Kansas City, Missouri

2026 MDF Regional Conference

April 11th

A One-Day Event to Unite
Community, Care, and a Cure for
Myotonic Dystrophy

Myotonic Dystrophy FOUNDATION

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The University of Kansas

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Myotonic Dystrophy Foundation

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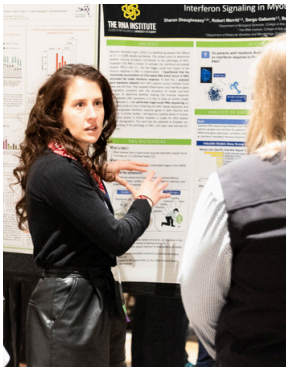
Myotonic Dystrophy FOUNDATION

2026 MDF Regional Conference

April 11th in Kansas City, Missouri



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.

Welcome Letter from the CEO

Welcome to the 2026 MDF Regional Conference in Kansas City, MO!

On behalf of the Board of Directors and staff of the Myotonic Dystrophy Foundation (MDF), welcome to the 2026 MDF Regional Conference in Kansas City, Missouri! We're honored to gather in our 18th year of conferences, bringing together over 70 community members from 12 states. Your presence strengthens and unites this community.

I extend my sincere appreciation to everyone who helped make this conference possible - especially our partners at the University of Kansas Medical Center for hosting us today - and the many volunteers, advocates, and supporters who continue to move this mission forward.

MDF Regional Conferences are designed to bring our community closer together—creating space to share experiences, connect with clinicians and researchers, and learn about progress toward treatments and a cure. Since launching this regional format in 2024, we've seen the power of gathering locally: making it easier for families to participate, ask questions, and engage in meaningful conversations. This year's program reflects your input, with sessions tailored to meet the diverse needs of our community.

Today, there is real reason for hope. As we move closer than ever to the first approved treatments for myotonic dystrophy, your involvement is more important than ever. We encourage you to join the Myotonic Dystrophy Family Registry (MDFR) to help advance research and stay informed about new opportunities. Please stop by the registry table today to talk with Dr. Olmos about the benefits of participating in the MDFR.

We also invite you to stay connected beyond today and take time to review our events calendar and resources on our newly designed website! This July, we'll celebrate Myotonic Dystrophy In Motion Month—a time dedicated to movement, wellness, and community engagement, with activities for everyone: www.myotonic.org/in-motion. We hope you will join in!

Thank you for being here, for supporting one another, and for being part of the progress ahead. If there's anything we can do to enhance your experience today, please don't hesitate to reach out.

Enjoy the conference—we're glad you're here.

Tanya Stevenson, EdD, MPH
Chief Executive Officer

Agenda for Saturday, April 11th

Time	Seville Ballroom	Roanoke	Rockhill	Union Hill	JOA Lounge Brookside
8:30 - 10:00 AM	Check In Opens				
9:00 - 10:00 AM	Breakfast				
10:00 - 10:45 AM	Welcome & Introduction MDF Staff Dr. Jeffrey Statland Dr. Constantine Farmakidis				
10: 45 - 11:00 AM	Break				
11:00 - 11:45 AM	Clinical Trials & Emerging Treatments Dr. Jeffrey Statland	Community Panel: Life Hacks			Getting to Know You & Ice-Breaker Games
11:45 - 12:00 PM	Break				
12:00 - 12:45 PM	DM1: What You Need to Know Dr. Constantine Farmakidis	DM2: What You Need to Know Dr. Hussien Alkully	Coping with Chronic Stress in DM Maria Arvanitakis	Genetic Testing, Counseling, & Family Planning Juliana Shapiro	Navigating Anxiety & Depression Emily Rosenbaum
12:45 - 1:45 PM	Lunch				
1:45 - 2:30 PM	Keeping Your Heart in Check: DM's Impact & Care Tips Dr. Pradeep Mammen	Managing Sleep & Pulmonary Health with DM Dr. Damien Stevens	Whole Health: Palliative Care & DM Dr. Taylor Jersak		Let's Get Moving: Exercise & DM Dr. Kristina Kelly
2:30 - 2:45 PM	Break				
2:45 - 3:00 PM	Movement Moment				
3:00 - 4:00 PM	Industry Updates				Stump the Doctor Dr. Jeffrey Statland
4:00 - 4:15 PM	Break				
4:15 - 5:00 PM	Stump the Doctor: DM1 & DM2 KUMC Panel				Karaoke & Games
5:30 - 7:00 PM	Reception, Networking, & Dance Party				

General Sessions

General sessions are for everyone! All general sessions are located in the Seville Ballroom.

10:00 - 10:45 AM

Welcome and Introduction to DM Resources

The Myotonic Dystrophy Foundation & the University of Kansas Medical Center

MDF works to enhance the quality of life of people living with DM and to drive research focused on treatments and a cure. This session will provide an overview of the day, a summary of the work of MDF, as well as the readily available tools and resources for the community, and a feature on the University of Kansas Medical Center and their work in the DM space.

11:00 - 11:45 AM

Clinical Trials and Emerging Treatments

Jeffrey Statland, MD

Professor, University of Kansas Medical Center

As academic and industry partners work to develop treatments and a cure for myotonic dystrophy (DM), it is more important now than ever that the DM community is ready to participate in trials. This talk will provide an overview of clinical trials and help attendees think about how to prepare for upcoming trials and studies.

2:45 - 3:00 PM

Movement Moment

Kris Kelly, PT, DPT, MS, EdM, NCS, CPT, PES, Assistant Research Professor

Department of Physical Medicine & Rehabilitation, University of Missouri

This movement moment will provide a short, interactive movement demonstration for all conference attendees to gently get moving, stretch, and break up a full day of sessions.

3:00 - 4:00 PM

Industry Updates

Representatives from biotechnology and pharmaceutical companies will provide updates on their drug development efforts in the DM field.

4:15 - 5:00 PM

Stump the Doctor: DM1 & DM2

Hussien Alkully, MD; Constantine Farmakidis, MD; Jeffrey Statland, MD

University of Kansas Medical Center

Stump the Doctor is an opportunity for community members to meet a leading expert in myotonic dystrophy and ask their most persistent challenging questions. Can you stump the doctor? *Note, this session will not be recorded.*

Breakout Sessions

Breakout Sessions at these Regional Conferences serve a variety of community interests and needs. They have been designed with community input and have a universal focus for all people affected by DM1 or DM2 and their families.



11:00 - 11:45 AM *(Roanoke)*

DM Life Hacks Community Panel

John Cooley, Joe Gibson, & Tamara Greer

Join MDF volunteer community leaders in a discussion on everyday victories through useful tips, tricks, and adaptive devices.

Please note, this session will not be recorded.

12:00 - 12:45 PM *(Seville Ballroom)*

DM1: What You Need to Know

Constantine Farmakidis, MD
Co-Director Muscular Dystrophy Clinic, Associate Professor,
University of Kansas Medical Center

This session is for those new to the DM1 community and those seeking a refresher. Join for an overview of DM1 including genetics, symptoms, self-management, and working with your clinical care team to ensure the best quality of life.

12:00 - 12:45 PM *(Roanoke)*

DM2: What You Need to Know

Hussien Alkully, MD
Neuromuscular Fellow, Kansas University Medical Center

This session is for those new to the DM2 community and those seeking a refresher. Join for an overview of DM2 including genetics, symptoms, self-management, and working with your clinical care team to ensure the best quality of life.

12:00 - 12:45 PM *(Rockhill)*

Coping with Chronic Stress in DM

Maria Arvanitakis, MS, LPC, CRC
Facilitator, Turning Point, University of Kansas Health System

Join this session for an overview of chronic stress and its potential impact on individuals living with DM. Learn practical strategies for recognizing stress, building resilience, and developing healthy coping techniques to support emotional well-being in daily life.

Breakout Sessions

Breakout Sessions at these Regional Conferences serve a variety of community interests and needs. They have been designed with community input and have a universal focus for all people affected by DM1 or DM2 and their families.

12:00 - 12:45 PM *(Union Hill)*

Genetic Testing, Counseling, & Family Planning in DM

Juliana Shapiro (Askren), MS, CGC
Genetic Counselor, University of Kansas Health System

Join this session to learn about genetic counseling and family planning and myotonic dystrophy. You'll get more information on what a genetic counselor is, how to get tested, and planning for the future.

1:45 - 2:30 PM *(Seville Ballroom)*

Keeping Your Heart in Check: DM's Impact & Care Tips

Pradeep P.A. Mammen, MD, FACC, FAHA, FHFSA
Division Chief: Advanced Heart Failure Therapeutics & Cardiac Transplantation, Vice Chair for Translational Research, Medical Director: KU Inherited Cardiomyopathy Clinic, Maureen & Marvin Dunn Professor in Cardiovascular Disease, Professor of Cardiovascular Medicine, KUMC

This session covers cardiac considerations in myotonic dystrophy including how DM can affect the heart, common issues, the latest research, and best practices for care.

1:45 - 2:30 PM *(Roanoke)*

Managing Sleep & Pulmonary Health with DM

Damien Stevens, MD, MS
Associate Professor of Medicine, Medical Director of Sleep Laboratory, KUMC

This session will explore the relationship between myotonic dystrophy and sleep and breathing issues, covering prevalence, impact, and emerging research. Gain practical insights and best practices for managing sleep and breathing issues in both DM1 and DM2, empowering better care and self-management.

1:45 - 2:30 PM *(Rockhill)*

Whole Health: Palliative Care & DM

Taylor H. Jersak, MD, FAAP
Assistant Professor of Palliative Medicine, Children's Mercy Kansas City, University of Kansas Health System

Join this session to gain valuable insights into how palliative care can support physical, emotional, and practical well-being at every stage of the DM journey.

Juvenile-onset Adult (JOA) Sessions

Juvenile-onset Myotonic Dystrophy is often defined as someone living with DM1 whose symptoms manifested in childhood or early adulthood, usually before age 21. These sessions are designed with community input from juvenile-onset adults (JOA) to provide networking, connection, and resource opportunities for this unique community. All JOA sessions are located in the Brookside Room.

Please note that these sessions are not recorded and are only intended for JOA individuals. All other attendees should participate in other sessions.

11:00 - 11:45 AM

Getting to Know You & Ice-Breaker Games

Lucie Shiffman, Senior Manager of Community Engagement, MDF

Let's get to know each other with some awesome team building games. We will work together to list the group rules for the JOA Lounge, which will be open to throughout the day so plan to stop by & chill out whenever you need to relax!

12:00 - 12:45 PM

Let's Talk About Feelings: Navigating Anxiety & Depression

Emily H. Rosenbaum, MA, LPCC, LCPC, LPC, Hartstone Collaborative

Join this session to explore depression and anxiety in the context DM. This interactive session will include an overview of key concepts, opportunities for discussion and reflection, and hands-on exercises to build practical coping skills.

1:45 - 2:30 PM

Let's Get Moving: Exercise & DM

Kris Kelly, PT, DPT, MS, EdM, NCS, CPT, PES, Assistant Research Professor
Department of Physical Medicine & Rehabilitation, University of Missouri

This session is a great opportunity to learn about exercise & DM and move. This movement session is for all levels and experiences.

3:00 - 4:00 PM

Stump the Doctor: JOA

Jeffrey Statland, MD
Professor, University of Kansas Medical Center

An opportunity for community members to meet a leading DM expert and ask their most persistent and challenging questions. Can you stump the doctor?

4:15 - 5:00 PM

JOA Games & Karaoke

Lucie Shiffman, Senior Manager of Community Engagement, MDF

Join the final JOA specific session of the day for fun activities and karaoke! Learn new games or share your favorites with the group. Don't miss this chance to strengthen your connection with the JOA Community!

Myotonic Dystrophy at a Glance

Myotonic dystrophy is the most common form of adult muscular dystrophy and considered the most variable of all known conditions. Do your part to help start conversations and educate your family, friends, and care providers about the basics of myotonic dystrophy (DM).

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

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

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

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

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

Myotonic dystrophy 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.

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

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

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

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.

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

Learn more at www.myotonic.org/at-a-glance

MDF Resources for Individuals Affected by DM

These publications are for a general audience to help understand the condition and various aspects of life with DM. You can access them all for free on the MDF website or email us at info@myotonic.org to get a hard copy mailed to you.

*An * indicates this resource is available in more than one language.*

Practical Advice for Anesthesia for Individuals with DM & their Families*

People living with DM may have severe and life-threatening reactions to anesthesia. This resource equips individuals living with DM and their families with crucial information to help navigate the anesthesia process.

Mental Health Handbook*

This resource highlights the social and emotional impact of DM, offering an overview of potential mental health challenges and available supports.

Myotonic Dystrophy & the Heart: A Community Guide*

For individuals living with myotonic dystrophy, heart or "cardiac" issues can pose a serious threat to their health. This resource aims to help people living with DM understand heart health risks and how they are managed.

Medical Alert Card*

Patients with myotonic dystrophy often exhibit adverse reactions to sedatives, anesthetics, and neuromuscular blocking agents. Ensure your emergency responders follow these critical guidelines.

My Clinic Visit Planner*

This planner enables families to discuss upcoming clinic visits and jot down important questions and information to help ensure it is covered at the appointment.

Going to School with DM: A Guide to Understanding Special Education and IDEA

This comprehensive resource helps parents and family members take advantage of the Individuals with Disabilities Act (IDEA).The Guide covers services and mandates for ages newborn through 21.

Exercise Guide & Exercise Infographic for Individuals with Myotonic Dystrophy*

This guide includes information on the benefits of exercise for DM, recommendations on aerobic activity, types of exercise, monitoring exercise, exercise strategies & finding motivation.

Health Insurance Considerations for People Living with Myotonic Dystrophy in the US

MDF created this resource to help you navigate the process of making sure your medical treatments and medications are covered, and how to appeal your claim if it is denied.

Nutrition Guide for Individuals with Myotonic Dystrophy*

Developed by nutritionists experienced with DM, this guide covers information on diet and DM, managing constipation, being overweight/underweight with DM, managing swallowing problems, feeding tubes, supplements, and sample meals.

Guide for Adults Affected by Juvenile-onset (JOA) DM & their Caregivers

This Guide is specifically designed to help families affected by juvenile-onset DM understand how to manage their lives, especially at transition points in development and education.

Applying for Social Security Disability Benefits Toolkit

The toolkit is designed to assist those affected by myotonic dystrophy in navigating the application process for Social Security Disability Insurance (SSDI) benefits and Supplemental Security Income (SSI) benefits. (US Health System only)

Employment Access Toolkit*

MDF created this Toolkit to help individuals navigate employment, covering how DM may affect work, job readiness, applications, resumes, interviews, and more (U.S. job system only).

Learn more at www.myotonic.org/resources

MDF Resources for Healthcare Professionals

These publications are for professional audiences – especially doctors and nurses – who treat and care for individuals living with DM. You can access them all for free on the MDF website or email us at info@myotonic.org to get a hard copy mailed to you.

*An * indicates this resource is available in more than one language.*

Care Guidelines for Speech and Language Pathologists Treating Adults and Children with Myotonic Dystrophy

This guide is written and reviewed by an international group of speech and language pathologists who are specialized in working with adults and children with DM.

Clinical Care Recommendations*

Resources to improve and standardize care developed by more than 65 leading DM clinicians in Western Europe, the UK, Canada, and the US. Resources include recommendations for DM1 in adults and children, and adults with DM2.

Clinical Care Recommendations for Cardiologists & Pulmonologists Treating Adults with Myotonic Dystrophy Type 1*

Two separate resources for cardiologists and pulmonologists that provide care recommendations for treating adults with DM1.

Clinical Recommendations for People of Pregnancy Potential with Myotonic Dystrophy*

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

Occupational Therapy Suggestions for the Management of a Myotonic Dystrophy Patient

A quick reference excerpt for clinicians from the full MDF Toolkit.

Practical Suggestions for the Anesthetic Management of a Myotonic Dystrophy Patient*

Regardless of the form of DM or the severity of DM symptoms experienced, severe and life-threatening reactions to anesthesia are possible and should be monitored whenever anesthesia is administered.

Respiratory Care Recommendations for Myotonic Dystrophy Patients During the COVID-19 Pandemic*

This guide includes tips for patients, caregivers, and medical providers on the use of noninvasive positive pressure ventilation (NIPPV) if you have been exposed to or have symptoms of COVID-19 infection.

The Role of Physical Therapy in the Assessment and Management of Individuals with Myotonic Dystrophy

These Physical Therapy Guidelines for DM address the role that the physical therapist plays in DM care.

Learn more at www.myotonic.org/resources

Myotonic Dystrophy Drug Development Pipeline

Below is a partial snapshot of drugs for myotonic dystrophy that are currently in clinical trials. The DM Drug Development Pipeline was developed by MDF and continues to evolve based on publicly available information. Academic institutions are not included. To view the full pipeline, scan the code to the right or visit www.myotonic.org/pipeline.

Company	Program	DM Subtype	Clinical Phase		
			1	2	3
AMO PHARMA	Tideglusib	CDM / DM1			
AVIDITY BIOSCIENCES A Novartis Company	AOC 1001 del-desiran	DM1			
LUPIN NEUROSCIENCES	Mexiletine	DM1 / DM2			
Dyne THERAPEUTICS	Dyne 101 z-basivarsen	DM1			
ARTHEx biotech	ATX-01	DM1			
HARMONY BIOSCIENCES	Pitolisant	DM1			
PepGen	PGN-EDODM1	DM1			
Juvena THERAPEUTICS	JUV-161	DM1			
VERTEX	VX-670	DM1			
sanofi	--	--			
SAREPTA THERAPEUTICS	SRP-1003	DM1			

International Myotonic Dystrophy Awareness Day

MDF is proud to be a founding member of the Global Alliance for Myotonic Dystrophy Awareness, a group of organizations and institutions from around the world working together to raise awareness of myotonic dystrophy. Learn more at: www.myotonic.org/international-dm-day

Make the Most Out of Your Conference Experience

Join us at a First Timers' Table!

Each MDF Regional Conference features First Timers' Tables at breakfast and lunch for those attending their first in-person MDF event. Look for the First Timers' table signs to connect with MDF and community leaders who can help you get oriented to the conference, MDF, and the myotonic dystrophy community.

Make the Most of Breaks

There are many breaks built into the conference agenda so you can take care of personal needs. Use this time to snap a photo, connect with others, or visit exhibitor booths.

Take A Photo!

Don't let your conference memories fade away: share your experience. Post on social media, tag Myotonic Dystrophy Foundation (MDF) or @MyotonicStrong, and use #MyotonicDystrophy to help raise awareness and stay connected with the community.

Don't forget to stop by the MDF step-and-repeat backdrop near registration for a fun photo background.

Myotonic Dystrophy Family Registry

Living with Myotonic Dystrophy?

We need to hear from you! Join the MDFR today and help support DM research.

By joining the MDFR you will:

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

Log in now at: www.myotonicregistry.org

Questions? Call us at 415-800-7777

Continue Learning with MDF

MDF Digital Academy

Watch hours of educational and inspiring videos from DM experts whenever you like. Browse by category and interest at: www.myotonic.org/digital-academy

Meet the DM Drug Developers Webinar Series

Since 2021, MDF has hosted biotechnology and pharmaceutical partners working on treatments and cures for myotonic dystrophy in special presentations for the community. In these sessions, they share progress and answer questions. View the complete series at: www.myotonic.org/meet-dm-drug-developers

Explore the Updated Find a Doctor Map

Finding medical professionals who understand myotonic dystrophy is one of the most important ways to help manage its symptoms. With input from the community, MDF has compiled a database of healthcare providers with experience caring for people living with DM. Find a medical professional in your area at: www.myotonic.org/find-a-doctor-map

Ask the Expert Series

MDF's virtual Ask the Expert series is available online, featuring DM experts across many body systems. Experts in digestion, brain, heart, speech and swallowing, mental health, lungs, and more share information and answer questions. View the complete archives at: www.myotonic.org/ask-expert-series

Sign Up for Emails

The MDF monthly e-newsletter, the MDF Dispatch, provides you with up-to-date information on research advances, DM daily living strategies, upcoming events and stories from our community members. Sign up to join our mailing list at: www.myotonic.org/sign-up

Need additional support? Get One-on-One Support at 415.800.7777

July is DM In Motion Awareness Month!

Movement makes connections, and we're excited to bring the DM community together through weekly Movement Monday programming, Movement and Meeting Happy Hours, and more! Special thanks to our Movement Committee for their continued support and development of this initiative!

Now through the end of July - order your limited-edition DM In Motion Awareness Month gear at www.myotonic.org/in-motion

Thank You to Our Amazing Volunteer Leaders!

2026 MDF Board of Directors

Thank you to our 2026 Board of Directors which is comprised of volunteer leaders from the public and private sectors, most of whom are either living with myotonic dystrophy or have loved ones living with the disease. The Board works closely with the MDF Staff and Scientific Advisory Committee. To learn more about MDF Board of Directors, visit: www.myotonic.org/board-directors

Jeremy Kelly • Martha Montag Brown • Elizabeth Florence, Esq • David Herbert • Andy Berglund, PhD
Rob Campagna, MD • Belen Esparis, MD • Charles Thornton, MD • David Berman, MBA • John Cooley
Peter DesForges • Haley Martinelli, Esq • John W. Day, MD, PhD • Thomas (Tom) McPeek

2026 MDF Support Group Facilitators

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. Learn more about our SGFs at: www.myotonic.org/sgfs

Alexandra LeBoeuf • Anke Klein • Ann Woodbury • Annette Rnjak • Araceli Mera • Bernhard Rogg
Beth Feigenblatt • Bill Nuttall • Caroline Easterling • Carolyn Valek • Chuck Hunt • Cindy Hubert
David Kugler • Emily Jones • Erin Beucler • Guillermo Zubillaga • Haley Martinelli • Jeannine DeSoi
Jim Dolan • Jodie Howel • John Cooley • Julian Easterling • Julie LeBoeuf • Kathie Thorsland • Kathy Coletta
Kelsey Freedman • Kim McPeek • Kim Reynolds •Kristen McClintock • Kyle Dunson • Lois Schenk
Luke Desforges • Lynn Schneider • Marie-Claude Sauvé • Mark Coplin • Mark Planco • Mindy Kim
Nathan Beucler• Patricia Gibson • Peggy Melton • Rob Besecker • Rose Albanese • Ryan Vogels
Samantha Welsh • Sarah Berman • Shaun Moore • Suzanne Perkins • Ted Salwin • Tom McPeek

Advocate for the DM Community: Your Voice Matters!

By raising awareness, we help influence research funding, therapy development, clinical trials, and care for people living with myotonic dystrophy.

Help change the future of DM today:

- Educate Policymakers – Learn how to engage officials to protect and expand research funding.
- Advocate with Confidence – Get tools to speak with representatives about DM research and care.
- Share your Story – Your experience can drive change.
- Strengthen DM Support – Celebrate advocacy wins and build relationships with key decision-makers.

www.myotonic.org/Advocate

DM INSIGHT Survey Results

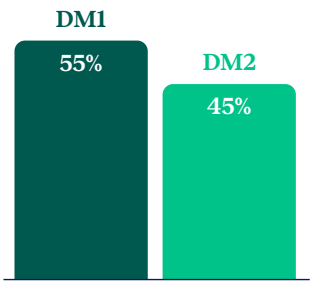
In 2025, the Myotonic Dystrophy Foundation completed DM INSIGHT, a community-informed study to better understand the real-life experiences of people affected by myotonic dystrophy. This work was made possible by members of the DM community who shared their perspectives.

This infographic highlights key findings related to daily life with DM, access to care, and support for caregivers. As new treatments move closer to reality, these insights can help guide healthcare providers, researchers, advocates, policymakers, and payors to support meaningful improvements in everyday life.

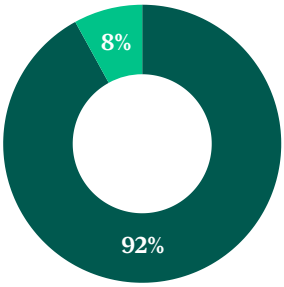
Who Participated?

447
People Living with DM

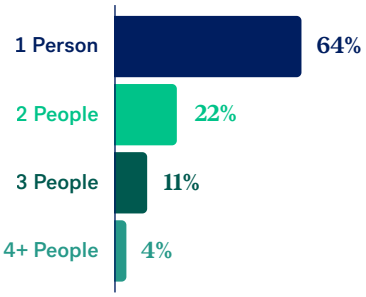
238
Caregivers



% of Respondents Diagnosed with DM1 or DM2



% of Respondents by Country (US. vs. Canada)



% of Caregiver Respondents by Number of People They Care For

Access to and Satisfaction of Healthcare

People with DM & their caregivers felt their medical care was average, with differences between DM1 & DM2.

DM1: Slightly more satisfied

DM2: Slightly less satisfied

Most Helpful Healthcare Professionals

45% Neurologists

38% General Medicine

What Drives DM Healthcare Satisfaction?

Your confidence in knowledge of anesthesia & cardiac risk.

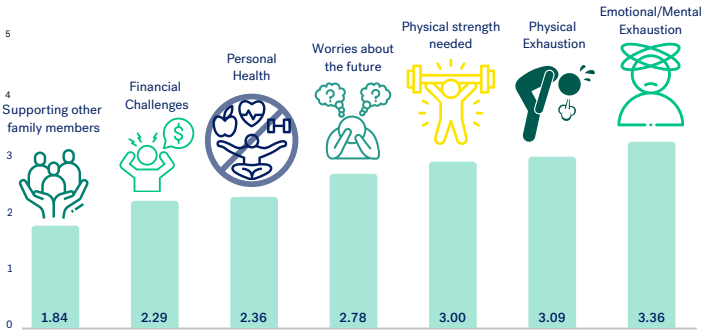
Confidence in your doctor's expertise in DM.

Seeing your doctor more frequently.

Shorter wait times to see your doctor.

Aspects of the Caregiver Experience

How Caregivers Rated Their Challenges: 1 = Low, 5 = High



All seven aspects of caregiver burden are strongly connected. Struggles in one area often spill over to others.

What are Predictors of Caregiver Burnout?

- Higher care needs means higher burden
- Fewer breaks mean higher burden

The Biggest Challenges for Caregivers

49% Addressing emotional or behavioral changes

45% Supporting daily activities or mobility

Myotonic Dystrophy FOUNDATION

Help Shape the Future of DM Treatments!

Participate in MDF's DM COMPASS Study

SCAN ME!

Your voice matters. The DM COMPASS Risk/Benefit Study gathers input to help inform the development of new therapies. MDF has partnered with Silicon Valley Research Group to host the survey and support analysis of the results.

Share your thoughts on potential myotonic dystrophy therapies, including which risks you would accept or not accept, and which benefits matter most. The risks described in this survey are based on hypothetical therapies and may or may not occur with any given treatment.

Shape the future of potential DM treatments!

- Who can participate? Individuals living with DM1 or DM2 living in the US and caregivers for individuals with DM1 or DM2.
- Why take the survey? Your responses will help guide the development of new therapies that reflect the priorities of the DM community.
- How long will it take? Approximately 20 to 25 minutes
- Incentive: Individuals who complete the survey can enter a random drawing for one of three $750 Visa gift cards.

Myotonic Dystrophy FOUNDATION

Community Connections

Send a Letter, Share a Smile!

The DM community can use your support. Many people living with myotonic dystrophy experience social isolation and may find it hard to connect. Send a heartfelt letter, drawing, or story to brighten someone's day.

Nominate a community member to receive letters.

Write a letter, share a joke, or draw a picture.

Mail or email your message, and MDF will forward it in a special care package!

Your words can make a difference—reach out today!

www.myotonic.org/community-connections

Myotonic Dystrophy FOUNDATION

Fundraise Your Way!

Are you celebrating a special occasion, participating in a fitness challenge, or hosting a community event?

You can make a difference with a fundraiser for MDF!

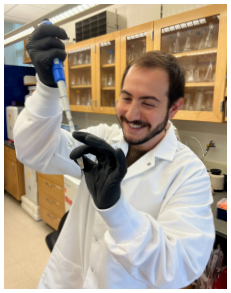
Your DIY (Do It Yourself!) Fundraiser empowers our work to support the DM community, provide essential resources, and accelerate research toward treatments and a cure.

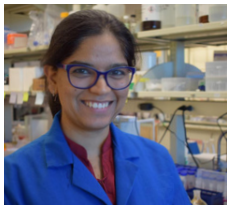
www.myotonic.org/DIY

SCAN ME!

Myotonic Dystrophy FOUNDATION

Apply for MDF Research Grants!





MDF is funding innovative research to drive progress in understanding, care, and treatments for myotonic dystrophy.

Pilot Grants - $50k

Research Fellowship - $55k | $105k
- Doctoral
- Postdoctoral

Early Career Grant - $190k
- Basic / Translational Science
- Clinical Research

Small Grants - $2.5k | $5k
- Conference travel
- Open-access fees

APPLY NOW!

www.myotonic.org/Grants

SCAN ME!

Patients are at the heart of everything we do at Astellas

Our Patient Centricity vision is to enable a future for Astellas where all healthcare innovation is driven by the needs of patients and caregivers. At Astellas we develop medicines by connecting patient and caregiver experiences at every stage of our medicines' development – from early research through to delivery and beyond.

Patients are the reason why Astellas exists, and they are the driving force at the center of everything we do.

PATIENTS ARE WHY™

astellas

Find out more at:

astellas.com/en/about/patient-centricity @AstellasUS

BrAAVe

The BrAAVe Study is a gene therapy clinical research study for persons living with myotonic dystrophy type 1 (DM1).

The purpose of this research study is to evaluate the safety and efficacy of the gene therapy investigational study drug in persons living with DM1. Our hope is to see if the investigational gene therapy can safely help them and improve their quality of life.

You may qualify for this study if you:

- Are 18 to 50 years of age.
- Have been diagnosed with non-congenital DM1, presenting with signs of myotonia and muscle weakness.

If you are interested in learning more about the research study, talk to your doctor or visit our website.

Interested in learning more?

sanofi

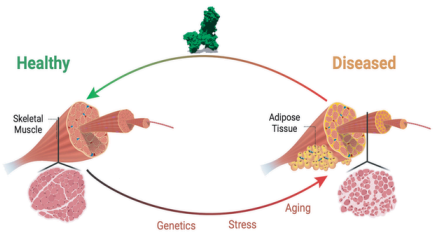
Juvena's mission is to reJuvenate lives with Medicine for Movement

Juvena THERAPEUTICS

Pioneering therapeutics for myopathies and metabolic diseases

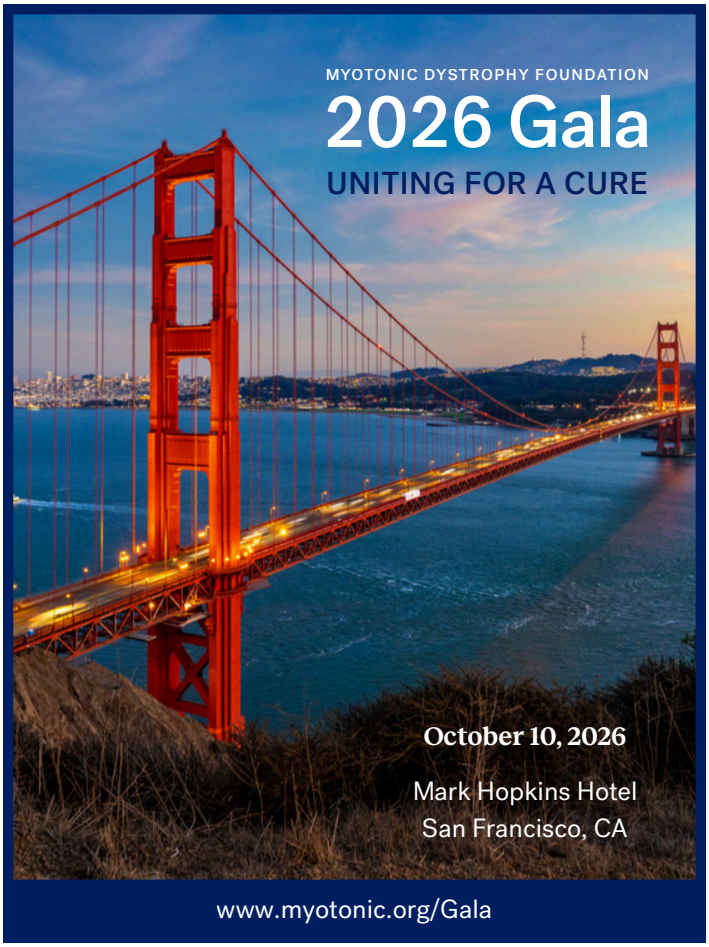
There are no currently approved muscle regeneration therapies for myotonic dystrophy, despite a significant unmet need. We combined our diverse library of stem-cell secreted proteins with our proprietary AI-enabled JuvNET platform to discover and translate our pipeline of biologic candidates.

The Juvena Solution



Restoring muscular and metabolic function through innovative biologics

Myopathies and metabolic diseases can result from a breakdown within the complex intercellular protein signaling pathways in the body. Until now, the available tools for mapping this biology limited the development of regenerative therapies and a significant unmet need exists. Juvena targets the dysregulation of proteins by engineering novel biologics to restore tissue homeostasis.








Myotonic Dystrophy FOUNDATION

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