBoeuf, Ontario, Canada
- Julie LeBoeuf, Ontario, Canada
- Haley Martinelli, DM2 Virtual Support Group
- Kristen McClintock, Jacksonville, FL, USA
- Kim McPeek, DM2 Caregivers Virtual Support Group
- Tom McPeek, DM2 Virtual Support Group
- Sherry Morris, Dallas, TX, USA
- Bill Nutall, Boston, MA, USA\* & Facebook Moderator
- Suzanne Perkins, MI, USA
- Bernhard Rogg, International German Speakers
- Ted Salwin, Indianapolis, IN, USA & Caregivers Support Group
- Carolyn Valek, Worthington, OH, USA & JOA Warriors
- Scott Virgo, MI, USA
- Patrick Welker, Dallas, TX, USA
- Samantha Welsh, VA, USA\* & Facebook Moderator
- Glenda Winson, New York City, NY, USA\*
- Ann Woodbury, Mountain West Region, USA & JOA Warriors
- Guillermo Zubillaga, New York City, NY, USA\*

\* New in 2022



# Care

*MDF provides critical resources and advocates for improved 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. While we work to increase DM education and awareness amongst clinical care teams, MDF seeks to empower individuals and families living with DM to be their own medical experts and advocates.

We've created a wealth of educational materials and resources for individuals, families, and clinicians, all accessible on our website.

## Did You Know?

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

## Empowering the DM Community through Education

### Warmline

MDF staff engages with newly diagnosed patients and others trying to navigate this complex disease on a daily basis. The team receives over **500 Warmline calls** and email each year with requests for resources, support, clinical care referrals, and education. Phone: **415-800-7777** or email [info@myotonic.org](mailto:info@myotonic.org).

### Digital Academy

The MDF Digital Academy is the largest DM-focused digital library the world, housing **170 videos and 130+ hours of content** about DM, ranging from educational webinars to conference archives to family stories, with topics ranging from disease understanding and the state of research, to symptom management and community interviews.

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

NEW IN  
2021!

### Ask the DM Expert

MDF has created a monthly webinar series called "Ask-the-DM-Expert," which connects families and the world's leading DM experts. The sessions feature lectures on DM-related topics and then open the floor up to participants to ask these experts their toughest questions in real time. Past session topics available to view now include: heart, lung, GI, intimacy, pain, palliative care, exercise, brain, speech, anxiety, and more. More than **4,000 attendees/views** since the program launched in 2021.

[www.myotonic.org/ask-expert-series](http://www.myotonic.org/ask-expert-series)

*"There are few opportunities to access these highly sought-after specialists.*

*This one is great, and free!"* – DM Community Member

NEW IN  
2021!

### Meet the DM Drug Developers

In this monthly webinar series, a different bio-technology and pharmaceutical partner, who is currently developing treatments for myotonic dystrophy, sits down with our community to share their progress and answer their questions in real time. This provides affected individuals, families, and caregivers with direct access to leaders at companies developing treatments. More than **5,000 attendees/views** since program launched in 2021.

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

## MDF's Myotonic Dystrophy Publications and Resources

With the help of the leading DM experts from around the world, MDF has developed over 24 publications on various topics in 9 languages for individuals living with DM and their clinical care teams. These publications have helped individuals and their clinicians understand the disease and the many challenges of living with DM, improve quality of care, and in some cases even save lives.

### PUBLICATION SPOTLIGHT

#### Our Standout Toolkits and Guidelines

- MDF Toolkit in 5 languages
- Clinical Care Recommendations for Adults with Myotonic Dystrophy Type 1 and Type 2
- Anesthesia Guidelines
- Respiratory Care Recommendations for Myotonic Dystrophy Patients During the COVID19 Pandemic

See the complete list of publications at [www.myotonic.org/toolkits-publications](http://www.myotonic.org/toolkits-publications)

*“I wanted to say thank you all for all of the new guidelines on your main website. It has been so helpful for me to gain more knowledge as my son and husband were diagnosed with type 1 this past year. It looks like a lot of work went into creating them.”*

— DM Community Member



**Hundreds** of clinical collaborators

**24** publications on the website

**8** NEW/updated publications in 2021 alone

**9** languages

**8,000+** visitors explored the MDF Body Systems Tool in 2021

**57,000** downloads of the MDF Toolkit and other online resources in 2021

## Find a Doctor

MDF's Find-A-Doctor map helps affected individuals, families, and caregivers connect with DM doctors and healthcare professionals in their local geographic area. Hundreds of clinicians can be found in locations around the globe.

[www.myotonic.org/find-a-doctor-map](http://www.myotonic.org/find-a-doctor-map)



# Cure

## *MDF is eliminating barriers to accelerate research toward treatments and a cure*

WHILE THERE ARE CURRENTLY no FDA approved treatments or cures for myotonic dystrophy, for the last seven years MDF has worked collaboratively with the MDF UK Board of Trustees to ensure our strategies to eliminate barriers are aligned, do not duplicate effort, and maximize our impact. Together, we have accelerated DM research and drug development by thoughtful investments in research, researchers, and initiatives designed to attract further investment from industry and the US government. We also help connect individuals living with DM to opportunities to participate in research, to better understand the disease and to progress towards treatments and a cure.

Thank you to our incredible community members, who have been active partners in bringing the research to this point, by supporting and participating in studies, joining registries, responding to surveys, and funding patient advocacy organizations like the MDF. The progress achieved would not be possible without the commitment and participation of people living with DM, their families, caregivers and friends.

### Did You Know?

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.

*“The investments MDF has made in DM research have changed the landscape of the field. Their support has directly influenced my career and allowed me to advance new research on DM.”*

— DM Researcher

### Research Highlights

- Over the last 7 years, MDF invested more than **\$6MM** in a drug development acceleration effort including 15 major initiatives designed to attract more industry investment, lower barriers to therapy development, and expand the amount of data available to drive discovery.
- MDF’s efforts have helped attract **more than 50 companies** and institutions into DM drug development with three new therapies entering clinical trials in 2021.
- MDF maintains the Myotonic Dystrophy Family Registry (MDFR), the largest DM-only patient registry in the world to collect data needed for better disease understanding and ensure that the community is organized and trial-ready.
- MDF’s virtual Study & Trial Resource Center informs, educates, and prepares the DM community for participation in DM studies.



In 2021, three drug therapies for DM entered Phase I/II clinical trials — a milestone for the DM community! Many MDF community members are involved in these historic trials.

Participate in an ongoing myotonic dystrophy study or trial at [www.myotonic.org/study-trial-resource-center](http://www.myotonic.org/study-trial-resource-center)



## MDF Research Fellows

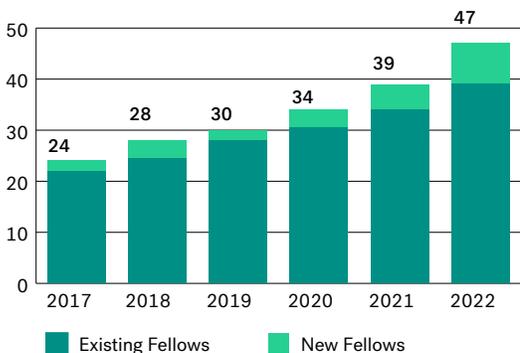
*MDF is developing the next generation of DM researchers and thought leaders!*

Since 2008, MDF has committed over **\$4MM** in total research funding to **47 fellows from 17 different distinguished institutions** in five countries. The Myotonic Dystrophy Foundation has provided two-year pre- and post-doctoral research fellowships to support new and innovative studies relevant to myotonic dystrophy. Through this program, the MDF invests in up-and-coming pre- and postdoctoral fellows to expand the base of committed DM researchers.

Over **90%** of former fellows have remained in the DM research field after completing their fellowships and cited that they chose to continue in the field due to their interaction with the community, interest in the disease, and the research skills they gained.

Together they have contributed to over **70** peer-reviewed publications now advancing DM academic knowledge and research.

### MDF Research Fellows



## Fund a Fellow

Help Fund the Future of DM Research! If you would like to help support the development of new DM researchers, the advancement of DM academic research and discoveries, and to provide hope for the DM community, please consider investing in our Fund-A-Fellow program!

Visit this site for more details:  
[www.myotonic.org/fellows](http://www.myotonic.org/fellows)

## Meet our 2021 Research Fellows!



**Kamyra Simone Edokpolor**  
*Emory University School of Medicine  
Atlanta, Georgia, US*

Kamyra Simone Edokpolor is a doctoral student whose research has the potential to inform therapeutic strategies for CNS symptoms in DM1. She is currently testing her hypotheses in mouse models and has found promising preliminary data.



**Maya Gosztyla**  
*University of California  
San Diego, California, US*

Maya Gosztyla is a doctoral student researching RNA-binding Proteins and RNA Localization in cDM1 Organoids. She is a co-author for an article recently published in *Nature Neuroscience*.



**Rong-Chi Hu**  
*Baylor College of Medicine  
Houston, Texas, US*

Rong-Chi Hu is a doctoral student whose research seeks to understand the mechanisms of the DM1 cardiac progression, as well as explore potential therapeutics.



**Benjamin M. Kidd**  
*University of Florida  
Gainesville, Florida, US*

Benjamin M. Kidd is a doctoral student focusing on identifying when DM affects the brain to support the use of potential therapeutics.



**Subodh Kumar Mishra, PhD**  
*The RNA Institute  
University of Albany, New York, US*

Dr. Subodh Kumar Mishra is a postdoctoral scholar researching potential dietary compounds that are part of natural sources that could have therapeutic impacts. Dr. Mishra presented the results of his research at several DM-related conferences.



## MDF Research Grants Move the DM Field Forward

More than **\$5.5MM** across **19** research grants has been awarded since 2015.

In 2022 MDF will award at least **2** NEW Early Career Research Grants totaling over **\$400,000** to help ensure DM experts stay focused on DM research!

### Example MDF-funded Research Grants

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

Project	Description
Natural History Data Capture	Set-up of the Myotonic Dystrophy Clinical Research Network (DMCRN); follow DM patients to understand disease progression, which will help validate endpoints and inclusion criteria for therapeutic trials.
Reach DM: Study to Promote Trial Readiness by Genetic Analysis and Telemedicine Assessments	This study of DM1-affected individuals in the MDF and National Registries seeks to determine feasibility for remote genetic testing and disease assessments.
Mouse Testing Facility	Jackson Laboratory positioned to conduct drug efficacy trials using HSA-LR mice (genetically altered to exhibit characteristics of DM).
Burden of Disease Study	Review of 785 DM patient records to determine the costs of disease diagnosis and progression; supports efforts to ensure that more patient costs are covered by insurance companies.
Induced Pluripotent Patient-derived Stem Cell Line (iPSC) Library	Creation of iPSCs from seven subjects for DM1 and DM2; available to private, public, and academic DM researchers and drug developers.
Population Based Prevalence Study	Random capture from 50,500 babies' heels to better understand true prevalence of DM; supports inclusion of DM on screening panels and will generate more attention from drug developers.

## Living with Myotonic Dystrophy and want to help?

The Myotonic Dystrophy Family Registry (MDFR) is the largest DM-only patient registry in the world. Over 2,400 members have registered. By joining the Myotonic Dystrophy Family Registry, you will aid researchers, pharmaceutical companies and other professionals seeking to:

- Identify participants for clinical trials and research studies
- Develop new, effective treatments for those living with DM
- Advocate on behalf of the DM community with decision makers in Washington, D.C. and elsewhere

Your participation is voluntary and your individual information will be kept completely confidential. The more participants and data we have in the Registry, the more significant its impact, and size is critically important to helping support clinical trial and research study recruitment.

For more information, visit [www.myotonic.org/registry](http://www.myotonic.org/registry)



## Myotonic Dystrophy Research Map

MDF is proud to serve as the nexus of care, research and drug development within the myotonic dystrophy community. To increase collaboration and general knowledge across the DM ecosystem, we have compiled publicly available research-related information on myotonic dystrophy into a single comprehensive database. To capture the interconnected nature of progress in the DM research field, we have illustrated the data in a visual network map — an intuitive, holistic, and explorable knowledge-building tool built for professionals and community members alike.

This project was designed to support, supplement, and streamline the work of researching and developing treatments for DM. The map represents the most current and relevant published knowledge of the DM research community across the world, and its inter-connectedness. MDF is delighted to connect the following entities within the DM research and drug development ecosystem:

- Researchers & Research Institutions
- DM Drug Developers and Biotech and Pharmaceutical Companies
- DM Studies, Trials & Publications
- DM Research Tools & Drug Therapies in Development

To learn more about the map, visit: [www.myotonic.org/DMmap](http://www.myotonic.org/DMmap)

An interactive, visual database of the current DM research ecosystem compiled by MDF

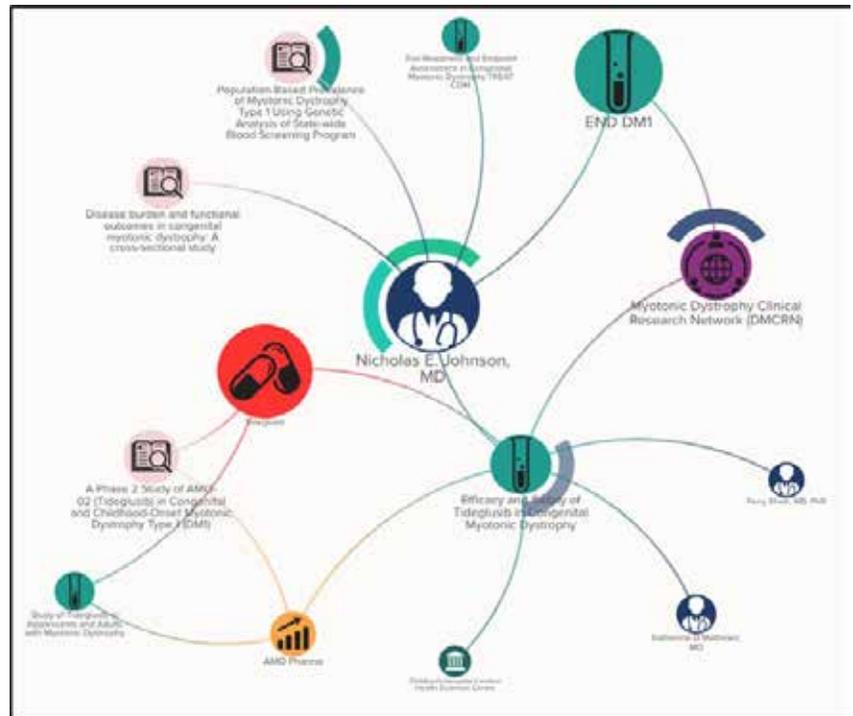
8,000+ Elements

16,000+ Connections

68,000+ Datapoints

To support, supplement & streamline the work of researching and developing treatments for DM.

## DM Research Map Dataset sample



## Research Funding Spotlight: DMCRN

Since 2012, MDF has helped expand the DM clinical trial infrastructure by co-funding the first-ever Myotonic Dystrophy Clinical Research Network (DMCRN), a network of 16 medical centers across the world with significant proficiency in myotonic dystrophy clinical care and research.

The DMCRN has totally changed the DM research landscape. The number of researchers in this remarkable collaborative network have free and unrestricted access to the data collected through DMCRN studies, and can publish the results of these studies. In the last 10 years, dozens of studies and publications have resulted from the collaboration of researchers in the DMCRN, dramatically lowering barriers to advancing DM science and research.

*DMCRN Principal Investigators:*

Nicholas Johnson, MD, *Virginia Commonwealth University*  
Charles Thornton, MD, *University of Rochester*

Participate in a study or trial at

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

# Advocacy

OVER THE PAST 15 YEARS, MDF Advocates have dramatically changed the myotonic dystrophy research and drug development landscape.

MDF has secured over \$8 million in funding under the Peer Reviewed Medical Research Program (PRMRP) over the past 5 years.

- DM is now included in the list of over 50 conditions eligible for research funding from the Department of Defense.
- Seeking Congressional support for DM eligibility for 6th year in a row.

MDF is also requesting \$10 million in new Congressionally Directed Medical Research Programs (CDMRP) research funding in FY23 budget.

Learn how to Advocate for the DM Community at [www.myotonic.org/advocate](http://www.myotonic.org/advocate)

## Advocacy Highlights

- In 2021, MDF helped to lead the efforts to declare and honor the very first International Myotonic Dystrophy Awareness Day on September 15th, and gathered the Global Alliance for Myotonic Dystrophy Awareness, an alliance of over 55 DM-focused organizations worldwide dedicated to increasing DM awareness.
- In 2020, MDF successfully advocated for the passage of a congressional bill that directs the US National Institutes of Health to make recruitment of new DM researchers a high priority.
- MDF's advocacy efforts for additional DM research funding at the National Institutes of Health and the Department of Defense has led to \$8.1 million in new government funding since 2018.
- With the help of DM medical professional partners, MDF advocated to professional medical associations worldwide to share DM Care Guidelines with their members.
- MDF assembled an all-new National Advocacy Committee consisting of board members and community volunteers to increase MDF's advocacy efforts in 2022 and beyond.
- MDF is currently working to ensure Fiscal Year 2023 Labor Health and Human Services Appropriations Report Includes NIH Repeat Expansion Diseases Initiative (REDI) Report Language.

## A Few of MDF's Historical Advocacy Achievements



**2014**  
Kayla Vittek and her mom Lisa Harvey testified before Congress in support of reauthorization of the MD-CARE Act.



**2015**  
MDF Annual Meeting in Washington, DC features congressional briefing on DM Research Funding.



**2016**  
MDF hosts Patient-Focused Drug Development meeting, with FDA senior leadership, to highlight perspectives of patients and caregivers to accelerate DM treatments and a cure.



**2017**  
MDF convinces Social Security to add Congenital DM to Compassionate Allowance Program, enabling individuals to quickly qualify for disability benefits including health insurance coverage.

Myotonic Dystrophy Voice of the Patient report delivered to FDA

## 2021-2024 Advocacy Strategies

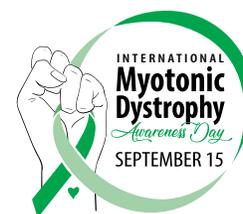
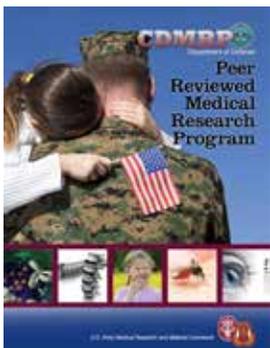
- Speak with a louder voice in Congress and State Capitals
  - Launch National Advocacy Committee
  - Recruit more individuals, families and friends to support increased research funding
- Increase congressional understanding of DM
  - Assist MDF advocates in building relationships with members of Congress and staff
  - New advocates and relationships mean increased Federal funding for DM research to accelerate new treatments and a cure
- Grow the Global Alliance to advance international myotonic dystrophy awareness and action



### Thank you

for co-sponsoring SR 336  
and SR 772!

- Sen. Tim Kaine (D-Virginia)
- Sen. Amy Klobuchar (D-Minnesota)
- Sen. Tina Smith (D-Minnesota)
- Sen. Cynthia Lummis (R-Wyoming)
- Sen. Susan Collins (R-Maine)



### 2018

US Senate adds DM to list of eligible conditions for research funding under the Department of Defense Peer Reviewed Medical Research Program (PRMRP),

**\$3.1MM PRMRP**

### 2019

Tim Haylon testified before the House Appropriations Committee to increase federal funding for DM research at the National Institutes of Health (NIH).

**\$2.4MM PRMRP**

### 2020

DM Research Provision in House Appropriations Report: "...support current efforts to develop the first ever FDA approved treatment for this inherited genetic disorder."

**\$2.3MM PRMRP**

### 2021

Senator Tim Kaine introduces Senate Resolution 336, declaring September 15 International Myotonic Dystrophy Awareness Day!

**\$300K PRMRP**

# Fundraising

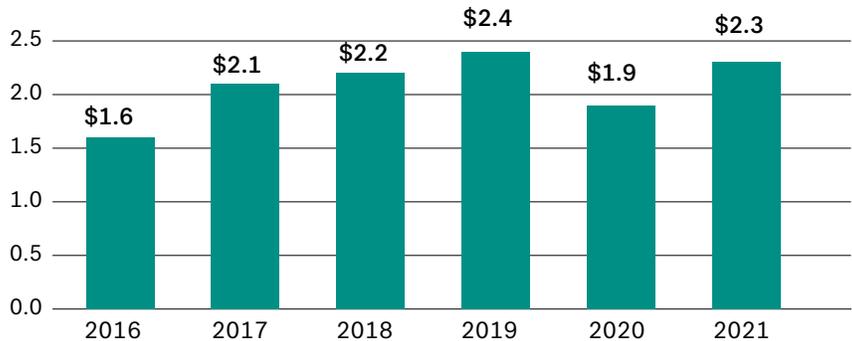
OUR DONORS AND GRASSROOTS ORGANIZERS are the reason MDF can do the critical work of supporting and connecting the myotonic dystrophy community, providing resources and advocating for care, and accelerating research toward treatments and a cure. Our deepest thanks to all who donated in 2021—we couldn't do this without you!

Call the office and speak with the MDF team or explore our fundraising toolkit at [www.myotonic.org/grassroots](http://www.myotonic.org/grassroots)

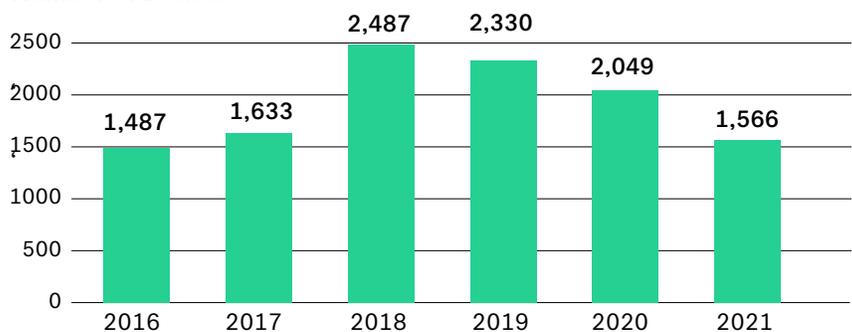
## MDF Donor Contributions

Donation dollars continue to trend higher for MDF, allowing us to invest further in our mission, yet the number of unique donors has fallen during the pandemic.

### Donation Dollars



### Number of Donors



## 2021 Virtual Gala



Our Annual Gala is our largest fundraising event of the year. The 2021 Virtual Gala is the biggest success we've had yet, raising over **\$1MM** to help fund critical programs and services for individuals and families living with myotonic dystrophy! A special thank you to all of our Gala Donors and Sponsors. We are truly in awe of your incredible generosity and support!

We would like to extend a huge thank you to our incredible Co-Chairs of the 2021 Virtual Gala: Martha Montag Brown, Erica Kelly, Leslie Lynch & Elizabeth Florence!

With the assistance of our MDF team, these passionate women were able to organize and execute our most successful fundraising event in MDF history—and all through the uncertainty of the COVID-19 pandemic! See more at [www.myotonic.org/gala](http://www.myotonic.org/gala)

### Did You Know?

Other names for myotonic dystrophy include myotonic muscular dystrophy (MMD) & Steinert's Disease for DM1 and Proximal myotonic myopathy (PROMM) for DM2.

## Grassroots Fundraisers

Community-led grassroots fundraisers are a crucial way our community members contribute to our mission of Community, Care, and a Cure. MDF provides assistance, tools, and starter kits to help our grassroots fundraisers reach their goals and have enormous impact on the lives of those living with DM! Learn more here: [www.myotonic.org/grassroots](http://www.myotonic.org/grassroots)

### Jim Dvorak — Swimming for a Cure!

After Heather and Marie Dvorak were both diagnosed with DM, Jim Dvorak hatched a plan to partner with his friend and neighbor, Andrew Hopkins, to compete in the Swim Around Lido Key race sponsored by Swim Without Limits, a 7-mile open water relay swim. Though the race was postponed, they raised double their original goal, raising almost \$20,000 in donations to MDF. Congratulations and thank you to Jim, Andrew, Heather, Marie, and all other supporters!



### Andrew Gulch Memorial Golf Outing

The Andrew Gulch Memorial Golf Outing is a hockey themed golf outing that is held annually in the Toledo area, on the last Saturday before the start of the College Football Season. The event is in memory of the Gulch family's son, Andrew, who was born with myotonic dystrophy and passed away in 2001. In 2021 they had a blast and raised over \$14,000. Since 2011, the Gulch family and friends have raised over \$125,000 to benefit research efforts of numerous muscle diseases! Congratulations and thank you to the Gulch and Beucler families, and all other supporters!



## Donor Spotlight The Klein Family

Les and Sheila Klein, and their children Adina and Jason, have been involved with MDF's research efforts for several years. The family's diagnostic odyssey began when Jason was only 25, as he sat down to lunch with his rabbi and began to share about how his hands and tongue had begun to "freeze" randomly. The rabbi recommended a neurologist, who soon diagnosed Jason with DM1. His sister Adina was also diagnosed with DM1, only a year later. Wanting the absolute best for her children, Sheila immediately began a comprehensive search to find the best care, treatment, and support for their children.

More than a decade later, still with no treatment in sight, the Kleins became determined to do more to change the future of the disease. Jason met with MDF's CEO right before the pandemic arrived to learn more about how his family might help move the mission forward. Since then, the Kleins have been actively involved with MDF.

At the end of 2021, MDF was selecting our 2022 Research Fellows from the most impressive applicant pool in the program's history. MDF had never before funded eight new fellows in one year, and the \$600,000 it would require hadn't been written into the operating budget for that year.

When the Klein family learned of MDF's need to support eight fellows to ensure they could remain focused on DM research, they stepped up to bridge a large funding gap. Thanks to the Kleins, and the few other families who answered this call, MDF invited all eight researchers to begin MDF Research Fellowships in 2022, and all eight accepted!

We are grateful for the Kleins for their crucial and ongoing support of MDF's mission!





# Myotonic Dystrophy FOUNDATION

663 Thirteenth Street, Suite 100

Oakland, CA 94612

[info@myotonic.org](mailto:info@myotonic.org)

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

## Ways to Give



### ONLINE

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



### MAIL

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

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



### PHONE

415-800-7777

The Myotonic Dystrophy Foundation (MDF), a nonprofit public charity, focuses 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. Your donations fund and support important research efforts, awareness raising activities, and advocacy meetings with legislators and federal agencies.

We are a 501(c)(3) nonprofit organization. All donations are tax-deductible to the fullest 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 ID is: 20-5014628.

**Thank you for your generous contributions.  
We couldn't do it without you.**