

Los Angeles, California

2024 MDF Regional Conference

May 18th

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



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FOUNDATION

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2024 MDF Regional Conference - Los Angeles, CA

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2024 MDF Regional Conference

May 18th in Los Angeles, California

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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 2024 MDF Regional Conference in Los Angeles, CA!

On behalf of the Board of Directors and staff of the Myotonic Dystrophy Foundation (MDF), I am delighted to welcome you to our 2024 MDF Regional Conference in Los Angeles, California! In our 16th year of conferences, we are thrilled to have over 125 community members joining us from more than 9 US states and 2 countries. Special thanks to our partners at UCLA Health for hosting us today.



This year's MDF Regional Conferences are a unique opportunity to bring our local communities closer together: sharing experiences, connecting with local clinicians and researchers, and learning about the latest progress towards treatments and a cure. After a long pandemic that kept us apart and isolated, MDF was eager to provide a space where families could engage, learn, ask questions, and participate in meaningful conversations – without having to travel long distances. Thanks to thoughtful input from hundreds of our community members, we have crafted an agenda to help meet the diverse needs and interests of our community.



I want to express our deep appreciation for the dedicated researchers and industry partners who have been instrumental in advancing myotonic dystrophy drug development. This has been an extraordinary year with new companies launching clinical trials and the announcement of a new Phase 3 clinical trial for myotonic dystrophy! Our community's engagement in clinical trials and studies is crucial to help get us over the finish line and bring a drug to market. We hope you have joined the Myotonic Dystrophy Family Registry to help further scientific knowledge and learn more about research opportunities ahead. We look forward to celebrating progress with you and embracing the hopeful time that lies ahead.



To further maximize our quality of life and get ready for new trials and studies, I hope you will join us this July as we dedicate the month to movement! July is Myotonic Dystrophy In Motion month so stay tuned for fun activities and ways to engage! www.myotonic.org/myotonic-dystrophy-motion

The entire MDF team looks forward to talking with you today! Please reach out if we can help make your conference experience better or you have any questions.

Enjoy the Conference!



Tanya Stevenson, EdD, MPH
Chief Executive Officer

Agenda for Saturday, May 18th

Time	Grand Horizon Salon A	Salon B + C	Salon E	JOA Lounge South Bay Room
8:30 - 10:00 AM	Check In Opens			
9:00 - 10:00 AM	Breakfast			
10:00 - 10:45 AM	Welcome & Introduction to DM Resources			JOA: Getting to Know You & Ice-Breaker Games
10: 45 - 11:00 AM	Break			
11:00 - 11:45 AM	DM: What You Need to Know	Mental Health & DM		JOA: Stump the Doctor
11:45 AM - 12:00 PM	Movement Moment			
12:00 - 12:15 PM	Break			
12:15 - 1:00 PM	DM Research: What You Need to Know About Clinical Trials	Sleep & Breathing in Myotonic Dystrophy		JOA: “Let’s Talk About Feelings”
1:00 - 2:00 PM	Lunch	Lunch Discussion with Avidity Biosciences (RSVP Required)	Lunch Discussion with Dyne Therapeutics (RSVP Required)	
2:00 - 2:45 PM	How Myotonic Dystrophy Affects the Heart	Community Panel: Life Hacks		JOA: Movement Moment
2:45 - 3:00 PM	Break			
3:00 - 4:00 PM	Industry Updates			JOA: Games & Karaoke
4:00 - 4:15 PM	Break			
4:15 - 5:00 PM	Stump the Doctor: DM1 & DM2			
5:00 - 5:30 PM	Break			
5:30 - 7:00 PM	Dinner & Dance Party!			

General Sessions

General sessions are for everyone! All general sessions are located in the [Grand Horizon Salon A](#).

10:00 - 10:45 AM

Welcome and Introduction to DM Resources

The Myotonic Dystrophy Foundation & UCLA Health

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 Iowa and their work in the DM space.

11:45 AM - 12:00 PM

Movement Moment

Movement can improve both the physical and mental well-being of people living with myotonic dystrophy. As a part of MDF's In Motion program, this session will help us break up sitting through our sessions with a set of accessible movement activities.

12:15 - 1:00 PM

DM Research: What You Need to Know About Clinical Trials

Perry Shieh, MD, PhD, Neurologist, UCLA Health

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.



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

Payam Soltanzadeh, MD, UCLA Health; and
John Day, MD, PhD, Stanford University

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?

Please 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 (*Grand Horizon Salon A*)

DM1 & DM2: What You Need to Know

Payam Soltanzadeh, MD, UCLA Health; and
John Day, MD, PhD, Stanford University

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.



11:00 - 11:45 AM (*Salon B + C*)

Mental Health & Myotonic Dystrophy

Melissa M. Dixon, PhD, MS, University of Utah Health

This session provides an overview of the challenges and strategies to maintain good mental health while living with DM. Attendees can look forward to taking home tips and tools they can apply to their own lives and dedicated time for follow up questions.



12:15 - 1:00 PM (*Grand Horizon Salon A*)

Respiratory and Sleep Health in Neuromuscular Disorders

Ravi S. Aysola, MD, Clinical Professor of Medicine, School of Medicine at UCLA

Join this session to explore the latest advancements in myotonic dystrophy research. This presentation will cover recent developments that continue to shape our understanding and management of DM. Gain insights from a leading researcher who will share the latest findings and discuss the future of DM.



2:00 - 2:45 PM (*Grand Horizon Salon A*)

Myotonic Dystrophy and the Heart

Daniel Cruz, MD, PhD, UCLA Health

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.



2:00 - 2:45 PM (*Salon B + C*)

DM Life Hacks Community Panel

Sarah Berman & John Cooley, MDF Support Group Facilitators

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.



Juvenile-onset Adult (JOA) Sessions

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

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

10:00 - 10:45 AM

Getting to Know You & Ice-Breaker Games

Ann Woodbury, Co-Facilitator of the MDF JOA Warriors Support Group

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!



11:00 - 11:45 AM

Stump the Doctor: JOA

Perry Shieh, MD, PhD, Neurologist, UCLA Health

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?



12:15 - 1:00 PM

JOA: Let's Talk About Feelings

Melissa M. Dixon, PhD, MS, University of Utah Health

Join us for a special JOA session centered on feelings. This session will focus on how feelings can affect our bodies and minds with an interactive discussion around strategies for managing overwhelming emotions.



2:00 - 2:45 PM

Get Moving with the JOAs

Lucie Shiffman, Senior Community Engagement Manager, MDF

This session is a great opportunity to move. This movement session is for all levels and experiences.



3:40 - 5:10 PM

JOA Games & Karaoke

Ann Woodbury, Co-Facilitator of the MDF JOA Warriors Support Group

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.

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 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.*

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 Myotonic Dystrophy: 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 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 United States

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*

This guide is written and reviewed by nutritionists specialized in working with individuals with DM, and includes 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) Myotonic Dystrophy and 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 the employment process and includes information on how DM can affect your job, how to assess your readiness to work, how to search and apply for a job, how to write a resume and cover letter, tips for interviewing and much more. (US Health System only)

Learn more at www.myotonic.org/toolkits-publications

MDF Resources for Healthcare Providers

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/toolkits-publications

International Myotonic Dystrophy Awareness Day

MDF is proud to be a founding member of the Global Alliance for Myotonic Dystrophy Awareness! Currently comprised of 57 organizations and institutions around the world dedicated to helping raise awareness about DM! Learn more at: www.myotonic.org/international-dm-day



Advocate for DM Research Funding!

Check out the MDF Advocacy Booth where you can connect with a DM community advocate to learn more about what you can do to increase awareness and support for myotonic dystrophy!



Special thank you to our National Advocacy Committee members who lead the charge in executing MDF's advocacy campaign to increase DM awareness, grow DM research funding, and advance policies to accelerate the approval of drugs to treat and eventually cure DM. Learn more about our latest advocacy efforts at: www.myotonic.org/advocate

Make the Most Out of Your Conference Experience



Take a Seat at Our First Timers' Tables!

Each MDF Regional Conference features First Timers' Tables at breakfast and lunch for those who are at their first ever in-person MDF event. Look out for the First Timers' table signs to connect with MDF and community leaders who will help orient you to the Conference, MDF, and the DM Community.



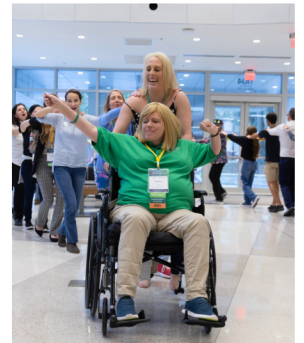
Take A Picture!

Don't let Conference memories fade away - showcase your experience! Post to social media, tag MDF (@MyotonicStrong), and use the hashtag #MyotonicDystrophy to amplify your voice, create lasting memories, and raise awareness for myotonic dystrophy.



Taking Breaks

There are many breaks built into the conference agenda so that you can take care of personal needs: use this time to snap a picture, network, visit exhibitor booths, and take in other MDF Conference experiences.



**Myotonic
Dystrophy**
Family Registry

Living with Myotonic Dystrophy? Help Change the Future of 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

View hours of educational and inspirational videos by DM experts whenever you would like. Sort by category and interest at: www.myotonic.org/digital-academy



Meet the DM Drug Developers

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



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 & stories from our community members. Sign up to join our mailing list at: www.myotonic.org/sign-emails-0



Find-a-Doctor Map

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



Ask-the-Expert

MDF's virtual series with DM experts is available online! Experts in digestion, brain, heart, speech and swallowing, mental health, lungs, and other body systems related to DM share information & answer community submitted questions. View the complete archives at: www.myotonic.org/ask-expert-series

Movement Makes Connections



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Come as you are.

July is DM In Motion Awareness Month!

Movement makes connections, and we're excited to bring the DM community together through weekly themes, action items, and resources. 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!

2024 MDF Board of Directors

Thank you to our 2024 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
Haley Martinelli, Esq • John W. Day, MD, PhD • Thomas (Tom) McPeek

2024 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 • Araceli Mera • Bernhard Rogg • Bill Nuttall
Carolyn Valek • Chuck Hunt • Cindy Hubert • David Kugler • Emily Jones • Guillermo Zubillaga • Janis Jaffe
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Contribute to Community, Care, and a Cure!

With your support, MDF provides assistance and information to our community, funds groundbreaking research, raises DM awareness, and advocates with legislators and federal agencies. *Help change the future of DM - donate today!*



DONATE VIA PHONE: 415.800.7777



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