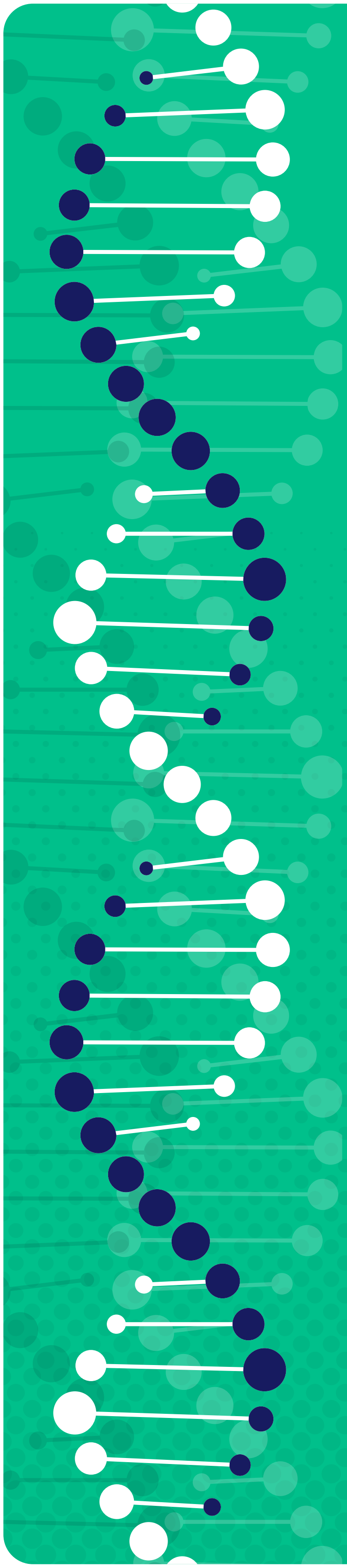
Myotonic
Dystrophy
FOUNDATION

2025 MDF International Conference

The Westin Indianapolis
May 1st - 4th

A Four-day Event to Unite Community, Care, and a Cure for Myotonic Dystrophy



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.

Myotonic Dystrophy Foundation

663 13th Street, Suite 100, Oakland, California 94612 | www.myotonic.org | 415.800.7777 | info@myotonic.org

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Table of Contents

Myotonic Dystrophy FOUNDATION

2025 MDF International Conference

MAY 1ST - 4TH

Breakout Sessions

MDF Team:

Welcome Letter





Welcome to the 2025 MDF International Conference!

On behalf of the Myotonic Dystrophy Foundation's (MDF) Board, staff, and the many volunteers who have brought this year's event to life, we are delighted to welcome you to our 2025 International Conference in Indianapolis! This year marks MDF's 16th conference, and we are honored to host over 600 attendees from over 9 countries.

Our conference unites the extraordinary myotonic dystrophy (DM) community: DM families, researchers, clinicians, industry partners, advocates, and allies, all working together toward care, research, and hope. A warm welcome to our first-time attendees—we are thrilled to have you with us!

Thanks to invaluable input from our community, we have planned over 70 sessions across seven tailored tracks for **DM1, DM2, CDM, Community, JOA, Caregivers, and DM Professionals.** This year's program is packed with exciting offerings: **General Sessions,** open to all attendees, highlighting MDF updates, groundbreaking research advancements, and moving stories from our community members; **Community Breakout Sessions** covering new and essential topics; the much-loved **First Timers' Tea** for newcomers to connect; and the ever-popular **Stump-the-Doctor** sessions, where experts take on the community's toughest questions. Attendees can also engage in five unique **Community Panels** and stay informed with **Industry Updates** highlighting the latest strides in DM drug development.

This year, we're especially excited about our interactive and fun events, including a **Masquerade Murder Mystery Dinner** on Friday, a dinner and dance party on Saturday, insightful poster sessions featuring cutting-edge research, and numerous networking opportunities to foster lasting connections. Don't miss the **Wellness Room** for relaxation and **Movement Moments** to keep you energized throughout the weekend.

Take time to visit exhibitor booths and connect with researchers during the poster showcase with over **70 DM-focused research posters**. Whether you're joining us in person or virtually, our conference platform ensures you can access sessions, resources, and connections anytime.

Thank you for joining us and for contributing to the success of this remarkable community. Please reach out to the MDF team if you need anything. We can't wait to meet many of you and hope this conference leaves you inspired, informed, and empowered.

Enjoy the conference!

Tanya Stevenson, EdD, MPH
Chief Executive Officer

Tanya Stevenson

Mindy Buchanan
Director of Programs

Mindy Buchanan

Emily Romney, MPA
Senior Manager of
Community Education

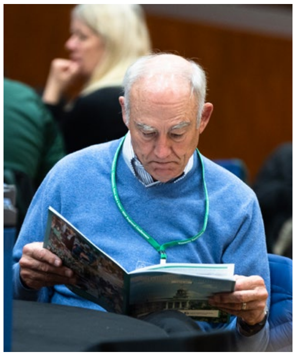
Emily Romney

Joining Virtually

Whether you'd like to view a session you couldn't attend in person or prefer to engage from the comfort of your hotel room, our virtual platform ensures you won't miss a thing.

Be sure to check your program to see which sessions are offered virtually.

Use the checklist below for the best virtual conference experience possible!



Preparing to Join the Zoom Event:

- Ensure you have a reliable internet connection.
  (A speed > 1.5 Mbps is recommended for HD video, check your speed at **www.fast.com** or **www.speedtest.net**)
  - If you have a slower connection, ensure that you are not competing for bandwidth with others in your household (streaming movies, playing video games online, etc...)
- Laptop or desktop computer
  - Use **Google Chrome** or **Mozilla Firefox** as your web browser
  - Download the Zoom video conferencing app from **www.zoom.us**
- Smartphone or Tablet
  - Download the Zoom app from the **Apple App Store** or **Google Play Store**

Join Us Virtually:

- Check the *email you registered with* for an email from **noreply-zoomevents@zoom.us** which contains your unique registration link. (Make sure to check your Junk & Spam folders)
- Use the "Join Now" link in your email.
- **Can't find the email from Zoom or forgot your password?**
  Contact **victoria@globalimpactagency.com** for support!

Before the Conference:

- Build your personal conference agenda! After logging into the Zoom Event, you can view the full agenda and bookmark sessions you'd like to attend.

At the Conference:

- Join sessions directly from the Zoom Events agenda.
- Ask questions during sessions using the built in Q&A feature.
- Explore our poster showcase by visiting the Research Poster Sessions to view each poster as a PDF.

Enjoy the Conference!

Need Technical Support?

If you have trouble logging into the system or need additional technical support, please email: **victoria@globalimpactagency.com**.

During the Conference on May 2nd & 3rd, join a Live Support Zoom from 9:00 am - 5:00 pm EST: **www.myotonic.org/tech-support**

Agenda for Friday, May 2nd

Time**	General Sessions & Meals Grand Ballroom 4+5	Professional Sessions* Capitol Ballroom 1+2	JOA Lounge Rec Room	Wellness Room Capitol Overlook North	Fireside Chats House
8:00 AM-9:00 AM		DM Professionals Breakfast			
9:00 AM-10:00 AM	First Timers' Tea	Logistics, Professional Session 1		Morning Self-Guided Meditation	
10:00 AM-11:00 AM	Community Breakfast			Quiet Space	
11:00 AM-11:45 AM	Welcome & State of the Foundation				
11:45 AM-12:00 PM	Break Movement Moment	Break	JOA Lounge Opens		
12:00 PM-1:00 PM	Breakout Sessions	Professional Session 2	JOA Session: Welcome and Get to Know You Games	Chair and Stand Up Zumba	You've Just Been Diagnosed, What's Next?
1:00 PM-1:15 PM	Break			Quiet Space	
1:15 PM-2:15 PM	Lunch	Networking Buddies Lunch			
2:30 PM-3:30 PM	Breakout Sessions	Professional Session 3 (end at 3:40)	JOA Session: Building Connections	Movements for Everyday Life	**Affected Men's Real Talk:** Managing Intimacy and Sexual Health Challenges with DM
3:30 PM-3:45 PM	Break			Quiet Space	
3:45 PM-4:45 PM	Industry Updates Part 1		JOA Lounge Karaoke/ Craft Time/Games		
4:45 PM-5:00 PM	Break Movement Moment	Break	JOA Movie Night		
5:00 PM-6:00 PM	Breakout Sessions	Professional Networking & Professional Poster Showcase			**European Patient Organizations:** A Unique Opportunity to Share and Celebrate
6:00 PM-6:30 PM	Break			Wellness Room Closes	
6:30 PM-8:30 PM	Welcome Dinner & Entertainment: Murder Mystery				

🟩 General Sessions ▢ Community ■ Professional ■ JOA / Life Works ■ Wellness ▢ Fireside Chats

Agenda for Friday, May 2nd *Breakouts*

Time**	Grand Ballroom 1 DM1	Grand Ballroom 2 DM2	Grand Ballroom 3 Caregivers	Chamber CDM	Council	Caucus	Cabinet Community
8:00 AM-9:00 AM					**MDF Fellows Breakfast** (Private)		
9:00 AM-10:00 AM							
10:00 AM-11:00 AM							
11:00 AM-11:45 AM							
11:45 AM-12:00 PM	Break						
12:00 PM-1:00 PM	**Unraveling DM1:** From Genetics to Care and Everything in Between	**DM2 Essentials:** Understanding, Managing, and Thriving with Myotonic Dystrophy Type 2		**Adulthood Ahead:** Essential Planning for Parents of Children with CDM	**Finding Relief:** Exploring Medications and Tools for Managing Pain in DM2	**Your Guide to Clinical Trials:** From Phases to Participation	**Nourishing Solutions:** Managing Dietary Needs and Swallowing with DM
1:00 PM-1:15 PM	Break						
1:15 PM-2:15 PM							
2:30 PM-3:30 PM	**Genetics Unveiled:** Understanding Testing, Counseling and Family Conversations about DM1	**Mind Matters:** Navigating Cognitive and Memory Challenges in DM2	**Building Resilience:** Family Dynamics and Support in the DM Journey		**Keeping Your Heart in Check:** DM1's Impact and Care Tips	**Life Works: Landing the Job:** Building Resumes and Acing Interviews	
3:30 PM-3:45 PM	Break						
3:45 PM-4:45 PM							
4:45 PM-5:00 PM							
5:00 PM-6:00 PM	**DM1: Networking & Social Hour**	**DM2: Networking & Social Hour**	**Caregiver Networking & Social Hour**	**CDM Family Networking & Social Hour**			**Living Better with DM:** Tools and Services of Occupational Therapy and Home Health
6:00 PM-6:30 PM	Break						
6:30 PM-8:30 PM							

■ DM1 ■ DM2 ■ Caregivers ■ CDM

** View full professional session title & speaker information on pages 18-21.*

*** All times listed in Eastern Daylight Time.*

Agenda for Saturday, May 3rd

Time**	General Sessions & Meals Grand Ballroom 4+5	Professional Sessions* Capitol Ballroom 1+2	JOA Lounge Rec Room	Wellness Room Capitol Overlook North	Fireside Chats House
8:00 AM-9:00 AM		DM Professionals Breakfast			
9:00 AM-10:00 AM	Community Breakfast	Professional Session 4		Morning Self-Guided Meditation	
10:00 AM-11:00 AM	Welcome & Stories of Inspiration		JOA Lounge Opens	Quiet Space	
11:00 AM-11:15 AM	Break Movement Moment				
11:15 AM-12:15 PM	Breakout Sessions	Lightning Round	JOA Session: Let's Get Moving!		Affected Women's Real Talk: Managing Intimacy and Sexual Health Challenges with DM
12:15 PM-12:30 PM	Break				
12:30 PM-1:30 PM	Exhibitor and Research Poster Showcase (Capitol Ballroom 1 + 2)	Exhibitor and Research Poster Showcase	JOA Session: Snack Smart: Making Easy, Healthy, Dysphasia Friendly, & Delicious Bites	Afternoon Gentle and Chair Yoga	
1:30 PM-1:45 PM	Break			Quiet Space	
1:45 PM-2:45 PM	Lunch				
2:45 PM-3:45 PM	Industry Updates Part 2		JOA Karaoke/ Free time		There's Hope: A Discussion About the Future of DM2 Research
3:45 PM-4:00 PM	Break Movement Moment	Young Investigators' Workshops			
4:00 PM-5:00 PM	Breakout Sessions				Voices for DM: Open Discussion on Advocacy
5:00 PM-5:15 PM	Break				
5:15 PM-6:15 PM	Breakout Sessions		Stump the Doctor: JOA		
6:00 PM-6:30 PM	Break			Wellness Room Closes	
6:30 PM-8:30 PM	Closing Dinner & Dance Party				

🟩 General Sessions ▢ Community ■ Professional ■ JOA / Life Works ■ Wellness ▢ Fireside Chats

Agenda for Saturday, May 3rd *Breakouts*

Time**	Grand Ballroom 1 DM1	Grand Ballroom 2 DM2	Grand Ballroom 3 Caregivers	Chamber CDM	Council	Caucus	Cabinet Community
8:00 AM-9:00 AM				**SAC Breakfast** (Private)			
9:00 AM-10:00 AM							
10:00 AM-11:00 AM							
11:00 AM-11:15 AM	**Break**						
11:15 AM-12:15 PM	**DM1 Community Discussion Panel:** Sharing Hope and Life Hacks	**DM2 Community Discussion Panel:** Stories of Hope and Helpful Tips	**Unaffected Male Caregivers Community Panel:** Prioritizing Self Care & Wellbeing	**CDM Community Discussion Panel:** Sharing Everyday Victories Through Useful Tips, Tricks, and Assistive Services	**Unaffected Female Caregivers:** Practical Tips for Self-Care		
12:15 PM-12:30 PM	**Break**						
12:30 PM-1:30 PM							
1:30 PM-1:45 PM							
1:45 PM-2:45 PM	**Global Alliance Lunch** (Private)			**Young Investigators' Workshop Lunch** (Private)			
2:45 PM-3:45 PM							
3:45 PM-4:00 PM	**Break**						
4:00 PM-5:00 PM	**Breathe Easy, Sleep Sound: Managing** Sleep and Pulmonary Health with DM1	**Genetics Unveiled:** Understanding Testing, Counseling and Family Conversations about DM2	**Unaffected Caregivers: Love in Transition:** Balancing Romantic Connections and Caregiving in DM	**Empowering Adult Children:** Managing Care, Service Delivery, and Living with CDM		**Thriving Under Pressure: Managing** Mental Health in Everyday Life	**Securing Tomorrow:** Financial Strategies and Planning for Your and Your Family's Future
5:00 PM-5:15 PM	**Break**						
5:15 PM-6:15 PM	**Stump the Doctor: DM1**	**Stump the Doctor: DM2**					
6:00 PM-6:30 PM	**Break**						
6:30 PM-8:30 PM							

■ **DM1** ■ **DM2** ■ **Caregivers** ■ **CDM**

** View full professional session title & speaker information on pages 18-21.*

*** All times listed in Eastern Daylight Time.*

General Sessions

General Sessions are for everyone! All General Sessions are located in **Grand Ballroom 4 + 5.**

THURSDAY, MAY 1ST

Early Arrival Reception
6:00 pm - 8:30 pm
Kick off the MDF 2025 Conference at our Early Arrivals Welcome Reception on Thursday evening! Join fellow attendees for a relaxed and friendly gathering to connect, mingle, and get acquainted before the full weekend begins. Enjoy light refreshments, meet new friends, and reconnect with familiar faces as we come together to build community and excitement for the days ahead.

FRIDAY, MAY 2ND

Martha Montag Brown
MDF Board Member

Tom McPeek
MDF Board Member, Volunteer Support Group Facilitator

First Timers' Tea
9:00 am – 10:00 am
The First Timers' Tea is an informational and networking event for those who have never before attended an MDF Conference. This session will be led by MDF and community leaders, and is designed to orient families to the Conference, to MDF, and to the DM Community.

Community Breakfast
10:00 am – 11:00 am
Join friends, family, and other attendees for breakfast!

Tanya Stevenson
EdD, MPH, MDF Chief Executive Officer

Mindy Buchanan
MDF Director of Programs

Andy Rohrwasser
PhD, MBA, MDF Chief Scientific Officer

Welcome & State of the Foundation
11:00 am – 11:45 am
MDF leadership will share exciting updates about the Foundation, International Myotonic Dystrophy Awareness Day, and discuss progress in the field of DM.

Networking Lunch: Networking Buddies Assigned Seating
Pre-registration required
1:25 pm – 2:15 pm | Capitol Ballroom 1 + 2
Get to know your fellow attendees over lunch. If you registered to participate in the Networking Buddies Program, please find your assigned table in Capitol Ballroom 1 + 2. See more about making the most out of the Networking Buddies program on page 23. Everyone else enjoy lunch with friends and family in Grand Ballroom 4 + 5!

Andy Rohrwasser
PhD, MBA, MDF Chief Scientific Officer

ARTHEx Biotech
Judy Walker
MD, FRCP(C), Chief Medical Officer

Avidity Biosciences
Kwadwo Bediako
SVP, DM1 Global Program Head

Juvena Therapeutics
Hanadie Yousef, *PhD, Co-Founder and CEO*

Dyne Therapeutics
Ash Dugar
PhD, MBA, Chief Medical Affairs Officer

Lupin Neurosciences
Alla Zozulya Weidenfeller, *PhD, Senior Director and Medical Lead*

Industry Updates Part 1
3:45 pm – 4:45 pm
With an opening statement from Dr. Andy Rohrwasser, MDF Chief Scientific Officer, representatives from biotechnology and pharmaceutical companies will provide updates on their drug development efforts in the the DM field, including DM2 and CDM.

Welcome Masquerade Murder Mystery Dinner & Entertainment
6:30 pm – 8:30 pm
Celebrate the end of Day 1 at the MDF 2025 Conference with an unforgettable Masquerade Murder Mystery Dinner! Interact with suspects from the comfort of your table, trade clues with other attendees, and unravel the mystery as the evening unfolds. Feel free to dress the part—don your finest attire, bring a Venetian mask, or embrace the elegance of the night. MDF will also provide masks for those who'd like to join in the masquerade. Dinner, one drink ticket, and a no-host bar will be included—get ready for a night of fun, mystery, and connection!

SATURDAY, MAY 3RD

Community Breakfast
9:00 am – 10:00 am
Join friends, family, and other attendees for breakfast!

Welcome & Stories of Inspiration
10:00 am – 11:00 am
Start the day off with a celebration of the DM community, as community leaders step onto the stage to receive special awards, including the 6th Annual Kayla Vittek Memorial Award for Outstanding Community Advocate, Support Group Facilitator Warrior Award, and MDF's Above and Beyond Award.

Lunch
1:45 pm – 2:45 pm
Get to know your fellow attendees over lunch. Join tables dedicated to your geographical region to meet community members and professionals in your area.

General Sessions

PepGen
Jenny Shoskes, *PharmD, Associate Director, Clinical Development*

Modalis Therapeutics
Keith Connolly, *PhD, Director of Molecular Biology and Genomics*

Vertex Pharmaceuticals
Asif Paker, *MD, MPH, Vice President, Clinical Development*

Arrakis
Sarah Mahoney, *PhD, Director, Discovery Biology*

Sanofi
Didier Rouy, *MD, PhD, Associate Group Project Head*

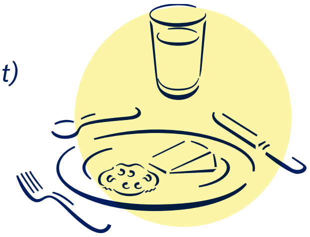
Industry Updates Part 2
2:45 pm – 3:45 pm
Our industry partners will provide updates on their drug development efforts in the DM Field, with a focus on DM1.

DM2 Community Members – Don't miss the Fireside Chat "There's Hope: A Discussion About the Future of DM2 Research" happening in House during this time. Join the conversation and stay informed about the latest advancements in DM2 research!

Closing Dinner & Dance Party!
6:30 pm – 8:30 pm
Join us for a dance party! Our DJ is playing everyone's favorite dance numbers so we can see your cool moves!

MDF is pleased to provide the following meals:

- Thursday evening advocacy award reception *(hors d'oeuvres & beverages)*
- Friday breakfast
- Friday lunch
- Friday evening dinner *(dinner & entertainment)*
- Saturday breakfast
- Saturday lunch
- Saturday dinner
- Sunday breakfast *(For Registered Workshop Attendees Only!)*



Tips to Help Maximize Your Conference Experience

1. **Divide & Conquer:** Make the most of simultaneous talks - attend different sessions if you're here with family or friends.
2. **Use the Breaks:** Enjoy ample breaks for networking, exploring, and recharging - stretch, mingle, or just chill!
3. **Take Notes:** Sessions are jam-packed with information - take notes so you can remember topics and follow up questions after the Conference.
4. **Seamless Recordings:** Enjoy recorded sessions on MDF's Digital Academy after the Conference.

   (Note, the following sessions will not be recorded: Community Discussion Panels, Networking & Social Hours, Stump the Doctor. Wellness Room sessions, Fireside Chats, and JOA Lounge activities are only available in person.)

Community Topics & Talks

Community Sessions are for everyone! They have been designed with community input, and have a universal focus for all people affected by DM1 or DM2 and their families.

FRIDAY, MAY 2ND

Seward B. Rutkove, *MD,*
Chair, Department of Neurology, Beth Israel Deaconess Center, Nancy Lurie Marks Professor of Neurology, Harvard Medical School

Your Guide to Clinical Trials: From Phases to Participation

12:00 pm - 1:00 pm | Caucus

This session provides an overview of the clinical trial process, helping attendees understand what to expect and how to prepare. It covers the different phases of trials, participant experiences, and key do's and don'ts. Attendees will leave with the knowledge needed to make informed decisions about trial participation.

Carolyn Martinez
M.A., CCC-SLP,
Houston Methodist

Anna Miller
MS, RD, LD,
Houston Methodist

Nourishing Solutions: Managing Dietary Needs & Swallowing with DM

12:00 - 1:00 pm | Cabinet

In this session, experts discuss how DM1 & DM2 impacts nutrition and swallowing. Attendees will learn about the latest research on these issues and gain practical advice on managing symptoms through dietary planning and adaptive tools that can improve quality of life.

Pam Glazener
OTR, ATP,
Houston Methodist

Wendy E. Schell
PT, DPT, MS, CSRS,
Houston Methodist

Living Better with DM: Tools & Services of Occupational Therapy & Home Health

5:00 pm – 6:00 pm | Cabinet

This session highlights how occupational therapy (OT) and home health services can improve the daily lives of those living with DM1 or DM2. Experts will discuss common mobility and self-care challenges and offer practical solutions through adaptive equipment and environmental modifications.

SATURDAY, MAY 3RD

Carol Applegate, *BSN, MSEd, MSN, JD,*
Indiana University Health

Securing Tomorrow: Financial Strategies & Planning for You & Your Family's Future

4:00 pm - 5:00 pm | Cabinet

This session addresses the financial challenges and future planning needs of individuals with DM1 & DM2 and their families. Topics include healthcare costs, creating a will, and strategies for reducing financial stress while planning for long-term care and legacy planning.

Beyond the Sessions

Discover MDF's free resources to support daily living with myotonic dystrophy:

Nutrition Guide for Individuals with DM
Written and reviewed by expert nutritionists, this guide covers DM-specific dietary needs—from constipation and weight management to swallowing support and sample meals.

Occupational Therapy Guidelines
A clinician-focused quick reference to help individuals with DM engage in meaningful daily activities.

Explore these and more at
www.myotonic.org/toolkits-publications

Taking Breaks

There are many breaks built into theconference agenda so that you can take care of personal needs, use this time to snap a picture, network, visit exhibit booths, check out research posters, and take in other Conference experiences.

Don't forget you may also attend sessions online. See **page 3** for detailed instructions on joining virtually!

DM1 Topics & Talks

These sessions were designed with community input from individuals living with DM1.

FRIDAY, MAY 2ND

Andrea Swenson, *MD,*
Medical Director,
University of Iowa Myotonic Dystrophy Clinic

Understanding DM1: From Genetics to Care & Everything in Between

12:00 pm - 1:00 pm | Grand Ballroom 1

This session provides an essential understanding of DM1, making it ideal for newly diagnosed individuals and those seeking to refresh their knowledge. Attendees will learn about the causes and genetics behind DM1, how the condition affects different systems of the body over time, and strategies for self-managing symptoms and building a strong clinical care team.

Katelyn Payne, *RN, CGC,*
Indiana University Health Neurology

Genetics Unveiled: Testing, Understanding Results, & Family Planning with DM1

2:30 pm – 3:30 pm | Grand Ballroom 1

This session offers a comprehensive look at the role of genetic testing and counseling in DM1. Attendees will learn about the testing process, how to interpret genetic results, the importance of working with a genetic counselor, and family planning considerations for those impacted by DM1.

Daniel Cruz, *MD, PhD,*
Professor of Medicine, Advanced Heart Failure
and Transplantation, Division of Cardiology
UCLA Health

Keeping Your Heart in Check: DM1's Impact & Care Tips

2:30 pm - 3:30 pm | Council

This session focuses on the cardiac complications associated with DM1. Participants will gain a deeper understanding of how DM1 affects heart function, the most common heart-related issues, and the best practices for self-management and care. The session will also cover the latest advancements in DM1 cardiac research, helping attendees stay informed about new developments.

DM1 Networking & Social Hour

5:00 pm – 6:00 pm | Grand Ballroom 1

Sponsored by Avidity Biosciences

Networking sessions at the conference provide safe and welcoming environments to connect and engage with your peers through discussions led by MDF Support Group Facilitators. A cash bar will be available prior to dinner.

SATURDAY, MAY 3RD

Sarah Clarke, *MD,*
DM1 Community
Member

Shaun Moore
DM1 Community
Member, MDF Volunteer
Support Group Facilitator

Chuck Hunt
DM1 Community
Member, MDF
Volunteer Support
Group Facilitator

Andrea Thacker Delcore
DM1 Community
Member

Community Discussion Panel: Those Affected by DM1: Sharing Hope & Life Hacks

11:15 am – 12:15 pm | Grand Ballroom 1

This panel session brings together members of the DM1 community to share personal experiences, practical tips, and life hacks that help them navigate daily life with DM1. Attendees will hear inspiring stories of resilience and learn creative solutions for managing symptoms, staying positive, and improving quality of life.

Cynthia Bodkin, *MD,*
Indiana University
Health

Gabriel Bosslet, *MD,*
Indiana University
Health

Breathe Easy, Sleep Sound: Managing Sleep & Pulmonary Health with DM1

4:00 pm – 5:00 pm | Grand Ballroom 1

In this session, attendees will explore how DM1 impacts sleep and respiratory health, two critical areas often affected by the disease. Hear from leading experts about common issues such as sleep disturbances and pulmonary complications, share the latest research findings, and provide practical advice for monitoring and managing these symptoms at home.

Jacinda Sampson, *MD, PhD,*
Clinical Associate Professor in Neurology
Stanford Neuroscience Health Center,
MDF Medical and Scientific Advisory Committee

Stump the Doctor: DM1

5:15 pm – 6:15 pm | Grand Ballroom 1

In this open Q&A format, participants get the chance to ask a leading expert in DM1 their toughest questions. Can you stump the doctor?

Beyond the Sessions

Understand Heart Health in DM

MDF's "Myotonic Dystrophy and the Heart: A Community Guide" aims to help people living with DM understand heart health risks and how they are managed.

SCAN ME!

DM2 Topics & Talks

These sessions were designed with community input from individuals living with DM2.

FRIDAY, MAY 2ND

Johanna Hamel, *MD,*
Assistant Professor of Neurology,
Pathology, and Laboratory Medicine
University of Rochester Medical Center

DM2 Essentials: Understanding, Managing, & Thriving with Myotonic Dystrophy Type 2
12:00 pm – 1:00 pm | Grand Ballroom 2
This session provides an essential understanding of DM2, making it ideal for newly diagnosed individuals and those seeking to refresh their knowledge. Attendees will learn about the causes and genetics behind DM2, how the condition affects different systems of the body over time, and strategies for self-managing symptoms and building a strong clinical care team.

Laurie Gutmann, *MD,*
Chair, Department
of Neurology,
Co-Director,
Neuroscience Institute,
Indiana University,
School of Medicine

Shashank Davé,
DO FAAPMR, Associate
Clinical Professor
of Physical Medicine,
Rehabilitation &
Neurology,
Indiana University,
School of Medicine

Finding Relief: Exploring Medications & Tools for Managing Pain in DM2
12:00 pm - 1:00 pm | Council
This session addresses pain commonly experienced by people with DM2. Attendees will learn about both pharmacological and non-pharmacological pain management options, with a focus on physical therapy and lifestyle modifications, as well as pain relief medications.

Araya Puwanant, *MD, MS,*
Wake Forest University School of Medicine

Mind Matters: Navigating Cognitive & Memory Challenges in DM2
2:30 pm – 3:30 pm | Grand Ballroom 2
This session delves into the cognitive challenges often faced by individuals with DM2, focusing on issues such as memory loss and executive function difficulties. Come to learn about the latest research on DM2's cognitive impact and practical techniques to help manage these symptoms in daily life.

DM2 Networking & Social Hour
5:00 pm – 6:00 pm | Grand Ballroom 2
Sponsored by Avidity Biosciences
Networking sessions at the conference provide safe and welcoming environments to connect and engage with your peers through discussions led by MDF Support Group Facilitators. A cash bar will be available prior to dinner.

SATURDAY, MAY 3RD

Judith Kroll
DM2 Community
Member

Elaine Samson
DM2
Community Member

Haley Martinelli, Esq
DM2 Community
Member, MDF
Volunteer Support
Group Facilitator,
MDF Board Member

Ryan Vogels
DM2 Community
Member, MDF
Volunteer Support
Group Facilitator

Community Discussion Panel:
Those Affected by DM2: Stories of Hope & Helpful Hacks
11:15 am – 12:15 pm | Grand Ballroom 2
In this panel session, individuals from the DM2 community will share their journeys, offering practical tips and life hacks for managing daily life with DM2. Attendees will gain insight into how others have overcome obstacles, discovered solutions for symptom management, and maintained a positive outlook.

Gabe Kringlen, *MS, LGC,*
University of Iowa Healthcare

Genetics Unveiled: Understanding Testing, Counseling & Family Conversations about DM2
4:00 pm – 5:00 pm | Grand Ballroom 2
This session explores the importance of genetic testing and counseling for individuals and families affected by DM2. Attendees will learn about the testing process, the role of genetic counselors, the importance of testing for registries and natural history studies, and planning for future generations.

Paloma Gonzalez-Perez, *MD, PhD,*
Massachusetts General Hospital

Stump the Doctor: DM2
5:15 pm – 6:15 pm | Grand Ballroom 2
In this open Q&A format, participants get the chance to ask a leading expert in DM2 their toughest questions. Can you stump the doctor?

There's More for DM2 Community Members at the Conference!

DM2 Research Fireside Chat
*See **page 17** for details.*

DM2 Active Research at Stanford & University of Florida
*See **page 29** for details.*

CDM Family Topics & Talks

These sessions were designed with community input from families with children living with congenital myotonic dystrophy (CDM). All CDM sessions are located in **Chamber**.

FRIDAY, MAY 2ND

Elizabeth Homes, *MBA, JD,*
Law Office of Elizabeth A Homes

Adulthood Ahead: Essential Planning for Parents of Children with CDM

12:00 pm – 1:00 pm

This session provides essential guidance for parents of children with Congenital DM as they prepare for their child's transition to adulthood. Topics include the legal, medical, and financial considerations in the United States to address before turning 18, as well as tips for building a strong support system to ensure a smooth transition.

CDM Family Networking & Social Hour

5:00 pm – 6:00 pm

Sponsored by Avidity Biosciences

Networking sessions at the conference provide safe and welcoming environments to connect and engage with your peers through discussions led by MDF Support Group Facilitators. A cash bar will be available prior to dinner.

Not Feeling Well?

We want to do our best to keep our community safe and healthy at this year's conference. If you are not feeling well, we ask that you please refrain from attending sessions in person. Fortunately, you'll still be able to engage in almost all the sessions live through our virtual platform, Zoom Events (see page 3 for information on how to connect to the virtual platform).

Please be current on vaccines, wear a mask if you've been sick recently, wash your hands with soap, and take all other precautions recommended by the CDC and local authorities.

SATURDAY, MAY 3RD

Laila Allen
CDM Family Member

Sarah Berman
CDM Family Member, MDF Volunteer Support Group Facilitator

Joy Bux
CDM Family Member

Cindy Hubert
CDM Family Member, MDF Volunteer Support Group Facilitator

Community Discussion Panel: CDM Family: Sharing Everyday Victories Through Useful Tips, Tricks, & Assistive Services

11:15 am – 12:15 pm

This community panel session provides attendees an opportunity to connect with other CDM families and learn valuable insights on supporting your CDM child. Panelists will provide practical tips for navigating everyday challenges like mobility and communication while also discussing assistive services and programs that have worked well for them.

Karly Sciortino-Poulter
Director of The Arc Advocacy Network,
The Arc Indiana

Empowering Adult Children: Managing Care, Service Delivery, & Living with CDM

4:00 pm – 5:00 pm

This session focuses on supporting an adult child with Congenital DM, offering strategies for promoting independence, navigating medical care, and exploring work and living options in the United States. Parents will leave with practical advice on how to best support their adult children as they grow and adapt.

Check out our other Community Topics and Talks on page 10

- Your Guide to Clinical Trials: From Phases to Participation
- Managing Dietary Needs & Swallowing with DM
- Securing Tomorrow: Financial Strategies & Planning for You & Your Family's Future
- Living Better with DM: Tools & Services of Occupational Therapy & Home Health

Caregiver Topics & Talks

This track was designed with community input from caregivers. Sessions for unaffected caregivers only are noted in the descriptions below.

FRIDAY, MAY 2ND

Brad Thompson, *LPC-S,*
The HALI Project

Building Resilience: Family Dynamics & Support in the DM Journey

2:30 pm – 3:30 pm | Grand Ballroom 3

This session examines how Myotonic Dystrophy affects family relationships, focusing on the emotional and logistical challenges caregivers face. Attendees will learn strategies for maintaining healthy family dynamics, fostering open communication, and providing effective support to loved ones living with DM. ***Open for all caregivers.***

Caregiver Networking & Social Hour

5:00 pm – 6:00 pm | Grand Ballroom 3

Sponsored by Avidity Biosciences

Networking sessions at the conference provide safe and welcoming environments to connect and engage with your peers through discussions led by MDF Support Group Facilitators. A cash bar will be available prior to dinner. ***Open for all caregivers.***

SATURDAY, MAY 3RD

Robert Campagna
MD, Unaffected Caregiver, MDF Board Member

Allan Posner
Unaffected Caregiver

Kyle Dunson
LCSW, Unaffected Caregiver, MDF Volunteer Support Group Facilitator

Ted Salwin
Unaffected Caregiver, MDF Volunteer Support Group Facilitator

Community Discussion Panel: Unaffected Male Caregivers: Prioritizing Self Care & Wellbeing

11:15 am – 12:15 pm | Grand Ballroom 3

Join a thoughtful panel of unaffected male caregivers as they discuss the unique challenges and rewards of supporting loved ones with DM. This session will offer honest conversations about maintaining mental and physical well-being while balancing caregiving responsibilities. Panelists will share practical self-care strategies, lessons learned, and insights on finding strength, connection, and resilience in their caregiving journeys. This sesssion is for ***unaffected caregivers only.***

Rose Albanese
Unaffected Caregiver, MDF Volunteer Support Group Facilitator

Julie Lebeouf
Unaffected Caregiver, MDF Volunteer Support Group Facilitator

Beth Feigenblatt
Unaffected Caregiver, MDF Volunteer Support Group Facilitator

Angie Wiggans
Unaffected Caregiver

Community Discussion Panel: Unaffected Female Caregivers: Practical Tips for Self Care

11:15 am – 12:15 pm | Council

Join an inspiring panel of unaffected female caregivers as they share hard-earned wisdom on prioritizing their own well-being while caring for loved ones. Discover practical, actionable self-care tips and learn how to give yourself permission to "put your oxygen mask on first" in this empowering discussion about balancing caregiving with self-care. This session is for ***unaffected caregivers only.***

Stacey B. Thacker, *MA, LMFT,*
Unaffected Caregiver

Unaffected Caregivers: Love in Transition: Balancing Romantic Connections & Caregiving in DM

4:00 pm – 5:00 pm | Grand Ballroom 3

This session explores how DM can affect romantic partnerships, offering practical advice for maintaining healthy relationships. The discussion will cover adjusting to changes in partnerships, communication strategies, and how to navigate the unique challenges that DM presents within a partnership. This session is for ***unaffected caregivers only.***

Check out our other Community Topics and Talks on page 10

- Your Guide to Clinical Trials: From Phases to Participation
- Managing Dietary Needs & Swallowing with DM
- Securing Tomorrow: Financial Strategies & Planning for You & Your Family's Future
- Living Better with DM: Tools & Services of Occupational Therapy & Home Health

Juvenile-Onset Adults Topics & Talks

This track was designed with community input from juvenile onset adults (JOA). JOA is often defined as someone 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 JOA Lounge in **The Rec Room** on the third floor.

Please note, *these sessions are only open to individuals living with JOA-DM.* All other attendees should participate in the other sessions.

FRIDAY, MAY 2ND

Welcome & Get to Know You Games
12:00 pm – 1:00 pm
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. The Lounge will be open to JOA members throughout the conference so plan to stop by and chill out whenever you need to relax!

Kelly Hartman, *MA,*
President & CEO, KQuad Consulting

Building Connections:
Gaining Confidence in Social Situations
2:30 - 3:30 pm
Join this session for a facilitated conversation focused around enhancing social skills for JOA's. Learn strategies for navigating social environments with confidence.

Evening Fun: Crafts, Games, Karaoke, & Movie Night
3:45 pm - 6:00 pm
Unwind and connect with friends in the JOA Lounge! Whether you're getting creative with crafts, playing games, singing your heart out at karaoke, there's something for everyone. Join us for our movie night where we'll be watching Inside Out 2! Hang out in the JOA Lounge for relaxed, fun-filled evenings designed to build community and make lasting memories.

The JOA lounge will have the following activities available during open lounge times:

- Card and board games
  (Uno, Balloon Volleyball, Mafia, etc.)
- Group Games
  (Selfie Challenges, Order Challenges)
- Crafts
  (Bracelet making, Coloring, etc)
- Karaoke
- Movie Night

SATURDAY, MAY 3RD

Donovan J. Lott, *PT, PhD, CSCS,*
Research Professor, University of Florida,
MDF Movement Committee

Let's Get Moving!
11:15 am – 12:15 pm
This session provides interactive exercises as well as practical advice on incorporating exercise into the daily routine of JOA community members.

Carolyn Martinez
M.A., CCC-SLP,
Houston Methodist

Anna Miller
MS, RD, LD,
Houston Methodist

Snack Smart: Making Easy, Healthy, Dysphasia Friendly, & Delicious Bites
2:30 - 3:30 pm
Join us for an interactive cooking demonstration designed for the JOA community, where you'll learn to prepare dysphagia-friendly and nutritious snacks. This hands-on session will provide practical tips for creating easy, healthy meals that support well-being while accommodating swallowing challenges. Connect with others, explore new recipes, and leave inspired to bring simple, delicious cooking into your daily routine.

Afternoon Fun: JOA Karaoke and Free Time
2:45 - 5:00 pm

Ericka Greene, *MD, FAAN, MACM,*
Houston Methodist

Stump the Doctor: JOA
5:15 pm – 6:15 pm
In this open Q&A format, participants get the chance to ask a leading expert in myotonic dystrophy their toughest questions. Can you stump the doctor?

Join the JOA Warriors every third Tuesday at 4pm Eastern online!

This virtual support group is for individuals diagnosed with DM as young adults.

Life Works Topics & Talks

New in 2025, this track was designed with community input from young adults living with DM. We are excited to introduce new Life Works sessions tailored to help young adults in their career and emotional well-being.

FRIDAY, MAY 2ND

Megan Stevenson, *MS,*
Director, Erskine Green Training Institute, The Arc Indiana

Landing the Job: Building Resumes & Acing Interviews
2:30 pm - 3:30 pm | Caucus
This session provides practical guidance on navigating the job search process, from creating a compelling resume to mastering interview techniques. Attendees will learn how to find a job that's the right fit for them and craft a resume that highlights their skills and experiences, even if they have limited job history. Experts will also share tips on how to prepare for interviews, including how to answer common questions, communicate both skills and accessibility needs effectively, and make a positive impression.

SATURDAY, MAY 3RD

Melissa Dixon, *PhD,*
Visiting Assistant Professor, Program Director, Utah Program for Inherited Neuromuscular Disorders, Dept. of Pediatrics, University of Utah

Thriving Under Pressure: Managing Mental Health in Everyday Life
4:00 pm - 5:00 pm | Caucus
In this session, young adults will explore the importance of mental health and learn strategies to manage stress, anxiety, and other challenges that may arise during transitions and navigating day to day life.

Check out our other Community Topics and Talks on page 10

- Your Guide to Clinical Trials: From Phases to Participation
- Managing Dietary Needs & Swallowing with DM
- Securing Tomorrow: Financial Strategies & Planning for You & Your Family's Future
- Living Better with DM: Tools & Services of Occupational Therapy & Home Health

Wellness Room Sessions

Take a break, rejuvenate, and focus on your well-being in the dedicated Wellness Room at the 2025 MDF Conference. This calming space, located in **Capitol Overlook North**, is designed to offer restorative activities that cater to all mobility levels. Whether you're looking to stretch, move, or simply relax in a quiet space, the Wellness Room has something for everyone.

Please note that Wellness Room sessions will NOT be provided virtually.

FRIDAY, MAY 2ND

Morning Self-Guided Meditation
9:00 am – 10:00 am
Start your day with calm and clarity in the Wellness Room. This self-guided meditation session offers a peaceful space to center yourself, reflect, and prepare for the day ahead. Take a moment to relax, breathe, and focus on your well-being at your own pace.

Gina Many, *PhD,*
Biomedical Scientist, Pacific Northwest National Laboratory

Chair & Stand Up Zumba
12:00 pm – 1:00 pm
Get moving with this fun, energetic Zumba session designed for all mobility levels.

Tina Duong, *MPT, PhD,*
Physical Therapist, Clinical Research Manager, Stanford University, MDF Movement Committee

Movements for Everyday Life
2:30 pm – 3:30 pm
This interactive session will provide attendees easy, practical movements to incorporate into daily routines for better mobility and health.

SATURDAY, MAY 3RD

Morning Self-Guided Meditation
9:00 am – 10:00 am
Start your day with calm and clarity in the Wellness Room. This self-guided meditation session offers a peaceful space to center yourself, reflect, and prepare for the day ahead. Take a moment to relax, breathe, and focus on your well-being at your own pace.

Lorna Jarrett, *DHSc, LPTA, NCPT,*
Owner, Brain Peace-Mindset Coaching and Consulting, Adaptive Living Solutions

Afternoon Gentle & Chair Yoga
12:30 pm – 1:30 pm
A gentle yoga session tailored to individuals with DM. Practice breathing techniques, stretching, and relaxation from the comfort of a chair.

Fireside Chats

Engage with leading experts in insightful small group discussions covering a wide range of topics. All Fireside Chats are located in **House**.

Please note that Fireside Chats will NOT be provided virtually.

FRIDAY, MAY 2ND

Jacinda Sampson *MD, PhD,*
Clinical Associate Professor in Neurology, Stanford Neuroscience Health Center, MDF Medical and Scientific Advisory Committee

You've Just Been Diagnosed, What's Next?
12:00 pm – 1:00 pm
An intimate discussion on what to expect after a diagnosis of DM. Learn about resources, medical care, and building a support network.

Helen Bernie, *DO, Indiana University Health*

Addie Jacobs, *MS, OTD, OTR, CSOT, Indiana University Health*

Affected Men's Real Talk:
Managing Intimacy & Sexual Health Challenges with DM
2:30 pm – 3:30 pm
A candid conversation about how DM affects men's sexual health and intimacy. Gain tools for managing these changes and maintaining healthy relationships. *Affected men only.*

Alain Geille
Head of the AFM-Téléthon French DM Patient Group, Founder of Euro-DyMA, the European Federation of Patient Associations Dealing with DM

Bas Haasakker
MA, Chair of the Dutch subgroup for DM1 of the Dutch National organization for Muscle diseases, Co-Founder of Euro-DyMA, the European Federation of Patient Associations Dealing with DM

European Patient Organizations:
A Unique Opportunity to Share and Celebrate
5:00 pm – 6:00 pm
Join this session to hear about Europe's latest updates including clinical trials, families support, and scientific programs.

SATURDAY, MAY 3RD

Cynthia Gagnon, *PhD, Faculty of Medicine and Health Sciences at Sherbrooke University, MDF Medical and Scientific Advisory Committee*

Loralei L. Thornburg, *MD, Maternal Fetal Medicine Program, University of Rochester Medical Center*

Affected Women's Real Talk: Managing Intimacy & Sexual Health Challenges with DM
11:15 am – 12:15 pm
A discussion on the unique challenges women with DM face in their sexual and reproductive health, with practical advice and resources. *Affected women only.*

Maurice S. Swanson, *PhD, Professor and Associate Director, Department of Molecular Genetics and Microbiology Center for NeuroGenetics and the Genetics Institute, University of Florida*

There's Hope: A Discussion About the Future of DM2 Research
2:45 pm – 3:45 pm
Join us for an inspiring discussion on DM2 research. Hear from a leading researcher in the DM2 space about ongoing research and the future implications on DM2.

Kevin Brennan
Principal, Bluebird Strategies, MDF Advocacy Consultant

Voices for DM: Open Discussion on Advocacy
4:00 pm – 5:00 pm
This session focuses on empowering DM community members to become advocates for better care and research funding. Learn how to advocate for yourself and your loved ones and get involved in DM advocacy at the local and national levels.

Take A Picture!

Don't let Conference memories fade away; showcase your experience! Post to social media, tag **@MyotonicStrong**, and use the hashtags **#MDF2025** and **#MyotonicDystrophy** to amplify your voice, create lasting memories, and raise awareness for myotonic dystrophy.

Don't forget to check out MDF's step and repeat located near registration for a fun background to your conference photos!

Professionals Topics & Talks

All professional sessions, including breakfast, are located in **Capitol Ballroom 1 + 2.**

Professional sessions will cover cardiac care, clinical trial endpoints, and cutting-edge advancements in gene editing, diagnostics, and patient-centered research. The program includes expert-led discussions, insights from the French DM Registry, and fast-paced presentations of groundbreaking studies during the Research Poster Lightning Round.

Young investigators are also invited to participate in career workshops offering tailored guidance on navigating academia, industry, and funding opportunities.

Thank you to our Cardiac Endpoints Workshop and Pharma Day sponsors **Avidity Biosciences**, **Dyne Therapeutics**, **Lupin Neurosciences**, **PepGen**, **Sanofi**, and **Vertex Pharmaceuticals**. Thank you to **Arrakis** and **ARTHEx Biotech** for their sponsorship of Pharma Day. *Cardiac Endpoints and Pharma Day are open to those with Professional registrations only.*

THURSDAY, MAY 1ST

Cardiac Endpoint Workshop in partnership with the Myotonic Dystrophy Clinical Research Network (DMCRN)

Nicholas E. Johnson, *MD, MSci, FAAN,*
Professor and Vice Chair of Research in Neurology,
Virginia Commonwealth University,
MDF Medical and Scientific Advisory Committee

Welcome & Introduction
12:00 pm - 12:05 pm

William J. Groh, *MD, MPH,*
Interim Chief of Staff, RH Johnson VAMC, Affiliate Professor of Medicine Medical University of South Carolina

EP, Cardiac Rhythms in DM: Arrhythmias & Their Impacts
12:05 pm – 12:20 pm
This session will review the impact of cardiac dysfunction in individuals living with myotonic dystrophy and the necessity to develop meaningful cardiac endpoints in clinical trials.

Tom Cooper, *MD,*
S. Donald Greenberg and R. Clarence & Irene H. Fulbright Professor
Baylor College of Medicine, MDF Medical and Scientific Advisory Committee

Mechanism of Cardiac Dysfunction in Myotonic Dystrophy
12:20 pm – 12:40 pm
This session will review the pathophysiology of cardiac dysfunction in myotonic dystrophy.

Jordana Kron, *MD,*
Professor of Cardiology
Virginia Commonwealth University

Measuring Cardiac Arrhythmias in Clinical Trials
12:40 pm – 1:00 pm
This session will review standard arrhythmia endpoints for consideration in clinical trials.

Discussion
1:00 pm – 1:30 pm

Greg Hundley,
MD, Professor School of Medicine Internal Medicine Virginia Commonwealth University

Jennifer Jordan,
Ph.D., M.S., FSCMR, Assistant Professor and ABET Coordinator, Department of Biomedical Engineering, Virginia Commonwealth University

Amy Ladd, *PhD,*
Associate Director, Pauley Heart Center

Role of Cardiac Imaging & Exercise in Cardiovascular Clinical Trials
1:30 pm - 2:00 pm
This session will review the use of cardiac imaging in clinical trials.

Discussion
2:00 pm – 2:30 pm

Pharma Day in Partnership with Euro-DyMA

Alain Geille
Head of the AFM-Téléthon French DM Patient Group, Founder of Euro-DyMA, the European Federation of Patient Associations Dealing with DM

Tanya Stevenson
EdD, MPH, MDF Chief Executive Officer

Bas Haasakker
MA, Chair of the Dutch subgroup for DM1 of the Dutch National organization for Muscle diseases, Co-Founder of Euro-DyMA, the European Federation of Patient Associations Dealing with DM

Andy Rohrwasser
PhD, MBA, MDF Chief Scientific Officer

Welcome & Introduction
3:00 pm - 3:10 pm

Professionals Topics & Talks

Nicholas E Johnson, *MD, MSci, FAAN*
Professor and Vice Chair of Research in Neurology, Virginia Commonwealth University, MDF Medical and Scientific Advisory Committee

Updates From END-DM1
3:10 pm - 3:20 pm

Keith Connolly, *PhD,*
Director of Molecular Biology and Genomics

Modalis Therapeutics
3:20 pm -3:40 pm

Isobelle Galeon
MD, MSCS, MPH, Clinical Research Director for the Rare Disease Therapeutic Area

Karin Knobe
MD, PhD, Global Head of Rare Disease

Martin Goulet
PhD, Cluster Head of Rare, RBD, and Neuromuscular Disease

Christian Mueller
PhD, Global Head of the Genomic Medicine Unit

Sanofi
3:40 pm - 4:00 pm

Alison McVie-Wylie, *PhD,*
Disease Area Executive, Muscular Dystrophies

Vertex Pharmaceuticals
4:00 pm - 4:20 pm

Sarah Mahoney, *PhD,*
Director, Discovery Biology

Arrakis
4:20 pm - 4:40 pm

Break
4:40 - 4:50 pm

Jeffrey Haroldson, *PharmD, MBA,*
Executive Director, Global Medical Affairs

Avidity Biosciences
4:50 pm -5:00 pm

Ash Dugar, *PhD, MBA,*
Chief Medical Affairs Officer

Dyne Therapeutics
5:00 pm - 5:10 pm

Jane Larkindale, *DPhil,*
VP of Clinical Science, PepGen

PepGen
5:10 pm - 5:20 pm

Alla Zozulya Weidenfeller, *PhD,*
Senior Director and Medical Lead

Lupin Neurosciences
5:20 pm - 5:30 pm

Judy Walker, *MD, FRCP(C),*
Chief Medical Officer

ARTHEx Biotech
5:30 pm - 5:40 pm

Hanadie Yousef, *PhD,*
Co-Founder and CEO

Juvena Therapeutics, Inc.
5:40 pm - 5:50 pm

FRIDAY, MAY 2ND

DM Professionals Breakfast
8:00 am - 9:00 am

Professional Session 1: Progress & Possibilities for DM Research

Andy Berglund
PhD, Professor, Director, The RNA Institute, Department of Biological Sciences at University of New York at Albany, MDF Medical and Scientific Advisory Committee Chair, MDF Board Member

Andy Rohrwasser
PhD, MBA, MDF Chief Scientific Officer

Nadine Ann Skinner
PhD, MPA, MDF Director of Evaluation and Research Programs

Welcome & Conference Logistics
9:00 am – 9:30 am
Join this session for an introduction to the professional track sessions, highlighting the key conference themes. Also learn essential information on scheduling, sessions, and opportunities for networking and collaboration.

Maurice S. Swanson, *PhD,*
Professor and Associate Director, Department of Molecular Genetics and Microbiology Center for NeuroGenetics and the Genetics Institute, University of Florida

Charting the Past: Historical Perspectives
9:30 am – 10:00 am
From gene discoveries, gain of function RNA, to the personalized DM therapies of tomorrow.

Professionals Topics & Talks

FRIDAY, MAY 2ND *(CONT.)*

Guillaume Bassez, *MD, PhD,*
Institut de Myologie, MDF Medical and Scientific Advisory Committee

Harnessing Patient Data: Current Therapeutic Approaches & Regulatory Insights from the French Registry
10:00 am – 10:30 am
The power of integrated and unified registries; an international blueprint?

Antoine Mangin, *PhD,*
Cardiff University, UK Dementia Research Institute

Innovations & Prospects for Gene Editing & Tuning
10:30 am – 11:00 am
Interventional strategies targeting the cause of DM, where are we?

Professional Session 2: Innovations in Genomics & Diagnostics

Darren Monckton, *PhD,*
University of Glasgow, MDF Medical and Scientific Advisory Committee

Genomics Unleashed: Insights & Implications
11:50 am – 12:20 pm
The causal repeats and interactions with modifier loci: Lessons and emerging knowledge from other repeat expansion diseases.

Amanda Singleton, *CGC*
Senior Director, Biopharma and Patient Advocacy
GeneDx

Cutting-Edge Techniques: Perspectives on Evolving Diagnostic Tools
12:20 pm – 12:50 pm
Long-read, short read sequencing technologies and bioinformatic solutions: From discovery tools to standards of clinical care.

Andreas Roos, *PhD,*
Adjunct Professor, University of Ottawa

Tech Integration: Omics & Beyond in Biomarker Research
12:50 pm – 1:15 pm
Connecting it all: repeat size, repeat composition, modifier loci and biomarkers of the disease.

Professional Session 3: Patient Perspectives & New Tools

Moderator Nicholas E. Johnson
MD, MSci, FAAN, Virginia Commonwealth University, MDF Medical and Scientific Advisory Committee

Jeannine DeSoi
BSN, RN, MDF Volunteer Support Group Facilitator, DM1 Community Member

Belen Esparis
MD, MDF Board of Directors, MDF National Advocacy Committee, Unaffected Caregiver

Patient Perspectives: Enhancing Studies & Clinical Trials
2:15 pm – 3:10 pm
Individuals with DM know the most: Listening to what is most relevant to the DM community and integration of this knowledge into research and clinical trial design.

Charles Thornton, *MD,*
Saunders Family Distinguished Professor in Neuromuscular Research, University of Rochester School of Medicine, MDF Medical and Scientific Advisory Committee, MDF Board of Directors

Classical DM1 with Large CTG Expansion at the Mouse Locus
3:10 pm – 3:40 pm
Emerging knowledge from novel mouse models: Replicating the human disease.

Professional Networking & Research Poster Showcase
5:00 pm – 6:30 pm
Dedicated time for DM professionals to connect, exchange ideas, and explore collaborative opportunities across disciplines, while viewing an exciting display of innovative research by young investigators and industry leaders, spotlighting cutting-edge developments in the DM field.

Apply for MDF Research Grants!

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

Apply now!
www.myotonic.org/grants

Professionals Topics & Talks

SATURDAY, MAY 3RD

DM Professionals Breakfast
7:30 am – 8:30 am

Professional Session 4:
DM & Body Systems

Eric Wang, *PhD,*
Associate Professor, Molecular Genetics & Microbiology, University of Florida, MDF Medical and Scientific Advisory Committee, MDF National Advocacy Committee

CNS Symptoms and Meaningful Clinical Trial Endpoints
8:30 am – 9:00 am
A summary from the clinical endpoint workshop focusing on CNS clinical presentations and meaningful endpoints in clinical trials.

Belinda Pinto, *PhD,*
Assistant Scientist, University of Florida

Highlighting New Research:
Excessive Daytime Sleepiness
9:00 am – 9:15 am
New research strategies aiming to understand CNS presentations and the common problem of daytime sleepiness.

Paloma Gonzalez-Perez, *MD, PhD,*
Massachusetts General Hospital

Highlighting New Research: Exercise in the DM2 population
9:15 am – 9:30 am
New research strategies emphasizing the importance of exercise in DM2.

Auinash Kalsotra, *PhD,*
Assistant Professor of Biochemistry, University of Illinois Urbana-Champaign

DM1 Adversely Affects Liver Function
& Lipid Metabolism
9:30 am – 10:00 am
DM1 and liver function: Not just important for the pharmacology of new drugs.

Moderator
Tom Cooper, *MD,*
Baylor College of Medicine, MDF Medical and Scientific Advisory Committee

Young Investigators' Poster Presentation:
Lightning Round Showcase
11:00 am - 12:30 pm
A fast-paced session where young investigators present their groundbreaking research, offering a glimpse into the future of DM studies.

Exhibitor & Research Poster Showcase
12:30 pm – 1:30 pm
An exciting display of innovative research by young investigators and industry leaders, spotlighting cutting-edge developments in the DM field.

Moderator
Andy Berglund, *PhD,*
Professor, Director, The RNA Institute, Department of Biological Sciences at University of New York at Albany, MDF Board Member

Moderator
Eric Wang, *PhD,*
Associate Professor, Molecular Genetics & Microbiology, University of Florida, MDF Medical and Scientific Advisory Committee, MDF National Advocacy Committee

Young Investigators' Workshop
3:50 – 6:30 pm

Johanna Hamel, *MD,*
Assistant Professor of Neurology, Pathology, and Laboratory Medicine, University of Rochester

Lukasz Sznadjer, *PhD,*
Assistant Professor, University of Nevada, Las Vegas

Career Paths: Academia Insights
Hear about careers in academia, from securing faculty positions, balancing research and teaching, to navigating tenure.

Ranjan Batra, *PhD,*
Vice President of Discovery, Research, & Translation, Lexeo Therapeutics

Jana Jenquin, *PhD,*
Senior Scientist, Novartis

Career Paths: Industry Insights
Career opportunities in industry, key skills for success, and how industry careers compare to academic paths.

Andy Rohrwasser, *PhD, MBA,*
MDF Chief Scientific Officer

Career Paths: AltAca Insights
Life outside academia and industry highlighting important roles in nonprofits, policy, foundations, financial institutions, and government, and how to leverage research skills in these fields.

Mentor 1:1
Funding Your Future: Aims Review & Mentor Guidance
Successful (grant) writing as an important skill set
(not just in academia).

Myotonic Dystrophy In Motion

Back by popular demand Myotonic Dystrophy in Motion **Movement Movements** will provide short movement demonstrations for all conference attendees, each lasting a few minutes. Each Movement Moment will focus on one of the four types of movement: endurance, strength, stretching, and balance. **All Movement Moments will take place in Grand Ballroom 4 + 5.** In addition to these short participatory demonstrations, all conference attendees are encouraged to join the **Movement Sessions** in the Wellness Room. You can learn more about all the Wellness Room activities on page 16.

The hope with Movement Moments and Movement Sessions is to emphasize the power of movement in every person's life. The movement experts and community members who designed this program, will provide small examples of ways to move together, safely and joyfully. All are welcome to participate if it is comfortable for you or simply observe. Note: adaptations for movements will be demonstrated.

FRIDAY, MAY 2ND

Friday Morning Movement Moment:

STRETCHING
11:45 am – 12:00 pm

Stretching increases flexibility and extensibility of the muscles and tendons. Stretches should be held for 60 seconds or 2-3 repetitions of 30 seconds each. Stretching requires a joint to be moved through your range of motion.

Ellen Shapiro, *C-IAYT, a certified yoga instructor and family member of a person living with DM1, will guide us through a session of gentle seated yoga as we use our breath to explore six basic movements of the spine. These can also be performed at home with little supervision.*

Friday Afternoon Movement Moment:

ENDURANCE EXERCISES
4:45 pm – 5:00 pm

Endurance exercises increase the heart rate and respiratory rate (breathing). Endurance activities should be performed at moderate intensity. You should be able to talk but not sing while doing the movements.

Gina Many, *PhD, is a health enthusiast with a BS in Exercise Science and a Master's degree is Exercise Physiology. Dr. Many will guide the audience through modified activities to get your body moving.*

SATURDAY, MAY 3RD

Saturday Morning Movement Moment:

BALANCE
11:00 am – 11:15 am

Balance training is a combination of exercises that challenge your sensory systems and strengthen the muscles that keep you upright including your core, trunk and legs. Balance training improves stability and helps prevent falls.

Tina Duong, *MPT, PhD, is the Director of Clinical Research and Development at Stanford University Neuromuscular division. Dr. Duong will guide the audience through Tai Chi, a Chinese practice based on slow and fluid movements that can help with balance.*

Saturday Afternoon Movement Moment:

STRENGTH
3:45 pm – 4:00 pm

Strength training can involve a variety of exercises that increase the strength of your muscles. Examples could include exercises that involve lifting weights or appropriately heavy objects, elastic resistance bands, or bodyweight exercises like squats or push-ups.

Kristina Kelly, *PT, DPT, MS, EdM, NCS, CPT, PES, is an Assistant Research Professor in the Department of Physical Medicine & Rehabilitation at the University of Missouri. Dr. Kelly will guide attendees through adaptive Active joint movement and stretching activities.*

Explore the MDF Exercise Guide & Infographic:
www.myotonic.org/toolkits-publications.

Engage with the Myotonic Dystrophy In Motion initiative:
www.myotonic.org/in-motion.

We are grateful to the **MDF Movement Committee** for working diligently to build out movement based programs. These programs help community members find new and safe ways to exercise. Tina Duong, MPT, PhD; Katy Eichinger, PhD, DPT; Donovan Lott, PT, PhD, CSCS; Nathan Beucler; Luke Bolt; Lorrie Gallagher; Margaret Singleton, MPH, Ryan Vogels

Myotonic
Dystrophy
IN MOTION
www.myotonic.org

Networking Buddies

Be the bridge between the DM Community & Research Professionals!

Returning in 2025 is our Networking Buddies program which fosters exciting ways to get to know one another. The Conference Networking Buddies system pairs DM Professionals with people living with myotonic dystrophy and their caregivers so that they can learn more about the lived experience of DM and community members can learn about the work of DM professionals.

Friday lunch for those registered to participate in the Networking Buddies program will be held in a different location! **Networking Buddies will be assigned a table number at check-in for Friday's Networking Lunch in the Capitol Ballroom.** Networking Buddies will be seated together. We encourage you to continue to connect with your buddies throughout the conference during breaks, sessions, and mealtimes

Community Workshops

New to this year's conference, MDF is excited to offer Sunday Morning Workshops, providing attendees with the opportunity for personalized, one-on-one consultations with experts in key areas of myotonic dystrophy care and support. **These workshops are available by appointment only to attendees who indicated interest during registration and received confirmation from MDF.** Workshops will take place on May 4th from 9 am - 12 pm in **Grand Ballrooms 1-3**.

During these interactive sessions, professionals will be available to discuss individualized concerns and provide expert guidance in the following areas:

- **Genetic Counseling and Family Planning** – Understanding genetic risks, testing options, and reproductive choices.
  - Karrie Hines Theoharis, PhD, MS, LCGC, Genetic Counselor
  - Gabriel Kringlen, MS, LGC, University of Iowa Healthcare
    Katelyn Payne, RN, CGC, Indiana University Health Neurology
  - Bethany Pollock, MS, CGC, Indiana University Health
  - Abigayle Rose, MS, Indiana University Health
  - Kelly Samson, LCGC, Genetic Counselor, Community Health Network
  - Allison Schartman, MS, CGC, Indiana University Health Maternal Fetal Medicine

- **Accessible and Adaptive Devices** – Strategies for improving mobility, strength, and daily function.
  - Pam Glazener, OTR, ATP, Houston Methodist
  - Wendy E. Schell, PT, DPT, MS, CSRS, Houston Methodist

- **The Patient Advocate Foundation** – Assistance with navigating healthcare systems, insurance, and financial resources.
  - Kimberly Humphrey, Case Manager
  - Courtney Jones, Senior Director of Case Management
  - Rita Matthews, Case Manager
  - Amber Padron, Assistant Director of Case Management
  - Beth Scott, Director of Case Management

- **Legacy Planning** – Guidance on wills, trusts, and long-term financial security for individuals with myotonic dystrophy and their families.
  - Carol Applegate, BSN, MSEd, MSN, JDJD, RN, Indiana University
  - Sarah Geis, Senior Director of Engagement, The Arc Indiana

MDF would like to honor and remember the lives of two Volunteer Support Group Facilitators who passed away this past year.

Glenda Winson was an essential part of the New York City/New Jersey Support Group and had been facilitating that group beginning in 2022, until her passing in September 2024, she was a beacon of compassion and understanding for so many within the myotonic dystrophy community.

Scott Virgo first started facilitating the Michigan Support Group in 2020, and continued in that role until his passing in February 2025. Scott was a beloved member of support groups across the midwest, and in the DM2 community. His positivity, strength and support impacted many in the communities he served.

Living with Myotonic Dystrophy?

You can Help Change the Future of DM Research. Join the Myotonic Dystrophy Family Registry (MDFR).

The MDFR is a patient self-reported online database gathering data from individuals affected by myotonic dystrophy (DM).

The more participants provide information through the Registry surveys and uploaded medical reports, the closer we get to understanding this disease and support efforts to find DM treatments and a cure.

By Joining the Registry, You Will:

- Help community members and health professionals learn more about the scope and impact of myotonic dystrophy
- Speed up research and care in myotonic dystrophy by providing critically needed information to scientists pursuing treatments and a cure for DM
- Help the Myotonic Dystrophy Foundation identify and organize the DM patient community for clinical trials and studies
- Review anonymous registry data on symptoms, demographics and more

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IT'S EASY • IT'S CONFIDENTIAL • THERE IS NO COST TO JOIN

Your voice counts and we need to hear from you!

Questions?

Contact the MDFR coordinator:

Sofia Olmos, PhD
415-800-777
coordinator@myotonicregistry.org
www.myotonicregistry.org

Log in now
www.myotonicregistry.org

Research Poster Abstracts Available Online!

Traditional poster sessions combine a visual representation of the findings of a paper/study – the poster - with the opportunity for individualized, informal discussion of the presenter's work. MDF is thrilled to make this opportunity available at this year's conference in a virtual format, where remote attendees can view posters.

www.myotonic.org/poster-abstracts-2025

The 2026 Conference Countdown Begins!

Get ready, we're gearing up to announce all the details for 2026—including dates, locations, and exciting partnerships!

Stay tuned for the big reveal and start planning to connect, learn, and grow with the DM community.

www.myotonic.org/countdown

SCAN ME!

Conference Speakers

Rose Albanese
MDF Volunteer Support Group Facilitator, Unaffected Caregiver
Unaffected Female Caregivers: Practical Tips for Self Care

Laila Allen
CDM Family Member
CDM Community Panel: Sharing Everyday Victories Through Useful Tips, Tricks, & Assistive Services

Carol Applegate, BSN, MSEd, MSN, JD
Indiana University Health
Securing Tomorrow: Financial Strategies & Planning for You & Your Family's Future

Guillaume Bassez, MD, PhD
Institut de Myologie, MDF Medical and Scientific Advisory Member
Harnessing Patient Data: Current Therapeutic Approaches & Regulatory Insights from the French Registry

Ranjan Batra, PhD
Lexea Therapeutics
Industry Insights: Careers Beyond Academia

Kwadwo Bediako
Avidity Biosciences
Industry Updates Part 1

Andy Berglund, PhD
University of Albany, MDF Medical and Scientific Advisory Committee Chair, MDF Board Member
Young Investigators Moderator
Host of Professional Sessions

Sarah Berman
MDF Volunteer Support Group Facilitator, CDM Family Member
CDM Community Panel: Sharing Everyday Victories Through Useful Tips, Tricks, & Assistive Services

Helen Bernie, DO
Indiana University Health
Affected Men's Real Talk: Managing Intimacy & Sexual Health Challenges with DM

Cynthia Bodkin, MD
Indiana University Health
Breathe Easy, Sleep Sound: Managing Sleep & Pulmonary Health with DM1

Gabriel Bosslet, MD
Indiana University Health
Breathe Easy, Sleep Sound: Managing Sleep & Pulmonary Health with DM1

Kevin Brennan
Bluebird Strategies, MDF Advocacy Consultant
Voices for DM: Open Discussion on Advocacy

Mindy Buchanan
MDF Director of Programs
Welcome & State of the Foundation
Welcome & Stories of Inspiration

Joy Bux
CDM Family Member
CDM Community Panel: Sharing Everyday Victories Through Useful Tips, Tricks, & Assistive Services

Robert Campagna, MD
MDF Board Member, Unaffected Caregiver
Unaffected Male Caregivers: Prioritizing Self Care & Wellbeing

Sarah Clarke, MD
DM1 Community Member
DM1 Community Panel: Sharing Hope & Life Hacks

Conference Speakers *(continued)*

Keith Connolly, PhD
Modalis Therapeutics
Pharma Day
Industry Updates Part 2

Tom Cooper, MD
Baylor College of Medicine, MDF Medical and Scientific Advisory Committee
Cardiac Endpoint Workshop: Mechanism of Cardiac Dysfunction in Myotonic Dystrophy
Research Poster Lightening Round Moderator

Daniel Cruz, MD, PhD
UCLA Health
Keeping Your Heart in Check: DM1's Impact & Care Tip

Shashank Davé, DO FAAPMR
Indiana University School of Medicine
Finding Relief: Exploring Medications & Tools for Managing Pain in DM2

Jeannine DeSoi, BSN, RN
MDF Volunteer Support Group Facilitator, DM1 Community Member
Patient Perspectives: Enhancing Studies & Clinical Trials

Melissa Dixon, PhD
University Of Utah
Thriving Under Pressure: Managing Mental Health in Everyday Life

Ash Dugar, PhD, MBA
Dyne Therapeutics
Pharma Day
Industry Updates Part 1

Kyle Dunson, LCSW
MDF Volunteer Support Group Facilitator, Unaffected Caregiver
Unaffected Male Caregivers: Prioritizing Self Care & Wellbeing

Tina Duong, MPT, PhD
Stanford University, MDF Movement Committee
Fireside Chat: Movements for Everyday Life
Movement Moment

Belen Esparis, MD
MDF Board Member,
MDF National Advocacy Committee,
Unaffected Caregiver
Patient Perspectives: Enhancing Studies and Clinical Trials

Beth Feigenblatt
MDF Volunteer Support Group Facilitator, Unaffected Caregiver
Unaffected Female Caregivers: Practical Tips for Self-Care

Cynthia Gagnon, PhD
Sherbrooke University, MDF Medical and Scientific Advisory Committee
Affected Women's Real Talk: Managing Intimacy & Sexual Health Challenges with DM

Isobelle Galeon, MD, MSCS, MPH
Sanofi
Pharma Day

Alain Geille
Euro-DyMA
Pharma Day Welcome & Introduction
European Patient Organizations: A Unique Opportunity to Share and Celebrate

Pam Glazener, OTR, ATP
Houston Methodist
Living Better with DM: Tools & Services of Occupational Therapy & Home Health

Paloma Gonzalez-Perez, MD, PhD
Massachusetts General Hospital
DM2 Stump the Doctor
Highlighting New Research: Exercise in the DM2 Population

Martin Goulet, PhD
Sanofi
Pharma Day

Ericka Greene, MD, FAAN, MACM
Houston Methodist Hospital
JOA Stump the Doctor

William J. Groh, MD, MPH
Medical University of South Carolina
Cardiac Endpoint Workshop: EP, Cardiac Rhythms in DM: Arrhythmias & Their Impacts

Laurie Gutmann, MD
Indiana University School of Medicine, Indiana University Health
Finding Relief: Exploring Medications & Tools for Managing Pain in DM2

Johanna Hamel, MD
University of Rochester Medical Center
DM2 Essentials: Understanding, Managing, & Thriving with Myotonic Dystrophy Type 2
Career Paths: Academia Insights

Jeffrey Haroldson, PharmD, MBA
Avidity Biosciences
Pharma Day

Kelly Hartman, MA
KQuad Consulting
Building Connections: Gaining Confidence in Social Situations

Bas Haasakker, MA
Euro-DyMA
European Patient Organizations: A Unique Opportunity to Share and Celebrate

Elizabeth Homes, MBA, JD
Law Office of Elizabeth A Homes
Adulthood Ahead: Essential Planning for Parents of Children with CDM

Cindy Hubert
MDF Volunteer Support Group Facilitator, CDM Family Member
CDM Community Panel: Sharing Everyday Victories Through Useful Tips, Tricks, & Assistive Services

Greg Hundley, MD
Virginia Commonwealth University
Cardiac Endpoint Workshop: Role of Cardiac Imaging & Exercise in Cardiovascular Clinical Trials

Chuck Hunt
MDF Volunteer Support Group Facilitator, DM1 Community Member
DM1 Community Panel: Sharing Hope & Life Hacks

Addie Jacobs, MS, OTD, OTR, CSOT
Indiana University Health
Affected Men's Real Talk: Managing Intimacy & Sexual Health Challenges with DM

Lorna Jarrett, DHCs, LPTA, NCPT
Brain Peace-Mindset Coaching and Consulting, Adaptive Living Solutions
Wellness Room: Chair & Gentle Yoga

Jana Jenquin, PhD
Novartis
Industry Insights: Careers Beyond Academia

Nicholas E. Johnson, MD, MSci, FAAN
Virginia Commonwealth University, MDF Medical and Scientific Advisory Committee
Cardiac Endpoint Workshop Welcome & Introduction
Pharma Day Updates From END-DM1
Moderator: Patient Perspectives: Enhancing Studies and Clinical Trials

Jennifer Jordan, Ph.D., M.S., FSCMR
Virginia Commonwealth University
Cardiac Endpoint Workshop: Role of Cardiac Imaging & Exercise in Cardiovascular Clinical Trials

Auinash Kalsotra, PhD
University of Illinois at Urbana-Champaign
DM1 Adversely Affects Liver Function & Lipid Metabolism

Kristina Kelly, PT, DPT, MS, EdM, NCS, CPT, PES
University of Missouri
Movement Moment

Karin Knobe, MD, PhD
Sanofi
Pharma Day

Gabe Kringlen, MS, LGC
University of Iowa Healthcare
Genetics Unveiled: Understanding Testing, Counseling & Family Conversations about DM2

Judith Kroll
DM2 Community Member
DM2 Community Panel: Stories of Hope & Helpful Tips

Jordana Kron, MD
Virginia Commonwealth University
Cardiac Endpoint Workshop: Measuring Cardiac Arrhythmias in Clinical Trials

Amy Ladd, PhD
Pauley Heart Center
Cardiac Endpoint Workshop: Role of Cardiac Imaging & Exercise in Cardiovascular Clinical Trials

Jane Larkindale, DPhil
PepGen
Pharma Day

Julie Lebeouf
MDF Volunteer Support Group Facilitator, Unaffected Caregiver
Unaffected Female Caregivers: Practical Tips for Self Care

Donovan J. Lott, PT, PhD, CSCS
University of Florida,
MDF Movement Committee
JOA Session: Let's Get Moving!

Conference Speakers *(continued)*

Sarah Mahoney, PhD
Arrakis
Pharma Day
Industry Updates Part 2

Gina Many, PhD
Pacific Northwest National Laboratory
Wellness Room: Chair & Stand-Up Zumba
Movement Moment

Antoine Mangin, PhD
Cardiff University, UK Dementia Research Institute
Innovations & Prospects for Gene Editing & Tuning

Haley Martinelli, Esq.
DM2 Community Member, MDF Board Member, MDF Volunteer Support Group Facilitator
DM2 Community Panel: Stories of Hope & Helpful Tips

Carolyn Martinez, M.A., CCC-SLP
Houston Methodist
Nourishing Solutions: Managing Dietary Needs & Swallowing with DM
Snack Smart: Making Easy, Healthy, Dysphasia Friendly, & Delicious Bites

Tom McPeek
DM2 Community Member, MDF Board Member, MDF Volunteer Support Group Facilitator
First Timers’ Tea

Alison McVie-Wylie, PhD
Vertex Pharmaceuticals
Pharma Day

Anna Miller, MS, RD, LD
Houston Methodist
Nourishing Solutions: Managing Dietary Needs & Swallowing with DM
Snack Smart: Making Easy, Healthy, Dysphasia Friendly, & Delicious Bites

Darren Monckton, PhD
Professor, University of Glasgow, MDF Medical and Scientific Advisory Committee
Genomics Unleashed: Insights & Implications

Martha Montag Brown
MDF Board Member
First Timers’ Tea

Shaun Moore
MDF Volunteer Support Group Facilitator, DM1 Community Member
DM1 Community Panel: Sharing Hope & Life Hacks

Christian Mueller, PhD
Sanofi
Pharma Day

Asif Paker, MD, MPH
Vertex Pharmaceuticals
Industry Updates Part 2

Katelyn Payne, RN, CGC
University of Indiana Health Neurology
Genetics Unveiled: Testing, Understanding Results, & Family Planning with DM1

Belinda Pinto, PhD
University of Florida
Highlighting New Research: Excessive Daytime Sleepiness

Allan Posner
Unaffected Caregiver
Unaffected Male Caregivers: Prioritizing Self Care & Wellbeing

Araya Puwanant, MD, MS
Wake Forest University School of Medicine
Mind Matters: Navigating Cognitive & Memory Challenges in DM2

Andy Rohrwasser, PhD, MBA
MDF Chief Scientific Officer
Pharma Day Welcome & Introduction
Welcome & State of the Foundation
Industry Updates Introduction
Career Paths: AltAca Insights

Andreas Roos, PhD
University of Ottawa
Tech Integration: Omics & Beyond in Biomarker Research

Didier Rouy, MD, PhD
Sanofi
Industry Updates Part 2

Seward B. Rutkove, MD
Harvard Medical School
Your Guide to Clinical Trials: From Phases to Participation

Ted Salwin
MDF Volunteer Support Group Facilitator, Unaffected Caregiver
Unaffected Male Caregivers: Prioritizing Self Care & Wellbeing

Jacinda Sampson, MD, PhD
Stanford Neuroscience Health Center, MDF Medical and Scientific Advisory Committee
DM1: Stump the Doctor
Fireside Chat: You’ve Just Been Diagnosed, What’s Next?

Elaine Samson
DM2 Community Member
DM2 Community Panel: Stories of Hope & Helpful Tips

Wendy E. Schell, PT, DPT, MS, CSRS
Houston Methodist
Living Better with DM: Tools & Services of Occupational Therapy & Home Health

Karly Sciortino-Poulter
The Arc Indiana
Empowering Adult Children: Managing Care, Service Delivery, & Living with CDM

Ellen Shapiro, C-IAYT
Yoga Instructor, MDF Community Member
Movement Moment

Jenny Shoskes, PharmD
PepGen
Industry Updates Part 2

Megan Stevenson, MS
The Arc Indiana
Lifeworks: Landing the Job: Building Resumes & Acing Interviews

Tanya Stevenson, EdD, MPH
MDF Chief Executive Officer
Pharma Day Welcome & Introduction
Welcome & State of the Foundation

Maurice S. Swanson, Ph.D.
University of Florida
Charting the Past: Historical Perspectives on Therapeutics & Opportunities for Foundations
There’s Hope: A Discussion About the Future of DM2 Research

Andrea Swenson, MD
University of Iowa Myotonic Dystrophy Clinic
Understanding DM1: From Genetics to Care & Everything in Between

Lukasz Sznajder, PhD
University of Nevada, Las Vegas
Career Paths: Academia Insights

Andrea Thacker Delcore
DM1 Community Member
DM1 Community Panel: Sharing Hope & Life Hacks

Brad Thompson, LPC-S
The HALI Project
Building Resilience: Family Dynamics & Support in the DM Journey

Stacey B. Thacker, MA, LMFT
Unaffected Caregiver
Unaffected Caregivers: Love in Transition: Balancing Romantic Connections & Caregiving in DM

Loralei L. Thornburg, MD
University of Rochester Medical Center
Fireside Chat: Affected Women’s Real Talk: Managing Intimacy & Sexual Health Challenges with DM

Charles Thornton, MD
University of Rochester School of Medicine, MDF Board Member, MDF Medical and Scientific Advisory Committee
Classical DM1 with Large CTG Expansion at the Mouse Locus

Ryan Vogels
MDF Volunteer Support Group Facilitator, DM2 Community Member
DM2 Community Panel: Stories of Hope & Helpful Tips

Eric Wang, PhD
University of Florida, MDF Medical and Scientific Advisory Committee, MDF National Advocacy Committee
Young Investigators Moderator
CNS Symptoms and Meaningful Endpoints

Judy Walker, MD, FRCP(C)
ARTHEx Biotech
Pharma Day
Industry Updates Part 1

Alla Zozulya Weidenfeller, PhD
Lupin Neurosciences
Pharma Day
Industry Updates Part 1

Angie Wiggans
Unaffected Caregiver
Unaffected Female Caregivers: Practical Tips for Self Care

Hanadie Yousef, PhD
Juvena Therapeutics
Pharma Day
Industry Updates Part 1

Continue Learning With MDF

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

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

PARTICIPATE IN THIS YEAR'S UPCOMING ASK-THE-EXPERT WEBINARS:

- **June:** *The Emotional Journey of DM: Coping with Progression & Uncertainty*
  Melissa Dixon, PhD, University of Utah
- **July:** *Seeing DM Clearly: Understanding & Managing Vision Challenges*
  Vinod Mootha, MD, UT Southwestern
- **August:** *Genetics of DM: Understanding Inheritance, Expansions, & Research*
  Andy Rorhwasser, PhD
- **September:** *Pediatric Endocrinology & DM: Growth, Hormones, & Metabolic Health*
  Nadia Merchant, MD, Children's Health Dallas
- **October & November webinars TBD!**

Myotonic Dystrophy Family Registry
The Myotonic Dystrophy Family Registry (MDFR) is a web-based database of information provided by families to aid researchers in developing treatments for DM and identifying participants for research studies and clinical trials. The MDFR enables MDF and others to review community-reported statistics and data about their experience of living with DM to aid advocacy and education efforts. Learn more and register here at: **www.myotonicregistry.org/**

Meet the DM Drug Developer
From 2021 through 2025, MDF invited biotechnology and pharmaceutical partners that are working on treatments and cures for myotonic dystrophy to sit down with our community, share their progress, and answer community questions. View the complete series here at:
www.myotonic.org/meet-dm-drug-developers

Find-a-Doctor Map
Finding medical professionals who understand myotonic dystrophy is one of the most important things you can do to help manage the unique symptoms of DM. With your help, MDF has compiled a database of community recommended medical professionals. Use the Find-a-Doctor Map to help you find medical professional in your area at: **www.myotonic.org/find-a-doctor-map**

Need additional support? Call our Warmline at 415.800.7777

Participate in DM Research at the Conference

Indiana University School of Medicine
Chancellor

Indiana University School of Medicine will be conducting a pilot study to better understand speech and swallowing challenges in individuals with DM1.

Stanford University School of Medicine
Capitol Overlook East

Stanford researchers invite you to participate in a study aimed at collecting movement data to develop video digital biomoarkers for DM1 and DM2 (located in Capital Overlook East) as well as an electroencephalogram (EEG) study for DM1 (located in Congress 2).

University of Florida
Senate 2

Researchers at the University of Florida will be studying possible causes of sleep disruption in individuals with DM, with a special focus on people affected by DM2.

University of Rochester
Senate 3

REACH DM at the MDF conference: children and adults with myotonic dystrophy type 1 are invited to participate in a short study visit at the MDF conference. What is involved: a blood draw for research genetic testing (we return the results via mail), surveys and functional assessments.

Wake Forest University School of Medicine
Cameral

Wake Forest University is conducting research to better understand cognitive changes in individuals with adult-onset DM1. Findings will enable comparisons with our ongoing DM2 brain study, offering insights into brain function differences and guiding the development of future studies in both conditions.

Exhibitor & Sponsor Showcase

Visit Our Amazing Exhibitors During the Conference!

Tables are set up throughout Grand Foyers 1-4.

AMO Pharma
ARTHEx Biotech
Avidity Biosciences
Myotonic Dystrophy Clinical Research Network (DMCRN)
DM Artisans Table
Dyne Therapeutics
Global Alliance for Myotonic Dystrophy Awareness
Indiana Canine Assistant Network (ICAN)
MDF Advocacy Table
MDF Family Registry
MDF Support Groups
MDF Resources
PepGen
Sanofi
Stanford University
University of Rochester
The Arc of Indiana
University of Florida
University of Utah
Vertex Pharmaceuticals

Resources for Individuals Living with DM and their Families

These publications are for a general audience to help understand the condition and various aspects of life with DM. You can access them and others 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

This resource equips individuals living with DM and their families with crucial information to safely navigate the anesthesia process.

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.

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.

Exercise Guide for Individuals with Myotonic Dystrophy* and Exercise Infographic

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

Mental Health Handbook*

Although DM has the potential to impact cognitive health and development, this resource emphasizes the social and emotional impact of the disease. It gives an overview of mental health issues that may affect people living with DM and potential resources.

Myotonic Dystrophy and 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.

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.

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 andtheir 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. It 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 Job System only)

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 and others 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.*

Clinical Care Recommendations and Quick Reference Guides*

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.

Practical Suggestions for the Anesthetic Management of a Myotonic Dystrophy Patient* and Quick Reference Guide

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.

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 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.

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

Order the MDF DM Essentials for Patients Box for your clinic today!

The box is free—just cover shipping and handling.

MDF Grant Recipients

We are pleased to introduce our current MDF Grantees. MDF is excited to fund exceptional researchers dedicated to advancing research in the field of myotonic dystrophy.

To learn more about MDF Funding opportunities go to: **www.myotonic.org/grants**.

Be sure to visit our researchers' posters at the conference during the Poster Showcase to talk with them live about their research!

View all the research posters online at: **https://www.myotonic.org/poster-abstracts-2025**.

2024 and 2025 Pilot Grants

Joel R. Chamberlain, PhD
Research Associate Professor
University of Washington

Paloma Gonzalez Perez, MD, PhD
Neuromuscular Attending
Massachusetts General Hospital

Emma Matthews, FRCP
Reader of Neurology and Consultant
Neurologist St. George's
University of London

Belinda Pinto, PhD
Assistant Scientist
University of Florida

Stephanie Tome, PhD
Research Associate
Sorbonne Université-Inserm

Arianna Tucci, MD, PhD
Medical Research Council (MRC)
Clinician Scientist, Queen Mary
University of London

2024 and 2025 Research Fellows

Haneui Bae, PhD
University of Illinois
Urbana-Champaign

Betty Bekele
Emory University

Louison Daussy
Institut National de la Santé et
de la Recherche Médical
- DR Paris Centre Est

Emily Davey
University of Florida

Sakura Hamazaki
University Rochester

Cécilia Légaré, PhD
SUNY University
at Albany

Diana Madrid Fuentes
Wake Forest University
Health Sciences

Alexandra L.
Marrero Quinones
Virginia Commonwealth
University

Cameron Niazi
University of Florida

Keep in touch! 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 community members. It is the best way to stay current on news in the DM community.

Sign up to join our mailing list at: **www.myotonic.org/sign-emails-0**

MDF Grant Recipients *(continued)*

2024 and 2025 Pilot Grants

Johanna Hamel, MD
Assistant Professor
University of Rochester

Tahereh Kamali, PhD
Postdoctoral Research Fellow
Stanford University

Lukasz Jakub Sznajder, PhD
Assistant Professor
University of Nevada, Las Vegas

Scott Uhlrich, PhD
Assistant Professor
The University of Utah

2024 and 2025 High Priority Grants

Samuel Carrell, MD, PhD,
Assistant Professor
Virginia Commonwealth University

Kristina Kelly, PT, DPT, MS, EdM, NCS, CPT, PES
Assistant Research Professor
University of Missouri

Lukasz Jakub Sznajder, PhD
Assistant Professor
University of Nevada, Las Vegas

Katarzyna Taylor, PhD
Research Assistant Professor
Adam Mickiewicz University

Become a DM Advocate & Increase DM Research Funding & Awareness

Your Voice Matters!

INTERNATIONAL
Myotonic
Dystrophy
Awareness Day
SEPTEMBER 15

By raising awareness, we influence research funding budgets, the development of new therapies, clinical trial efforts, and initiatives to improve the quality and cost of care for all people living with DM. You can help change the future of DM today: www.myotonic.org/advocate

---

Learn About Educating Federal and State Elected Officials About Need for DM Research

- How to Talk with Your Representatives About DM & Research Priorities
- Share Your Story with Maximum Impact
- Celebrate Our Successes in Building Relationships and Support

---

Celebrate International Myotonic Dystrophy Awareness Day on September 15th!

- Share the DM-At-A-Glance poster at school or work.
- Ask neighbors and shops in your community to display an International DM Awareness window sign.
- Share MDF Toolkits & Resources with your healthcare providers.
- For tips, suggestions, and resources, visit www.myotonic.org/international-dm-day.

2025 MDF Support Groups

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

Find your support community below. Learn more about our SGFs at: **www.myotonic.org/sgfs**.

Volunteer Support Group Facilitators

Alexandra LeBoeuf
Canada
Virtual Support Group

Ann Woodbury
JOA Warriors Support Group, JOA Caregivers Support Group

Annette Rnjak
Caregiver Support Group

Araceli Mera
International Spanish Speakers Support Group

Barbara Ochoa
California Regional Support Group

Bernhard Rogg
International German Speakers Support Group

Beth Feigenblatt
Florida Regional Support Group

Bill Nuttall
New England Regional Support Group, DM1 Facebook Group Moderator, Adult Facebook Chat Moderator

Caroline Easterling
Maryland Regional Support Group

Carolyn Valek
Ohio Regional Support Group, JOA Warriors Support Group

Chuck Hunt
South East Regional Support Group

Cindy Hubert
Washington Regional Support Group

David Kugler
International Spanish Speakers Support Group

Emily Jones
Finger Lakes and Upstate New York Regional Support Group

Erin Beucler
DM1Facebook Group Moderator

Guillermo Zubillaga
New York City/New Jersey Regional Support Group

Haley Martinelli
DM2 Support Group, Affected Women's Support Group

Jan Jaffe
New York City/ New Jersey Regional Support Group

Jeannine DeSoi
New England Regional Support Group, Affected Women's Support Group

Jim Dolan
Affected Men's Support Group

Volunteer Support Group Facilitators *(continued)*

Jodie Howell
Virginia Regional Support Group

John Cooley
Kansas City Regional Support Group

Jonathan Freedman
Washington Regional Support Group

Julian Easterling
Maryland Regional Support Group

Julie LeBoeuf
Canada Virtual Support Group, International French Speakers Support Group

Kathie Thorsland
Mountain West Regional Support Group

Kelsey Freedman
DM2 Facebook Group Moderator

Kim McPeek
DM2 Caregivers Support Group

Kim Reynolds
DM2 Facebook Group Moderator

Kristen McClintock
Florida Regional Support Group,
DM Virtual Happy Hour

Kyle Dunson
JOA Caregivers Support Group

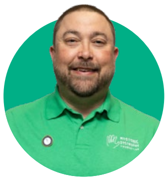
Lois Schenk
Finger Lakes and Upstate New York Regional Support Group

Lynn S
Texas Regional Support Group

Marie-Claude Sauvè
Canada Virtual Support Group, International French Speakers Support Group

Mark Coplin
Portland Regional Support Group

Mindy Kim
South East Regional Support Group, DM Virtual Happy Hour



Nathan Beucler
Ohio Regional Support Group, Caregiver Facebook Group Moderator

Patirica Gibson
Kansas City Regional Support Group

Peggy Melton
Texas Regional Support Group, JOA Caregiver Support Group

Rob Besecker
Chicago Regional Support Group

Rose Albanese
California Regional Support Group

Ryan Vogels
Chicago Regional Support Group, Affected Men's Support Group

Samantha Welsh
Virginia Regional Support Group

Sarah Berman
Caregivers for Children Living with Congenital Myotonic Dystrophy Support Group, International French Speakers Support Group

Shaun Moore
Affected Men's Support Group

Suzanne Perkins
Michigan Regional Support Group

Ted Salwin
Caregiver Support Group

Tom McPeek
DM2 Support Group & Facebook Moderator

Contribute to Community, Care, and a Cure!

In 2024, 84% of MDF's budget was dedicated to DM Research, Care, and Advocacy programs. With your support, we create and share critical resources and information, fuel groundbreaking science, raise awareness, and advocate for the DM community with legislators and federal agencies.

DONATE BY MAIL: Myotonic Dystrophy Foundation
663 Thirteenth St., Suite 100 Oakland, CA 94612

DONATE ONLINE: www.myotonic.org/donate

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

2025 MDF Medical and Scientific Advisory Committee

The MDF Medical and Scientific Advisory Committee is comprised of individuals who collectively have devoted more than one hundred years to studying muscle diseases, specifically myotonic dystrophy. Read more here at: **https://www.myotonic.org/meet-team/Medical-and-Scientific-Advisory-Committee**.

Andy Berglund, PhD
Committee Chair
The RNA Institute
University at Albany

Guillaume Bassez, MD, PhD
Institut de Myologie

Kathie Bishop, PhD
Acadia Pharmaceuticals

Tom Cooper, MD
Baylor College of Medicine

John W. Day, MD, PhD
Stanford University

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

Cynthia Gagnon, PhD
Sherbrooke University

Darren Monckton, PhD
University of Glasgow

Laura Ranum, PhD
University of Florida

Jacinda Sampson, MD, PhD
Stanford University

Charles Thornton, MD
University of Rochester

Eric Wang, PhD
University of Florida

2025 MDF Board of Directors

The Myotonic Dystrophy Foundation's Board of Directors 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 Medical and Scientific Advisory Committee. To learn more about MDF Board of Directors go to **www.myotonic.org/board-directors**.

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Board Chair &
Lifetime Trustee

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Berglund, PhD

Martha Montag
Brown
Vice-Chair

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Campagna, MD

Elizabeth
Florence, Esq.
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Haley
Martinelli, Esq.

Thomas "Tom"
McPeek

Charles
Thornton, MD

2025 MDF Staff & Consultants

To learn more about MDF staff go to **www.myotonic.org/staff**.

Tanya Stevenson,
EdD, MPH
Chief Executive Officer

Andy Rohrwasser,
PhD, MBA
Chief Scientific Officer

Mindy Buchanan
Director of Programs

Scarlett Chidgey
Director of Development

Kleed Cumming
Director of
Communications
& Technology

Nadine Ann Skinner, PhD, MPA
Director of Evaluation
and Research Programs

Emily Romney, MPA
Senior Manager of
Community Education

Lucie Shiffman
Senior Manager of
Community Engagement

Sofia Olmos, PhD
Myotonic Dystrophy
Family Registry
Coordinator

Genevieve Wiegleb
Communications
Associate

Interns & Consultants

Juan Arboleda
Public Health Resource
Development Intern

Kevin Brennan
Advocacy
Consultant

Lydia Dunn
Clinical Education
Support Intern

Mindy Kim
Registry Outreach
Specialist

Danny Kuei
Family Registry
Data Intern

Annalise Kalmanoff
2024 MDIM Intern,
2025 Conference
Consultant

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PHARMA

BRONZE

LUPIN
NEUROSCIENCES

Global Alliance for DM Awareness

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

ASMNM
MUSCULAR DYSTROPHY AUSTRALIA
ABMM Téléthon.be
NMD4C
MUSCULAR DYSTROPHY CANADA / DYSTROPHIE MUSCULAIRE CANADA
MUSKELSVINDFONDEN plads til forskelle!

EURO-DYMA
AFMTÉLÉTHON INNOVER POUR GUÉRIR
DGM Deutsche Gesellschaft für Muskelkranke e.V.
MDA ΕΛΛΑΣ
CENTRO CLINICO Nemo
fm fm

DM family
Maastricht UMC+
MD NEDERLAND
SPIERZIEKTEN NEDERLAND
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VNIVERSITAT DE VALÈNCIA Translational Genomics Group
ASRIMM
LUPIN NEUROSCIENCES

AMO PHARMA
CDM Cure DM Research ♥ Support ♥ Awareness
MD Myotonic Dystrophy SUPPORT GROUP
Muscular Dystrophy UK Fighting muscle-wasting conditions
St George's University Hospitals NHS Foundation Trust NHS Outstanding care every time
TREAT-NMD Neuromuscular Network

UK Myotonic Dystrophy Patient Registry
Newcastle University
John Walton Muscular Dystrophy Research Centre
University College London Hospitals NHS Foundation Trust NHS
AskBio
astellas GENE THERAPIES
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AVIDITY BIOSCIENCES

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EXPANSION THERAPEUTICS
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HOUSTON Methodist LEADING MEDICINE

Juvena THERAPEUTICS Decoding Secreted Proteins
K-STATE
SCHOOL OF MEDICINE DEPARTMENT OF NEUROLOGY
The Jackson Laboratory Leading the search for tomorrow's cures
MDA Muscular Dystrophy Association
Myotonic Dystrophy FOUNDATION

Myotonic Dystrophy Family Registry
Connecting people with researchers
Paul D. Wellstone Muscular Dystrophy Research Centers
PepGen
Stanford MEDICINE
UF Center for NeuroGenetics College of Medicine UNIVERSITY of FLORIDA

NeuroGenetics Student Organization at the University of Florida
THE RNA INSTITUTE COLLEGE OF ARTS & SCIENCES UNIVERSITY AT ALBANY
UNIVERSITY of I HEALTH CARE
U HEALTH UNIVERSITY OF UTAH
VERTEX
VCU School of Medicine
Wake Forest School of Medicine

Myotonic Dystrophy
IN MOTION
www.myotonic.org

Movement Matters

Join MDF's second annual Myotonic Dystrophy In Motion Awareness Month this July!

- Movement Classes Each Monday in July!
- Educational Webinars
- Community Happy Hours
- Movement Competitions

Register Early—our first 50 registrants who sign up by June 1st will get an MDF-branded Exercise Band!

Only available to US participants.

www.myotonic.org/In-Motion-Month

Myotonic Dystrophy
FOUNDATION

Your Voice Matters!

Advocate for the DM Community

By raising awareness, we influence research funding, therapy development, clinical trials, and care for everyone living with DM.

Help change the future of myotonic dystrophy today:

- Educate Policymakers – Learn how to engage officials to protect and expand DM research funding.
- Advocate with Confidence – Get tools to talk with representatives about DM research and healthcare needs.
- Share your Story – Personal stories drive change—learn how to make yours impactful.
- Strengthen DM Support – Celebrate advocacy wins and build relationships with key decision-makers.

SCAN ME!

www.myotonic.org/Advocate

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paf
Patient Advocate Foundation

Solving insurance & healthcare access issues since 1996

The Patient Advocate Foundation is a national 501(c)(3) nonprofit that offers case management services and financial assistance to individuals with chronic, life-threatening, or debilitating illnesses. For more information or to connect with someone who can help, visit patientadvocate.org or call 800-532-5274.

MYOTONIC DYSTROPHY CLINICAL RESEARCH NETWORK

DMCRN
MYOTONIC DYSTROPHY CLINICAL RESEARCH NETWORK

CONTACT:

RUBY LANGESLAY,
NETWORK COORDINATOR
RUBY.LANGESLAY@VCUHEALTH.ORG

●

JENNIFER RAYMOND,
NETWORK COORDINATOR
JENNIFER.RAYMOND@VCUHEALTH.ORG

Active DMCRN Natural History Studies

Establishing Biomarkers and Clinical Endpoints in Myotonic Dystrophy Type 1 (END-DM1)

This is an observational study following DM1 patients at 3-4 visits over 24 months.

Inclusion Criteria:

- 18-70 years old at time of enrollment
- Clinical diagnosis of DM1 or positive genetic test

Exclusion Criteria:

- Symptomatic renal or liver disease, uncontrolled diabetes or thyroid disorder, or active malignancy other than skin cancer
- Current alcohol or substance abuse
- Concurrent enrollment in clinical trial from DM1 or participation in the past 6 months of enrollment
- Pregnancy at any time point of participation
- Concurrent medical condition that would compromise participation or safety

*Non-ambulatory participants are not excluded, but limited to 15% of total enrollment

Assessing Pediatric Endpoints in DM1 (ASPIRE-DM1)

This is an observational study following CDM/ChDM patients at 4 visits over 18 months.

Inclusion Criteria:

- Neonate to 4 yrs, 11 mos at time of enrollment
- Diagnosis of CDM or ChDM, both symptomatic and genetically confirmed

Exclusion Criteria:

- Any other illness that would compromise participation or safety
- Significant trauma within 1 month of enrollment
- Internal metal or devices
- History of bleeding disorder or platelet count <50,000
- History of reaction to local anesthetic

DMCRN Sites

Contact Jennifer or Ruby for which sites are actively recruiting for END-DM1 and ASPIRE-DM1 studies.

NORTH AMERICA

HOUSTON METHODIST NEUROLOGICAL INSTITUTE

THE OHIO STATE UNIVERSITY

STANFORD UNIVERSITY

UNIVERSITE DE SHERBROOKE – QUEBEC

UNIVERSITY OF CALIFORNIA, LOS ANGELES

UNIVERSITY OF CALIFORNIA, SAN DIEGO

UNIVERSITY OF COLORADO, DENVER

UNIVERSITY OF FLORIDA

UNIVERSITY OF IOWA

UNIVERSITY OF KANSAS

UNIVERSITY OF ROCHESTER

UNIVERSITY OF TEXAS, SAN ANTONIO

VIRGINIA COMMONWEALTH UNIVERSITY

EUROPE

CENTRO CLINICO NEMO

FRIEDRICH-BAUR INSTITUT - LMU MUNICH

RADBOUD UNIVERSITY – NL

ST. GEORGE'S, UNIVERSITY OF LONDON

UNIVERSITY COLLEGE LONDON

ASIA-PACIFIC

AOMORI NATIONAL HOSPITAL

UNIVERSITY OF AUCKLAND

Our Motivation is YU

Loraine
DM1 Advocate and her family

We are deeply grateful for the invaluable contributions of **MDF and all** who have contributed to **MARINA**®, **MARINA-OLE**™**, and now the HARBOR**™ **study**. Together we are shaping the future of DM1 treatment.

We are proud to support the important work of the Myotonic Dystrophy Foundation as we continue to advance meaningful therapies for the myotonic dystrophy community.

PepGen™

Committed to Developing a Transformative Therapy for the Treatment of Mytonic Dystrophy Type 1 (DM1)

Driven by our proprietary Enhanced Delivery Oligonucleotide (EDO) platform, PepGen is creating a pipeline of potentially disease-modifying investigational therapeutics with the potential to safely and effectively target the underlying cause of serious genetic neuromuscular and neurological disorders.
Our mission is to deliver transformative therapies to improve the lives of people living with neuromuscular diseases and their families.

PepGen's lead compound in DM1 (PGN-EDODM1) is designed to restore normal function

PGN-EDODM1 is designed to target the repeat sequence mutation that is the root cause of DM1 to restore normal processing of proteins. Clinical trials are ongoing: FREEDOM-DM1 (clinicaltrials.gov) and FREEDOM2-DM1 (clinicaltrials.gov)

Contact

Jane Larkindale, Ph.D.—Vice President of Clinical Science
Alayna Tress, MPH—Director, Patient Advocacy

Email: **community@pepgen.com**

Jubal and his family, living with DM1

Visit our website to learn about our approach, our pipeline, our work with the DM community and upcoming clinical trials

---

VERTEX®
THE SCIENCE *of* POSSIBILITY

Vertex creates new possibilities in medicine so people with serious diseases can live better lives.

We work with leading researchers, doctors, public health experts and other collaborators who share our vision for transforming the lives of people with serious diseases, their families and society.

Vertex is a proud supporter of the 2025 Myotonic Dystrophy Foundation Conference.

www.vrtx.com

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.

Interested in learning more?

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

Sanofi - BrAAVe Study - Recruitment Flyer - 14-Mar-2025 - English (Principal) - V1.1
[BrAAVe-RF-EnP]

sanofi

AMO
PHARMA

New medicines. Better lives.

NOVARTIS

Myotonic Dystrophy FOUNDATION

Community Connections

Send a Letter, Share a Smile!

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

- **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

2025 Gala

Reaching New Heights

Thursday, Sept. 18
Tribeca Rooftop, New York

Become a Support Group Facilitator!

MDF is seeking co-facilitators to help lead support groups and connect our community with vital resources.

Current MDF Support Group openings:

- Caregivers of Children with CDM
- DM2 Caregivers
- Regional Groups: Michigan; Mountain West; New York City / New Jersey; Portland, Oregon

www.myotonic.org/SGF-App

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

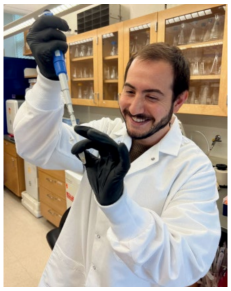
Early Career Grant - $190k
- Basic / Translational Science
- Clinical Research

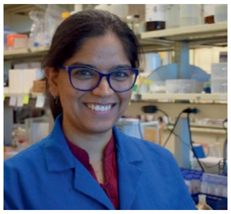
Small Grants - $2.5k | $5k
- Conference travel
- Open-access fees

APPLY NOW!

www.myotonic.org/Grants

SCAN ME!





SCAN ME!

DM-INSIGHT

Share Your Story. Shape the Future of Myotonic Dystrophy Care.

Scan the QR Code Above to Begin the Survey!

MDF is conducting a survey in the USA and Canada to better understand the experiences of people affected by myotonic dystrophy, their caregivers, and healthcare providers to enhance support, resources, and access to new treatments for the DM community.

Your input will help:

- Improve diagnosis and treatment
- Expand access to care and support
- Inform research, advocacy, and policy

Who can participate?

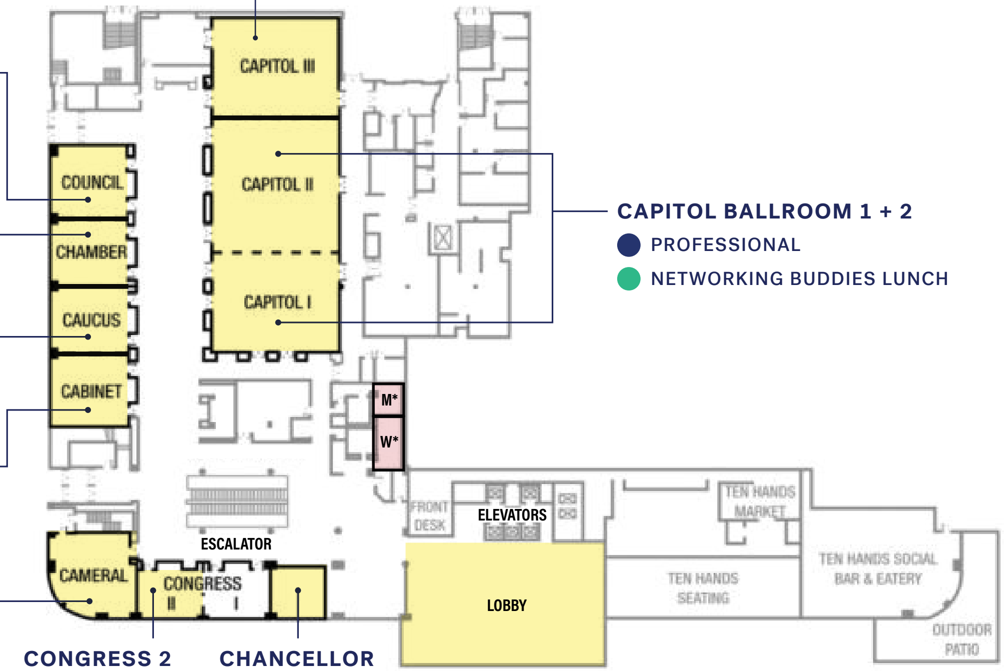
- *Only adults in the USA and Canada*
- People living with DM1 or DM2
- Caregivers
- Healthcare providers

The landscape of DM care is changing. Your voice matters to help shape better care and access for the DM community!

www.myotonic.org/insight

MDF Conference Area Map | First Floor

FIRST FLOOR



CAPITOL BALLROOM 3
- ● POSTER SHOWCASE ROOM

COUNCIL
- ● DM1
- ● DM2
- ● CAREGIVERS

CHAMBER
- ● CDM

CAUCUS
- ● LIFE WORKS
- ● COMMUNITY

CABINET
- ● COMMUNITY

CAMERAL

WAKE FORREST RESEARCH

CAPITOL BALLROOM 1 + 2
- ● PROFESSIONAL
- ● NETWORKING BUDDIES LUNCH

CONGRESS 2

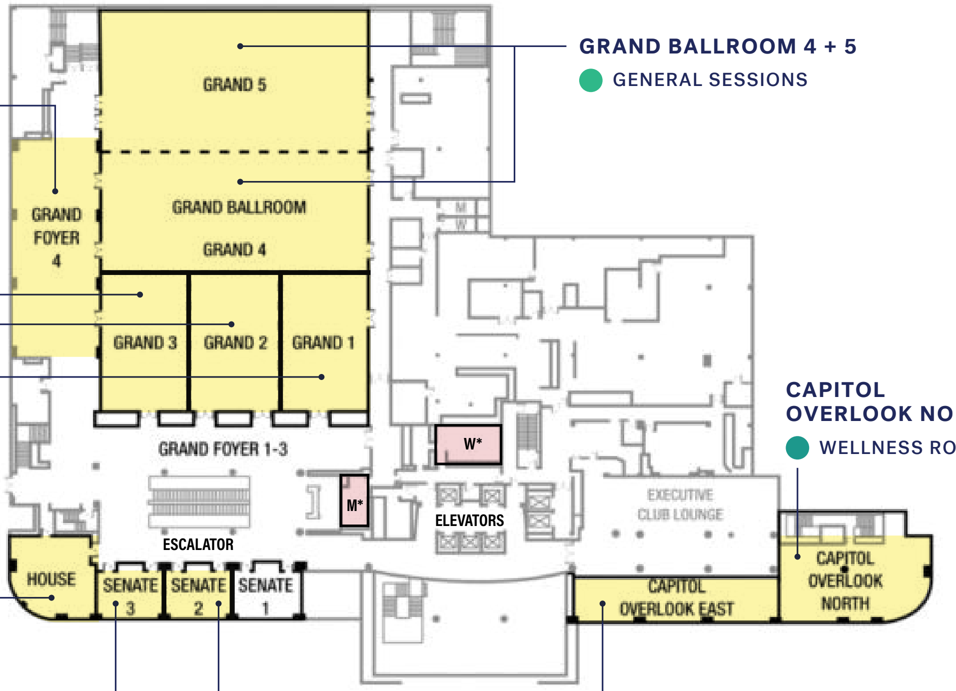
STANFORD EEG RESEARCH

CHANCELLOR

UNIVERSITY OF INDIANA RESEARCH

MDF Conference Area Map | Second Floor

SECOND FLOOR



GRAND FOYER 4
● EXHIBITOR HALL

GRAND BALLROOM 3
● CAREGIVERS

GRAND BALLROOM 2
● DM2

GRAND BALLROOM 1
● DM1

HOUSE
● FIRESIDE CHATS

GRAND BALLROOM 4 + 5
● GENERAL SESSIONS

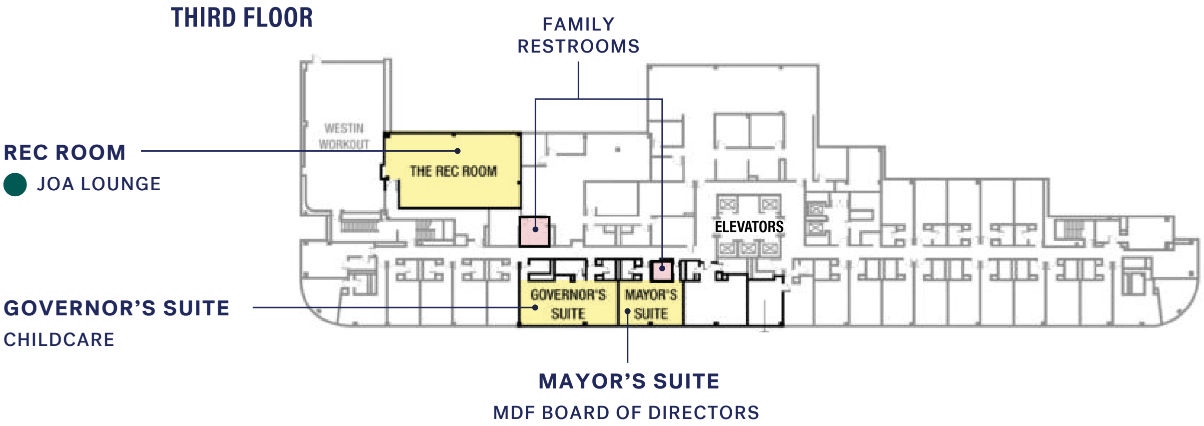
CAPITOL OVERLOOK NORTH
● WELLNESS ROOM

SENATE 3
UNIVERSITY OF ROCHESTER RESEARCH

SENATE 2
UNIVERSITY OF FLORIDA RESEARCH

CAPITOL OVERLOOK EAST
STANFORD MOVEMENT RESEARCH

MDF Conference Area Map | Third Floor



THIRD FLOOR

FAMILY RESTROOMS

REC ROOM

● JOA LOUNGE

GOVERNOR'S SUITE

CHILDCARE

MAYOR'S SUITE

MDF BOARD OF DIRECTORS

Myotonic
Dystrophy
FOUNDATION

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