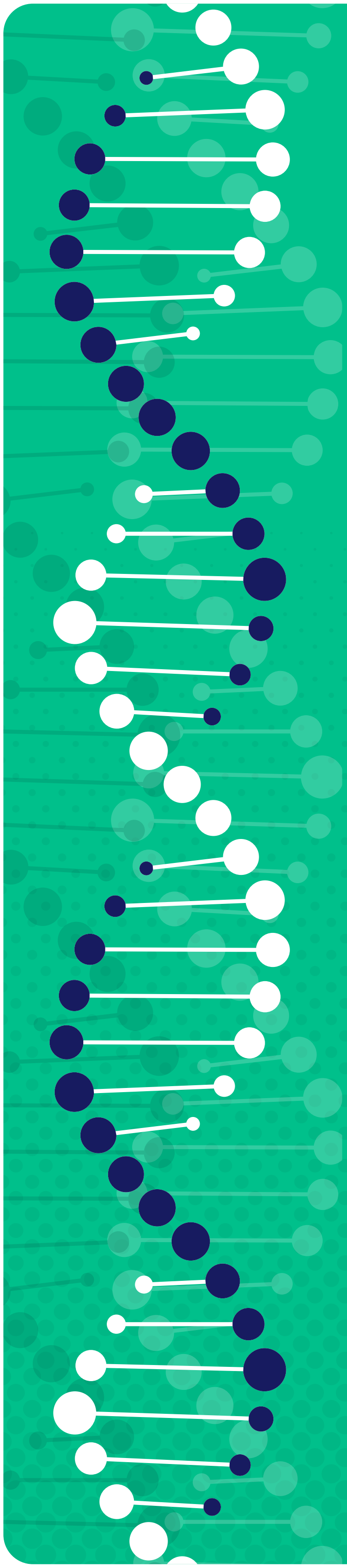
Myotonic
Dystrophy
FOUNDATION

2023 MDF Annual Conference

Renaissance Washington DC Downtown Hotel
September 7th, 8th & 9th

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

ACCESS THE VIRTUAL CONFERENCE



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

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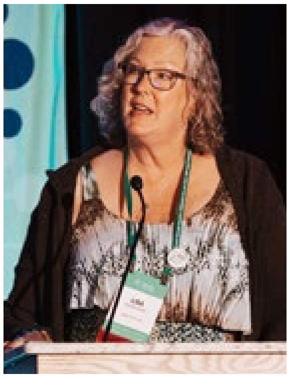
Myotonic Dystrophy FOUNDATION

2023 MDF Annual Conference

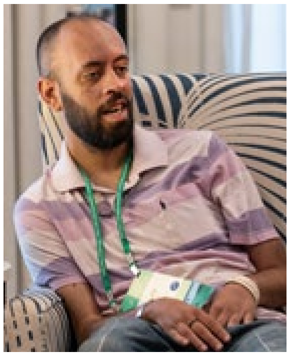
SEPTEMBER 7TH, 8TH & 9TH

Breakout Sessions

MDF Team:



Welcome Letter from the CEO



Welcome to the 2023 MDF Annual Conference!

On behalf of the Myotonic Dystrophy Foundation's (MDF) Board, staff, and countless volunteers who have helped make this year's conference possible, I am thrilled to welcome you to our second hybrid conference! For the 15th year in a row, MDF is delighted to bring together our powerful community of DM families, researchers, clinicians, industry partners, regulators, members of the Global Alliance for Myotonic Dystrophy Awareness, and many other allies. A warm embrace to the over 120 people attending the conference for the very first time!

Thanks to thoughtful input from hundreds of community members, we have organized an agenda to help meet the diverse needs and interests of our community, including over 45 sessions geared toward a variety of audiences: **DM1, DM2, CDM, Caregivers, JOA, Community, and DM Professionals.** Everyone is welcome at the General Sessions to hear updates from the Foundation, an overview from the FDA, advancements in research, inspirational stories, highlights of treatment advances, special awards, and more. **Community Breakout Sessions** are also open to everyone and will cover a variety of in-demand topics. Back by popular demand, **First Timers' Tea** and **Stump-the-Doctor** sessions return this year, four different community panels are offered, Industry Updates will highlight the latest news from nine companies making incredible progress in DM drug development, and, in line with MDF's Myotonic Dystrophy In Motion initiative, **Movement Moments** will provide short movement activities for all conference attendees.

I hope you take advantage of the phenomenal array of activities designed to rejuvenate both mind and body in the new **Wellness Room** this year. We have also designed exciting new ways to get to know one another: the **Conference Networking Buddies** lunch, which pairs DM Professionals with people living with myotonic dystrophy and their families! Please don't miss dinner on Friday night where you are sure to be wowed by **mentalist and magician, David Gerard!**

I invite you to explore the exhibitor booths, as well as over **50 DM-focused research posters** displayed around the ballroom - and talk with their authors during Saturday afternoon's Exhibitor & Poster Showcase. You might even encounter one of **MDF's Research Fellows!**

We are excited that ALL attendees have an online conference profile this year and may access the virtual conference platform to attend sessions online at any time. Login to **https://app.swapcard.com/** or use the SwapCard app from the comfort of your own home or your hotel room to attend sessions remotely!

Please reach out to the MDF team if you need anything and be sure to stop and introduce yourself! I look forward to meeting you all and hope you truly enjoy the next few days.

All my best,

Tanya Stevenson

Tanya Stevenson, EdD, MPH
Chief Executive Officer

Advocacy Day | Thursday, September 7th

Advocacy Day begins with check-in and a Community Breakfast & Advocate Training, sponsored by Avidity Biosciences, where we will review everything you need to know to have a successful congressional meeting. Following our breakfast training, attendees will board a shuttle bus to Capitol Hill. (MDF shuttle busses will be running between Capitol Hill and the Hotel throughout the day.) Once on Capitol Hill, groups of attendees organized by state and congressional districts will meet with their elected officials to raise awareness and request support.

Pre-registration is required to attend Advocacy Day

Attire: While business attire is not required, we ask that advocates not wear shorts or t-shirts during your visits. Long pants, skirts, and collared shirts are recommended. Please wear comfortable shoes as you may have to walk long distances from the bus stop to your meetings. Attendees with mobility challenges will want to allow extra time for travel and taking breaks.

Detailed information including room maps, bus schedule, and congressional meeting leave-behind materials will be provided inside the MDF Advocacy Day Packets given out during the Breakfast & Advocate Training.

Congratulations to Our 2023 Congressional Advocacy Award Recipients!

SENATOR TIM KAINE
(D) Virginia

SENATOR CYNTHIA LUMMIS
(R) Wyoming

We gratefully honor Senators Kaine and Lummis for their exceptional efforts in raising awareness for myotonic dystrophy, providing hope, and supporting our community.

AVIDITY BIOSCIENCES

Advocacy Day Breakfast and Training brought to you by Avidity Biosciences

THURSDAY, SEPTEMBER 7TH

- **Advocacy Day - Pre-registration Required**
  8:00 am - 10:30 am
  Potomac Ballroom Pre-function Area
  Registration for the conference and Advocacy Day will be open prior to breakfast.
- **Advocacy Day Training Breakfast**
  9:00 am - 10:00 am
  Receive your Advocacy Day Packets and meet your fellow delegates and state captain for a breakfast training.
- **Buses to Capitol**
  10:30 am - 4:00 pm
  For registered Advocacy Day attendees only.
  Buses to the Capitol with wheelchair accommodation capability will run every 15 minutes between the hotel and the Senate and House stops on Capitol Hill. Please plan to leave the hotel at least 45-minutes in advance of your scheduled meetings, and/or Congressional Briefing Lunch.
- **Congressional Briefing Lunch**
  12:30 pm - 1:30 pm
  Russell Building, Room 385
  Join us for a MDF sponsored U.S. Senate lunch briefing on how federal research funding is advancing our knowledge of the causes of DM and moving us closer to new treatments and a cure. Senator Amy Klobuchar (D-MN), a cosponsor of last year's Senate resolution declaring September 15th as International Myotonic Dystrophy Awareness Day, has been invited to make introductory remarks and we will be joined by senior scientists at the NIH, CDMRP, and Avidity Biosciences.
- **Meetings Debrief**
  3:30 pm - 4:30 pm
  Anacostia Ballroom Salon F
  After your congressional meetings, please visit us to drop off your one-page congressional feedback form that will provide advocates with an opportunity to share what you learned during your visits.
- **Early Arrival Reception & 2023 Congressional Leadership Awards**
  6:00 pm - 7:30 pm
  Potomac Ballroom Salon 1 + 2
  Join us on the eve of the MDF Annual Conference for a reception honoring U.S. Senators Tim Kaine (D-VA) and Cynthia Lummis (R-WY), the sponsors of last year's International Myotonic Dystrophy Awareness resolution, who are being recognized with the 2023 MDF Congressional Leadership Award. The reception will include hors d'oeuvres, one drink ticket, and no host bar.

Agenda for Friday, September 8th

Time**	Potomac Ballroom (Salons 1+2)	Anacostia Ballroom (Salons D+E)	JOA Lounge Meeting Room 11+12	Wellness Room Brought to you by Harmony Biosciences Anacostia Ballroom (Salon F)
8:00 AM-9:00 AM		DM Professionals Breakfast		
9:00 AM-10:00 AM	First Timers' Tea	DM Professional 1*		Morning Chair Yoga with Ellen Shapiro
10:00 AM-11:00 AM	Community Breakfast	DM Professional 2*		Quiet Space
11:00 AM-11:45 AM	Welcome & State of the Foundation			
11:45 AM-12:00 PM	**Break**		JOA Lounge Opens	
12:00 PM-1:00 PM	**Breakout Sessions**	DM Professional 3*	JOA: Welcome & Get to Know You Games	Focus on Breathing Techniques with Lorna Jarrett
1:00 PM-1:15 PM	**Break**			Quiet Space
1:15 PM-2:15 PM	Networking Lunch			
2:15 PM-3:30 PM	DM Drug Development & Approval Considerations with the FDA		JOA: Building Shared Connections with Others	Afternoon Chair Yoga with Ellen Shapiro
3:30 PM-3:45 PM	**Break**			Fireside Chat: Managing Activities of Daily Living with Dr. Samar Muslemani
3:45 PM-4:45 PM	Industry Updates Part 1		JOA Entertainment: Magician & Mentalist David Gerard	Quiet Space
4:45 PM-5:00 PM	**Break**			
5:00 PM-6:00 PM	**Breakout Sessions**	Professional Networking & Social Hour	JOA: Networking / Games & Free Time	Fireside Chat: Effective & Empathetic Communication with Dr. Dedee Caplin
6:00 PM-6:30 PM	**Break**		JOA Lounge Closes	Wellness Room Closes
6:30 PM-8:30 PM	Dinner & Entertainment: Magician & Mentalist David Gerard			

🟩 General Sessions ◻ Community ■ Professional ■ JOA ■ Wellness ◻ Fireside Chats ■ Quiet Space

Agenda for Friday, September 8th *(continued)*

Time**	Potomac Ballroom (Salon 3)	Meeting Room 2	Meeting Room 3	Meeting Room 4	Meeting Room 8+9
8:00 AM- 9:00 AM					
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	DM1: Disease Development & Symptom Management	DM2: Disease Development & Symptom Management	Social Security Administration Demystifies the Benefits Process	CDM: Communication Considerations in CDM Workshop	DM1: Cognitive Impairment Considerations in Activities of Daily Living
1:00 PM- 1:15 PM	**Break**				
1:15 PM- 2:15 PM					
2:15 PM- 3:30 PM					
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	Managing Sleepiness & Sleep Disturbances (DM1 & 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 page 14.*
*** All times listed in Eastern Daylight Time.*

Agenda for Saturday, September 9th

Time**	Potomac Ballroom (Salons 1+2)	Anacostia Ballroom (Salons D+E)	JOA Lounge Meeting Room 11+12	Wellness Room Brought to you by Harmony Biosciences Anacostia Ballroom (Salon F)
8:00 AM-9:00 AM		DM Professionals Breakfast		Morning Gentle & Chair Pilates with Lorna Jarrett
9:00 AM-10:00 AM	Community Breakfast	DM Professional 4* DM Professional 5*		Quiet Space
10:00 AM-11:00 AM	Welcome & Stories of Inspiration		JOA Lounge Opens/ Free Time	
11:00 AM-11:15 AM	Break	DM Professional 6*		
11:15 AM-12:15 PM	Breakout Sessions		JOA: Let's Talk About Feelings	Fireside Chat: Clinical Trials & Studies with Dr. Nick Johnson
12:15 PM-12:30 PM	Snack Break			
12:30 PM-1:30 PM	Exhibitor & Research Poster Showcase		JOA Lounge Free Time	Afternoon Gentle & Chair Pilates with Lorna Jarrett
1:30 PM-1:45 PM	Break			Quiet Space
1:45 PM-2:45 PM	Lunch	SAC Meeting (Private)		
2:45 PM-3:45 PM	Industry Updates Part 2		JOA Karaoke/ Free Time	Fireside Chat: How to Raise Awareness for DM with Kevin Brennan
3:45 PM-4:00 PM	Break			Quiet Space
4:00 PM-5:00 PM	Breakout Sessions		Stump the Doctor: JOA	
5:00 PM-5:15 PM	Break			
5:15 PM-6:15 PM	Breakout Sessions		JOA Lounge Free Time	Fireside Chat: Research Report Out with Dr. Tina Duong
6:00 PM-6:30 PM	**Break**		JOA Lounge Closes	Wellness Room Closes
6:30 PM-8:30 PM	Closing Dinner & Dance Party			

■ General Sessions □ Community ■ Professional ■ JOA ■ Wellness □ Fireside Chats ■ Quiet Space

Agenda for Saturday, September 9th *(continued)*

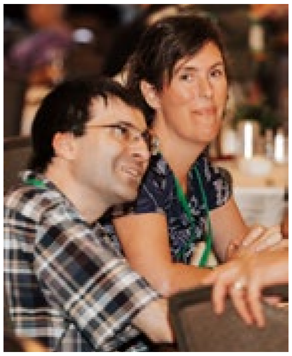
Time**	Potomac Ballroom (Salon 3)	Meeting Room 2	Meeting Room 3	Meeting Room 4	Meeting Room 8+9
8:00 AM-9:00 AM		Global Alliance Breakfast (Private)			MDF Fellows Breakfast (Private)
9:00 AM-10:00 AM					
10:00 AM-11:00 AM					
11:00 AM-11:15 AM	Break				
11:15 AM-12:15 PM	DM1 Community Panel: Life Hacks	Unaffected Male Caregivers Community Panel: Self-care	Unaffected Female Caregivers Community Panel: Managing Grief & Shame		DM2 Community Panel: Surviving & Thriving with DM2
12:15 PM-12:30 PM	Snack Break				
12:30 PM-1:30 PM					
1:30 PM-1:45 PM	Break				
1:45 PM-2:45 PM					
2:45 PM-3:45 PM					DM2: Gastrointestinal Considerations
3:45 PM-4:00 PM	Break				
4:00 PM-5:00 PM	Stump the Doctor: DM1	Stump the Doctor: Mobility			Stump the Doctor: DM2
5:00 PM-5:15 PM	Break				
5:15 PM-6:15 PM	New Horizons in the DM Field	DM2: Cardiac Considerations		Improving Quality of Life Through Palliative Care	Mental Health & DM: Answering Your Questions
6:00 PM-6:30 PM	Break				
6:30 PM-8:30 PM					

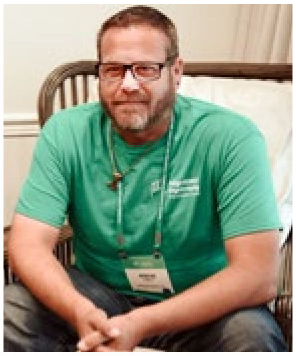
🟦 DM1 🟥 DM2 🟪 Caregivers ⬜ CDM

** View full professional session title & speaker information on page 14.*

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

Joining Virtually





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

Suggested setup:

- A good 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 **SwapCard** and **Zoom** apps from the **Apple App Store** or **Google Play Store**

Joining Virtually:

- Check your email for an email from **noreply@swapcard.com** inviting you to create your profile. (Make sure to check your Junk & Spam folders)
- Already have a profile? Visit **https://app.swapcard.com/** and log in!
- New to SwapCard or forgot your password? Visit **https://app.swapcard.com/**, enter the email you registered for the conference with, and click "Send me a magic link"

Before the Conference:

- Build your personal conference agenda:
  - Once logged into the SwapCard website or app, click on **Agenda**
  - Click on a Session to find speaker information and a description of the presentation
  - Click on the Bookmark Icon 🔖 to add it your personal agenda
  - Quickly access your saved sessions by navigating to **My Event**
- Get to know attendees, speakers, sponsors and exhibitors
  - To access a directory of all conference attendees, click on **Attendees**
  - To find out more about our presenters, click on **Speakers**
  - Click on a person's name to access their profile
  - If they accept your connection request, you can learn more about them, send files and messages, and start impromptu video calls!

At the Conference

- To join a session, navigate to its detail page by clicking **Agenda** or **My Event**
- Chat with others and submit questions to the presenters using the **Live Discussion** box
- Visit our exhibitor showcase by clicking on **Exhibitors**

Enjoy the Conference!

Need Technical Support?

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

During the Conference on September 8th and 9th, join a Live Support Zoom from 9:00 am - 5:00 pm EST: **https://myotonic.org/2023-tech-support**

Breakout Sessions

DM1 Topics & Talks

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

FRIDAY, SEPTEMBER 8TH

- **DM1: Disease Development and Symptom Management**
  12:00 pm - 1:00 pm
  Potomac Ballroom Salon 3
  Johanna Hamel, MD
  University of Rochester Medical Center
  This session is ideal for those new to the DM1 community and those seeking a refresher. Learn from a leading expert about the causes and genetics underlying DM1, how and when in life it can affect different symptoms of the body, tips for self-management of symptoms, and how to best work with your clinical care team to ensure the best quality of life.

- **DM1: Cognitive Impairment Considerations in Activities of Daily Living**
  12:00 pm - 1:00 pm
  Meeting Room 8+9
  Samar Muslemani, MOT, MSc, PhD
  Universite de Sherbrooke
  Issues related to cognitive impairments are sometimes misunderstood, especially when considering how they may relate to independence and activities of daily living (ADLs). These misunderstandings can sometimes create conflicts or prejudice. Learn from a leading expert about how cognitive impairments influence the ability to accomplish ADLs and social roles. And how occupational therapists and other healthcare professionals can help with ADL difficulties related to cognitive impairments.

- **DM1 Networking Session & Social Hour**
  5:00 pm - 6:00 pm
  Potomac Ballroom Salon 3
  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, SEPTEMBER 9TH

- **Community Discussion Panel:**
  Those Affected by DM1: Life Hacks – Sharing Everyday Victories Through Useful Tips, Tricks, and Adaptive Devices
  11:15 am - 12:15 pm
  Potomac Ballroom Salon 3
  Mark Coplin, MDF Support Group Facilitator
  Julie Getzkin, DM1 Community Member
  Laura Gershenson, DM1 Community Member
  Community Discussion Panels at the conference are sessions lead by MDF volunteer community leaders. Each community has a dedicated discussion panel and topics chosen by community leaders.

- **Stump the Doctor: DM1**
  4:00 pm - 5:00 pm
  Potomac Ballroom Salon 3
  Ericka Greene, MD, Houston Methodist Hospital
  Stump the Doctor Sessions at the conference are an opportunity for community members to meet a leading expert in myotonic dystrophy and ask their most persistent, challenging questions. *Can you stump the doctor?*

Check out our other Community Topics and Talks on page 12

- Social Security Administration Demystifies the Benefits Process
- Managing Sleepiness & Sleep Disturbances
- Stump the Doctor: Mobility
- New Horizons in the DM Field
- Mental Health Considerations: Answering Your Questions
- Improving Quality of Life Through Palliative Care

COVID-19 Precautions

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 all the sessions live through our virtual platform, SwapCard (see page 8 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.

Breakout Sessions

DM2 Topics & Talks

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

FRIDAY, SEPTEMBER 8TH

- **DM2: Disease Development & Symptom Management**
  12:00 pm - 1:00 pm
  Meeting Room 2
  Chad Heatwole, MD, MS
  University of Rochester Medical Center
  This session is ideal for anyone within the DM2 community. Learn from a leading expert about the causes and genetics underlying DM2, how and when in life it can affect different systems in the body, monitoring, and how best to self-manage and work with your care team to ensure the best quality of life. Learn, too, about the latest research in DM2 and how you can get involved.

- **DM2: Networking Session & Social Hour**
  5:00 pm - 6:00 pm
  Meeting Room 2
  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.

Check out our other Community Topics and Talks on page 12

- Social Security Administration Demystifies the Benefits Process
- Managing Sleepiness & Sleep Disturbances
- Stump the Doctor: Mobility
- New Horizons in the DM Field
- Mental Health Considerations: Answering Your Questions
- Improving Quality of Life Through Palliative Care

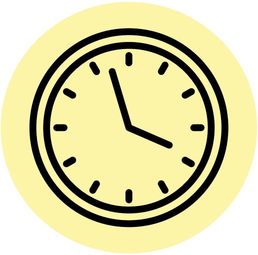
SATURDAY, SEPTEMBER 9TH

- **Community Discussion Panel: Those Affected by DM2: Surviving & Thriving with DM2**
  11:15 am - 12:15 pm
  Meeting Room 8+9
  Elizabeth Costa, DM2 Community Member
  Haley Martinelli, ESQ, MDF Board Member, Support Group Facilitator
  Lynn Schneider, DM2 Community Member
  Scott Virgo, DM2 Community Member
  Community Discussion Panels at the conference are sessions lead by MDF volunteer community leaders. Each community has a dedicated discussion panel and topics chosen by community leaders.

- **DM2: Gastrointestinal Considerations in DM2**
  2:45 pm - 3:45 pm
  Meeting Room 8+9
  Irene Sonu, MD
  Stanford University
  This session will explore the ways in which DM2 affects the GI system, treatment and management strategies aimed at alleviating GI issues, and improving quality of life.

- **Stump the Doctor: DM2**
  4:00 pm - 5:00 pm
  Meeting Room 8+9
  Araya Puwanant, MD
  Wake Forest University
  Stump the Doctor Sessions at the conference are an opportunity for community members to meet a leading expert in myotonic dystrophy and ask their most persistent, challenging questions. *Can you stump the doctor?*

- **DM2: Cardiac Considerations**
  5:15 pm - 6:15 pm
  Meeting Room 2
  Pradeep P.A. Mammen, MD, FACC, FAHA, FHFSA
  University of Kansas Medical Center
  Learn from a leading expert about the effect of DM2 on the heart.



Taking Breaks

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

Don't forget you may also attend sessions online. See page 8 for detailed instructions on joining virtually!

Breakout Sessions

Caregivers Topics & Talks

This track was designed with community input for unaffected caregivers as well as individuals with any type of DM interested in the various topics.

FRIDAY, SEPTEMBER 9TH

- **Caregivers Networking Session & Social Hour**
  5:00 pm - 6:00 pm
  Meeting Room 3
  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, SEPTEMBER 9TH

- **Community Discussion Panel: Unaffected Male Caregivers: Practical Tactics for Self-Care**
  11:15 am - 12:15 pm
  Meeting Room 2
  Stephen Price, Caregiver
  John Fitzpatrick, MDF Board Member
  Alan Layman, Caregiver
  Community Discussion Panels at the conference are sessions lead by MDF volunteer community leaders. Each community has a dedicated discussion panel and topics chosen by community leaders.

- **Community Discussion Panel: Unaffected Female Caregivers: Practical Tactics for Managing Grief and Shame**
  11:15 am - 12:15 pm
  Meeting Room 3
  Lois Schenk, Caregiver
  Charlotte Schenk, Caregiver
  Samantha Welsh, MDF Support Group Facilitator
  Community Discussion Panels at the conference are sessions lead by MDF volunteer community leaders. Each community has a dedicated discussion panel and topics chosen by community leaders.

CDM Family Topics & Talks

These sessions were designed with community input for families with children living with CDM.

FRIDAY, SEPTEMBER 8TH

- **CDM: Communication Considerations in Congenital Myotonic Dystrophy Workshop**
  12:00 pm - 1:00 pm
  Meeting Room 4
  Kiera Berggren, MA/CCC-SLP, MS
  Virginia Commonwealth University
  Lauren Brand, MS, CCC-SLP
  Lauren Brand Speech
  This working session begins with a short presentation on communication concerns in CDM, followed by a structured discussion on the consideration for parents and caregivers regarding communication in children with CDM.
- **CDM Family Networking Session & Social Hour**
  5:00 pm - 6:00 pm
  Meeting Room 4
  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.

Thank You to Our Advocacy Day State Captains!

We appreciate your leadership educating your legislators on Capitol Hill.

Rebecca Coplin *Oregon*
Loraine Dressler *California*
Belen Esparis *Pennsylvania*
Jodie Howell *Virginia*
Chuck Hunt *Georgia*
Emily Jones *New York*
Haley Martinelli *Ohio*
Samantha Welsh *Virginia*

Take A Picture!

Don't let Conference memories fade away; showcase your experience! Post to social media, tag **@MyotonicStrong**, and use the hashtags **#MDF2023** 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!

Breakout Sessions

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, SEPTEMBER 8TH

- **Social Security Administration Demystifies the Benefits Process**
  12:00 pm - 1:00 pm
  Meeting Room 3
  Diana Varela, Public Affairs Specialist
  Social Security Administration
  Joyce Lee, Medical Relations Officer
  Department on Disability Services
  Gain a better understanding of the role of the Social Security Administration (SSA) programs, benefits, and services available to the DM community in the US. Leaders from the SSA will join this panel to help demystify the application, denial, and appeal process.
- **Managing Sleepiness & Other Sleep Disturbances**
  5:00 pm - 6:00 pm
  Meeting Room 8+9
  John Day, MD, PhD
  Stanford University
  This session will cover sleepiness and other sleep disturbance considerations for both DM1 and DM2. Learn from a leading expert about sleepiness, and other sleep disturbances related to DM1 and DM2, best practices for care, monitoring, and self-management. Learn also about the latest research and how you can get involved.

SATURDAY, SEPTEMBER 9TH

- **Stump the Doctor: Mobility**
  4:00 pm - 5:00 pm
  Meeting Room 2
  Tina Duong, MPT, PhD, Stanford University
  Katy Eichinger, PhD, DPT, University of Rochester
  Stump the Doctor Sessions at the conference are an opportunity for community members to meet a leading expert in myotonic dystrophy and ask their most persistent, challenging questions. *Can you stump the doctor?*
- **New Horizons in the DM Field**
  5:15 pm - 6:15 pm
  Potomac Ballroom Salon 3
  John Day, MD, PhD, Stanford University
  Charles Thornton, MD, University of Rochester
  Hear about the significant progress in the DM field from leading experts during this panel discussion. Learn about the impact the community can have on continuing the progress toward treatments and a cure.
- **Mental Health & Myotonic Dystrophy: Answering Your Questions**
  5:15 pm - 6:15 pm
  Meeting Room 8+9
  Melissa Dixon, PhD, University of Utah
  Benjamin Gallais, PhD, Universite de Sherbrooke
  Ruth Sheldon, MPH, MSW,
  Myotonic Dystrophy Foundation
  Join this panel of experts as they share insight and engage in dialogue with the audience to answer questions about mental health, and the new MDF Mental Health Handbook. Visit the Mental Health Handbook poster in the Poster Showcase and add your anonymous question to be asked during the session.
- **Improving Quality of Life Through Palliative Care**
  5:15 pm - 6:15 pm
  Meeting Room 4
  Laurie Gutmann, MD
  Indiana University
  Palliative care is specialized medical care for people living with a serious illness. This type of care is focused on providing relief from the symptoms and stress of the illness with a goal to improve quality of life at any stage. Learn from a leading expert in palliative care on how this specialty can support those affected by DM and their families.

Join Us Virtually!

Can't attend a session in person? Don't worry, MDF has you covered. You can join us live through this year's virtual platform, SwapCard. You'll be able to see the presenter, view the presentation slides, and even ask questions. Check out page 8 for detailed instructions on how to join.

Breakout Sessions

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 **Meeting Rooms 10+11.**

Please note that these sessions are for JOA individuals only. All other attendees should participate in the other sessions.

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)
- Karaoke

FRIDAY, SEPTEMBER 8TH

- **Getting to Know You & Ice-Breaker Game**
  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!
- **Building Social Connections with Others**
  2:15 pm - 3:30 pm
  Melissa Dixon, PhD
  University of Utah
  This session will focus on working with the group on how to build social ties to promote meaningful connections with friends and significant others. This session is open only to JOA community members.
- **Entertainment: Magician & Mentalist David Gerard**
  3:45 pm - 4:45 pm
  Friday night's entertainer, Magician and Mentalist David Gerard, will be on hand in the JOA Lounge for a special engagement.
- **JOA Networking & Social Hour**
  5:00 pm - 6:00 pm
  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, SEPTEMBER 9TH

- **Let's Talk About Feelings**
  11:15 am - 12:15 pm
  Melissa Dixon, PhD
  University of Utah
  Back by popular demand, this session will focus on how feelings affect our bodies and minds. This will include an interactive discussion about strategies for learning to manage overwhelming emotions.
- **Stump the Doctor: JOA**
  4:00 pm - 5:00 pm
  Jacinda Sampson, MD, PhD
  Stanford University
  Stump the Doctor Sessions at the conference are an opportunity for community members to meet a leading expert in myotonic dystrophy and ask their most persistent, challenging questions. *Can you stump the doctor?*

Join the JOA Warriors every third Tuesday at 4pm Eastern online! This virtual support group is for individuals diagnosed with DM as young adults. For more information about this group or the next meeting, contact Ann at **info@myotonic.org**

Thank You!

The Myotonic Dystrophy Foundation extends its sincere appreciation to the **Cohen Family Trust & AHLIFE** for their scholarship program supporting juvenile-onset adult attendance at the 2023 MDF Annual Conference.

Breakout Sessions

Professionals Topics & Talks

All professional sessions, including breakfast, are located in **Anacostia Ballroom D+E.**

We are excited to bring together leading scientists, clinicians, and experts in the field to present the latest findings, cutting-edge research, and innovative approaches to understanding and treating myotonic dystrophy. This track was designed with expert guidance from members of MDF's Scientific Advisory Committee. Attendees must have a professional registration. The 2023 MDF Annual Conference Professional track serves as a crucial platform for knowledge exchange, collaboration, and collective efforts to advance research efforts, all with the shared goal of accelerating progress towards effective treatments and ultimately, a cure for myotonic dystrophy.

FRIDAY, SEPTEMBER 8TH

- **DM Professionals Breakfast**
  8:00 am - 9:00 am
- **Professional Session 1**
  9:00 am - 9:30 am
  Krzysztof Sobczak, PhD
  Instytut Biologii Molekularnej i Biotechnologii, Adam Mickiewicz University, Poznań, Poland
  Involvement of Aberrant Splicing of the NFIX Transcription Factor in Transcriptome Changes in Muscles of DM Patients

  9:30 am - 10:00 am
  Kalak Reddy, PhD
  State University of New York at Albany's RNA Institute, New York, United States
  Toxic RNA Selective Screening to Identify New Drugs, Drug Targets and Genetic Modifiers for Myotonic Dystrophy
- **Professional Session 2**
  10:00 am - 10:30 am
  Cécile Martinat, PhD, I-Stem - Institut des cellules Souches pour le Traitement et l'Etude des maladies Monogéniques, Corbeil-Essonnes, France
  Use of Human Pluripotent Stem Cells for Deciphering Myotonic Dystrophy Type 1

  10:30 am - 11:00 am
  Auinash Kalsotra, PhD, Carl R. Woese Institute of Genomic Biology, Cancer Center@Illinois, Division of Nutritional Sciences, University of Illinois, Urbana-Champaign, Illinois, United States
  Myotonic Dystrophy Type 1 Adversely Affects Liver Function and Lipid Metabolism
- **Professional Session 3**
  12:00 pm - 12:30 pm
  Vinod Mootha, MD, University of Texas Southwestern Medical Center, Dallas, Texas, United States
  Age-related Corneal Disease Mediated by Expanded CUG Repeat RNA

  12:30 pm - 1:00 pm
  Darren Monckton, PhD
  University of Glasgow, Scotland, United Kingdom
  Genetic Modifiers of Huntington Disease: Biological Insights and Therapeutic Opportunities

SATURDAY, SEPTEMBER 9TH

- **DM Professionals Breakfast**
  8:00 am - 9:00 am
- **Professional Session 4**
  9:00 am - 9:30 am
  Mario Gomes-Pereira, PhD, Sorbonne Université, Inserm, Association, Institut de Myologie, Paris, France
  Brain Disease Mechanisms in Myotonic Dystrophy and Why Neurons Aren't the Whole Story
- **Professional Session 5**
  9:30 am - 11:00 am
  Thomas Cooper, MD, Moderator
  Research Poster Lightning Round
- **Professional Session 6**
  11:00 am - 11:30 am
  Johanna Hamel, MD, University of Rochester Medical Center, Rochester, New York, United States
  Disease Severity and Progression in Myotonic Dystrophy Type 2

  11:35 - 11:50 am
  Araya Puwanant, MD, MS
  Wake Forest University School of Medicine, Winston Salem, North Carolina, United States
  Insights Into muscle Pathology: Imaging Analysis and Clinical Endpoints in Myotonic Dystrophy Type 2

  11:50 am - 12:10 pm
  Avery Engelbrecht, Graduate AST-R, Center for NeuroGenetics, Department of Molecular Genetics and Microbiology, University of Florida at Gainesville, Florida, United States
  2022 MDF Fellow
  Generation and Characterization of a DM2 BAC Mouse Model

OTHER SESSIONS INCLUDE:

Build Connections for a Cure: Join the Myotonic Dystrophy Foundation's professional networking session. Connect with researchers, scientists, pharmaceutical representatives, government officials, and funders dedicated to advancing myotonic dystrophy research. Forge partnerships, share insights, and accelerate progress towards treatments and a cure. Don't miss this unique opportunity to collaborate and make a difference.
Friday, September 8, 5:00-6:00 PM

Accelerating Knowledge: Experience rapid-fire talks in our Lightning Round featuring top poster submissions on myotonic dystrophy. Predoctoral, postdoctoral, and early career scholars present their groundbreaking research, showcasing the most promising findings. Witness the future of myotonic dystrophy research condensed into concise, dynamic talks. Engage with rising stars in the field and gain valuable insights.
Saturday, September 9, 9:30- 11:00 AM

Discover Breakthroughs in Myotonic Dystrophy Research: Engage with cutting-edge research at our Poster Showcase. Pre-doctoral, post-doctoral, and early career scholars, alongside industry experts, present their innovative findings on myotonic dystrophy. Explore the latest advancements, network with leading researchers, and witness the potential for new treatments. Don't miss this vibrant display of scientific excellence.
Saturday, September 9, 12:30-1:30 PM

General Sessions

General sessions are for everyone! All general sessions are located in **Potomac Ballroom (Salon 1+2).**

THURSDAY, SEPTEMBER 7TH

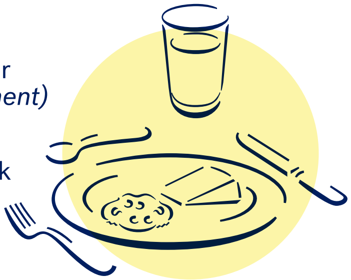
- **Early Arrival Reception & Advocacy Awards**
  6:00 pm - 7:30 pm
  Join us on the eve of the MDF Annual Conference to honor US Senators Tim Kaine (D-VA) and Cynthia Lummis (R-WY) in receiving MDF Advocacy Awards. The reception will include hors d'oeuvres, one drink ticket, and no host bar.

FRIDAY, SEPTEMBER 8TH

- **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!
- **Welcome & State of the Foundation**
  11:00 am - 11:45 am
  MDF leadership and Board of Directors will share exiting updates about the Foundation, International Myotonic Dystrophy Awareness Day, and discuss progress in the field of DM.
- **Networking Lunch – Networking Buddies Assigned Seating**
  1:15 pm - 2:15 pm
  Get to know your fellow attendees over lunch. If you registered to participate in the Networking Buddies program, please find your assigned table. See more about making the most out of the Networking Buddies program on page 17. Everyone else enjoy lunch with friends and family!
- **DM Drug Development & Approval Considerations with the FDA**
  2:15 pm - 3:00 pm
  Nicholas Johnson, MD, MSci., FAAN, Virginia Commonwealth University ; Michelle Campbell, PhD, Federal Drug Administration; Ami K. Mankodi, MD, Federal Drug Administration, Kevin Brennan, Jeannine DeSoi, Mark Planco, and Haley Martinelli
  Gain insight and understanding on the important role the FDA plays in the drug approval process through a presentation from the FDA. This informative presentation will be followed by a panel discussion with the FDA, a DM expert clinician, and members of the DM Community. This session provides an opportunity for our community and clinicians to share the serious medical challenges facing our community with the FDA.
- **Industry Updates - Part 1**
  3:45 pm - 4:45 pm
  Andrew Berglund, PhD, University at Albany, Li-Jung Tai, MD, PhD, Avidity Biosciences, Jeremy D. O'Connell, PhD, Juvena Therapeutics, Didier Rouy, MD, PhD, Sanofi Judy Walker, MD, ARTHEx Biotech
  With an opening statement from Dr. Andy Berglund, MDF Scientific Advisory Committee member, representatives from biotechnology and pharmaceutical companies will provide updates on their drug development efforts in the DM field.

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 snack break
- Saturday lunch
- Saturday dinner



- **Welcome Dinner & Entertainment**
  6:30 pm - 8:30 pm
  Celebrate the end of a wonderful Day 1 at the MDF Annual Conference through the wonder of tonight's entertainer, Magician and Mentalist David Gerard. Dinner, two drink tickets, and no host bar will be included.

SATURDAY, SEPTEMBER 9TH

- **Community Breakfast**
  9:00 am - 10:00 am
  Join friends, family, and other attendees for breakfast
- **Stories of Inspiration from the DM Community**
  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 5th Annual Kayla Vittek Memorial Award for Outstanding Community Advocate, Support Group Facilitator Warrior Award, and Lifetime Achievement Award.
- **Exhibitor & Research Poster Showcase**
  12:30 pm - 1:30 pm
  Network and connect with poster authors conducting DM research, industry partners, and others working in the DM space.
- **Networking 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.
- **Industry Updates Part 2**
  2:45 pm - 3:45 pm
  Ash Dugar, PhD, MBA, Dyne Therapeutics, Ashling Holland, PhD, PepGen Inc., TBD, Harmony Biosciences; Heather Sadlish, PhD, Regenta Therapeutics, Mike Snape, AMO Pharma
  Companies will provide updates on their drug development efforts in the DM Field.
- **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!

Wellness Room Sessions

New to this year's conference is the Wellness Room located in **Anacostia Ballroom Salon F.** Throughout Friday & Saturday, the Wellness Room welcomes you to take advantage of a delightful array of activities designed to rejuvenate attendees:

Quiet Spaces	Step away from the conference buzz to find tranquility in our designated quiet space (see Agenda on pages 4 & 6)
Movement & Mindfulness Moments	Join our experienced instructors specializing in working with DM and/or other neuromuscular disease communities for invigorating movement moments and soothing mindfulness exercises.
Fireside Chats	Engage with leading experts in insightful 45-minute-long small group discussions covering a wide range of topics.

Wellness Room
brought to you by
Harmony Biosciences

Please be aware that space is limited on a first come first serve basis for all Wellness Room activities. Find each session's maximum capacity listed below. *Wellness Room sessions will NOT be provided virtually.*

FRIDAY, SEPTEMBER 8TH

- **Movement: Morning Chair Yoga**
  8:00 am - 8:45 am | Capacity: 20
  Ellen Shapiro, C-IAYT
  This 45-minute movement session is for all levels and experiences.
- **Mindfulness: Focus on Breathing Techniques**
  12:00 pm - 12:45 pm | Capacity: 30
  Lorna Jarrett, LPTA, MS, AIB-VR/CON, NSCA.
  This 45-minute session provides an overview on mindful breathing techniques.
- **Movement: Afternoon Chair Yoga**
  2:15 pm - 3:00 pm | Capacity: 20
  Ellen Shapiro, C-IAYT
  This 45-minute movement session is for all levels and experiences.
- **Fireside Chat: Managing Activities of Daily Living**
  3:00 pm - 3:45 pm | Capacity: 40
  Samar Muslemani, MOT, MSc, PhD
  Universite de Sherbrooke
  Myotonic Dystrophy can impact all aspects of your life. This session focuses on the tools and resources available to improve the everyday life of people living with DM. Whether or not you attended Dr. Muslemani's session on this topic earlier in the day, this fireside chat is an excellent opportunity to ask questions and learn from fellow attendees.
- **Fireside Chat: Effective & Empathetic Communication**
  5:00 pm - 5:45 pm | Capacity: 40
  Dedee Caplin, PhD
  University of Utah
  This session provides an excellent opportunity to explore ways of communicating with your workplace, healthcare team, friends, and family. Learn how to share your experiences with people who don't understand DM or its impact.

SATURDAY, SEPTEMBER 9TH

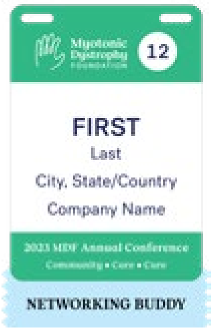
- **Movement: Morning Gentle & Chair Pilates**
  8:00 am - 8:45 am | Capacity: 20
  Lorna Jarrett, LPTA, MS, AIB-VR/CON, NSCA
  This 45-minute movement session is for all levels and experiences.
- **Fireside Chat: Clinical Trials & Studies**
  11:15 am - 12:00 pm | Capacity: 40
  Nicholas Johnson, MD, MSci, FAAN
  Virginia Commonwealth University
  Join us for an inspiring discussion on the rapid progress in DM research, bringing hope for new treatments and a cure. Dr. Nick Johnson will lead the discussion and answer your questions on participating in clinical trials, natural history studies, and more!
- **Movement: Afternoon Gentle & Chair Pilates**
  12:00 pm - 1:15 pm | Capacity: 20
  Lorna Jarrett, LPTA, MS, AIB-VR/CON, NSCA
  This 45-minute movement session is for all levels and experiences.
- **Fireside Chat: How to Raise Awareness for DM**
  2:45 pm - 3:30 pm | Capacity: 40
  Kevin Brennan
  Bluebird Strategies
  Make a difference! Learn how you and your family can raise DM awareness in your communities and influence local and national governments. Whether you joined MDF's Advocacy Day or are simply eager to create change, this session is a great opportunity to learn how you can get involved. Don't miss this chance to be a driving force in DM awareness and advocacy!
- **Fireside Chat: Good Move! Upcoming Technologies Changing the Way We Assess Human Movement**
  5:15 pm - 6:00 pm | Capacity: 40
  Tina Duong, MPT, PhD
  Stanford
  Accurate measurement of human movement is essential in the context of clinical trials and functional assessments. An innovative smartphone video-based movement analysis tool, OpenCap, shows potential to elevate existing trial outcomes. Join us in a discussion to explore optimal ways to develop this technology to meet your needs.

Networking Buddies

Be the bridge between the DM Community & Research Professionals!

In response to community feedback, we've designed some new and 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.

Networking Buddies will be assigned a table number at check-in for Friday's Networking Lunch. Networking Buddies will be seated together. We encourage you to continue to connect with your buddies throughout the conference during breaks, sessions, and mealtimes.



Lookout for the Networking Buddy name badge ribbon!

If you signed up to be a Networking Buddy, you will be assigned at least one Buddy. We invite you to connect with as many Networking Buddies as you can!

What Should You Do with Your Buddies?

- **Introduce yourself:**
  - *Where are you from?*
  - *How are you connected to DM?*
  - *Is this your first conference?*
- **Community Member questions to Professionals:**
  - *What do you do in the DM field?*
  - *What got you interested in working in DM?*
  - *What has you most excited about the future of this field?*
  - *Are you giving a talk/sharing a poster here?*
- **Professionals questions to Community Members:**
  - *What do you wish clinicians/researchers/other professionals knew about DM?*
  - *What have you found interesting in the sessions so far?*
  - *What's your favorite part of the conference?*
  - *What has you most excited or hopeful about the future of DM research?*

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! Need a break but don't want to miss a session? Use SwapCard to watch online! (see page 8).
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, and Communication Considerations in CDM Workshop. Wellness Room and JOA Lounge activities are only available in-person.)

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. 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. Each Movement Moment will focus on one of our three of the four types of movement: endurance, strength, stretching, and balance. All are welcome to participate if it is comfortable for you, or simply observe. *Note: adaptations for movements will be demonstrated.*

Friday Morning Movement Moment: BALANCE

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

Friday Afternoon Movement Moment: ENDURANCE EXERCISES

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.

Lorna Jarrett, LPTA, MS, AIB-VR/CON, NSCA is a health and wellness professional and pilates instructor with specialization in pilates for MS & neurological disorders. Lorna's training philosophy is to use Pilates as a tool with physical therapy protocols to move your body from function level to a level of optimal performance. Lorna will guide the audience through modified activity to get your body moving.

Saturday Morning Movement Moment: STRETCHING

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, 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.

Myotonic
Dystrophy
IN MOTION
www.myotonic.org

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

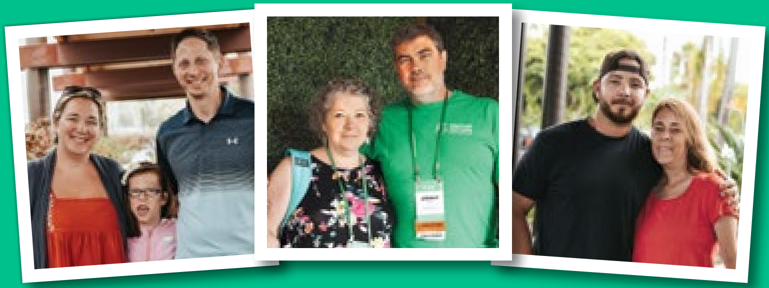
Engage with the Myotonic Dystrophy In Motion initiative: **www.myotonic.org/in-motion.**

We are grateful to the **MDF Movement Committee** for putting the Movement Moments together: Nathan Beucler; Luke Bolt; Teresa Cummings; Tina Duong, MPT, PhD; Katy Eichinger, PhD, PT, DPT, NCS; Lorrie Gallagher; Mindy Kim; and Ellen Shapiro.

Myotonic
Dystrophy
Family Registry

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

Join Today!



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

By joining the MDFR you will:

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

It's easy!
Log in now: www.myotonicregistry.org
***Questions?* Call us at 415-800-7777**

Presentation Abstracts Available Online!

MDF's DM Professional Track will provide presentation abstracts to support education and connection, and to act as an archive of the incredible proceedings of these sessions focused on the state of the science and care in myotonic dystrophy.

www.myotonic.org/professional-session-abstracts-2023-mdf-annual-conference

Research Poster Abstracts Available Online!

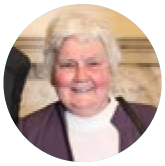
Traditional poster sessions combine a visual representation of the findings of a paper/study – the poster - with the opportunity for individualized, informal discussion of the presenter's work. MDF is thrilled to make this opportunity available at this year's conference in a virtual format, where remote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

www.myotonic.org/research-posters-2023-mdf-annual-conference

Conference Speakers

Kiera Berggren, MA/CC-SLP, MS
Speech Language Pathologist
Virginia Commonwealth University
CDM: Communication Considerations in Congenital Myotonic Dystrophy Workshop

Andy Berglund, PhD
Professor, Director, The RNA Institute, Department of Biological Sciences at University of Albany, MDF Scientific Advisory Committee
Welcome State of the Foundation, & Industry Updates Part 1



Margaret Bowler
Founding Chair
Myotonic Dystrophy Support Group UK
Stories of Inspiration

Lauren Brand, MS, CCC-SLP
Speech Language Pathologist
Lauren Brand Speech
CDM: Communication Considerations in Congenital Myotonic Dystrophy Workshop

Kevin Brennan
Principal, Bluebird Strategies
MDF Advocacy Consultant
Advocacy Day Breakfast and Training, Congressional Briefing, Advocacy Award Reception, DM Drug Development and Approval Considerations with the FDA, Fireside Chat: How to Raise Awareness for DM

Mindy Buchanan
Director of Programs
Myotonic Dystrophy Foundation
Stories of Inspiration

Michelle Campbell, PhD
Associate Director, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience
U.S. Food and Drug Administration
DM Drug Development and Approval Considerations with the FDA

Dedee Caplin, PhD
Clinical Director of Medical Psychology and Psychiatry Programs in Pediatric Behavioral Health, University of Utah
Wellness Room Fireside Chat: Effective & Empathetic Communication

Thomas Cooper, MD
S. Donald Greenberg and R. Clarence & Irene H. Fulbright Professor,
Baylor College of Medicine
MDF Scientific Advisory Committee
Research Poster Lightning Round

Mark Coplin
MDF Support Group Facilitator
DM1 Community Discussion Panel: Life Hacks Sharing Everyday Victories Through Useful Tips, Tricks and Adaptive Devices

Rebecca Coplin, MHA
MDF National Advocacy Committee
Advocacy Day: Advocacy Awards Reception

Elizabeth Costa
DM2 Community Member
Community Discussion Panel: Surviving and Thriving with DM2

Lindsey A. Criswell, MD, MPH, D.Sc.
Director, National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)
National Institutes of Health
Advocacy Day: Congressional Briefing

John Day, MD, PhD
Professor of Neurology, Pediatrics & Pathology, Stanford University School of Medicine, Board of Directors, Scientific Advisory Committee
Managing Sleepiness & Other Sleep Disturbances, New Horizons in the DM Field

Jeannine DeSoi
DM1 Community Member, MDF Support Group Facilitator
DM Drug Development and Approval Considerations with the FDA

Melissa Dixon, PhD
Visiting Assistant Professor, Program Director, Utah Program for Inherited Neuromuscular Disorders, Dept. of Pediatrics, University Of Utah
JOA: Building Social Connections, JOA: Let's Talk About Feelings, Mental Health & Myotonic Dystrophy: Answering Your Questions

Tina Duong, MPT, PhD
Physical Therapist, Clinical Research Manager, Stanford University, MDF Movement Committee
Stump the Doctor: Mobility, Wellness Room Fireside Chat: Good Move! Upcoming Technologies Changing the Way We Assess Human Movement, Movement Moments

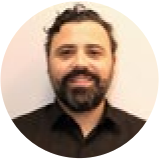
Katy Eichinger, PhD, DPT
Associate Professor, Dept. of Neurology
University of Rochester Medical Center,
MDF Movement Committee
Stump the Doctor: Mobility

Avery Engelbrecht, Graduate AST-R
University of Florida at Gainesville,
MDF Fellow
Generation and Characterization of a DM2 BAC Mouse Model

Belen Esparis, MD
MDF Board of Directors,
National Advocacy Committee
Advocacy Day: Advocacy Awards Reception, First Timers' Tea

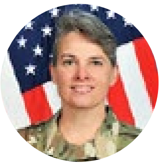
Conference Speakers *(continued)*

John Fitzpatrick
MDF Board of Directors
Community Discussion Panel: Practical Tactics for Self-Care for Unaffected Male Caregivers



Benjamin Gallais, PhD
Faculty of Medicine and Health Sciences
Universite de Sherbrooke
Mental Health & Myotonic Dystrophy: Answering Your Questions

Laura Gershenson
DM1 Community Member
DM1 Community Discussion: Life Hacks Sharing Everyday Victories Through Useful Tips, Tricks and Adaptive Devices



Colonel Sarah Goldman, PhD.
Director, Congressionally Directed Medical Research Programs (CDMRP)
U.S. Army Medical Research and Development Command
Advocacy Day: Congressional Briefing

Julie Getzkin
DM1 Community Member
DM1 Community Discussion: Life Hacks Sharing Everyday Victories Through Useful Tips, Tricks and Adaptive Devices

Mario Gomes-Pereira, PhD
Research Associate and Scientist
Sorbonne Universite, Inserm, Centre de Recherche en Myology, Paris, France
Brain Disease Mechanisms in Myotonic Dystrophy & Why Neurons Aren't the Whole Story

Ericka Greene, MD
Sumner Family Chair in Neuromuscular Research, Director of the Neuromuscular Clinic, Director of the Neurology Residency Program, Houston Methodist Hospital
Stump the Doctor: DM1

Laurie Gutmann, MD
Professor of Neurology, Chair, Department of Neurology
Indiana University
Improving Quality of Life Through Palliative Care

Johanna Hamel, MD
Assistant Professor of Neurology, Pathology and Laboratory Medicine
University of Rochester Medical Center
DM1: Disease Development and Management, Disease Severity and Progression in Myotonic Dystrophy Type 2

Lisa Harvey-Duren
MDF's Founding Executive Director
Stories of Inspiration

Chad Heatwole, MD, MS
Professor of Neurology, Director, Center for Health and Technology
University of Rochester Medical Center
DM2: Disease Development & Symptom Management

David Herbert
MDF Board of Directors
First Timers' Tea

Chuck Hunt
MDF Support Group Facilitator, National Advocacy Committee
Advocacy Day: Congressional Briefing

Lorna Jarrett, LPTA, MS, AIB-VR/CON, NSCA
Owner, Brain Peace-Mindset Coaching and Consulting, Adaptive Living Solutions
Wellness Room: Mindfulness: Focus on Breathing Techniques, Morning and Afternoon Gentle and Chair Pilates Movement Moments

Nicholas Johnson, MD, MSci, FAAN
Associate Professor and Vice Chair of Research in Neurology, Virginia Commonwealth University
MDF Scientific Advisory Committee
DM Drug Development and Approval Considerations with the FDA, Wellness Room Fireside Chat: Clinical Trials & Studies

Emily Jones
MDF Support Group Facilitator, National Advocacy Committee
Advocacy Day: Advocacy Awards Reception

Auinash Kalsotra, PhD
Assistant Professor of Biochemistry
University of Illinois at Urbana Champagne
Myotonic Dystrophy Type 1 Adversely Affects Liver Function and Lipid Metabolism

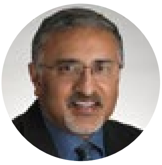
Jeremy Kelly
Chair, MDF Board of Directors
Welcome & State of the Foundation

Senator Amy Klobuchar *(Invited)*
U.S. Senator (D-MN)
U.S. Congress
Advocacy Day: Congressional Briefing

Alan Layman
Caregiver
Community Discussion Panel: Practical Tactics for Self-Care for Unaffected Male Caregivers

Conference Speakers *(continued)*

Joyce Lee
Medical Relations Officer
Disability Determination Division
Social Security Administration
Social Security Administration Demystifies the Benefits Process



Pradeep P.A. Mammen, MD, FACC, FAHA, FHFSA
Division Chief: Advanced Heart Failure Therapeutics and Cardiac Transplantation, Vice Chair for Translational Research
University of Kansas Medical Center
DM2: Cardiac Considerations

Ami K. Mankodi, MD
Medical Officer
U.S. Food and Drug Administration
DM Drug Development and Approval Considerations with the FDA

Cecile Martinant, PhD
President of the French Society for Stem Cell Research, I-Stem - Institut des cellules Souches pour le Traitement et l'Etude des maladies Monogéniques
Use of Human Pluripotent Stem Cells for Deciphering Myotonic Dystrophy Type 1

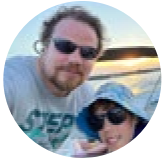
Haley Martinelli, Esq.
MDF Board of Directors, Support Group Facilitator
Community Discussion Panel: Surviving and Thriving with DM2, DM Drug Development and Approval Considerations with the FDA

Darren Monckton, PhD
Professor, University of Glasgow,
MDF Scientific Advisory Committee
Genetic Modifiers of Huntington Disease: Biological Insights and Therapeutic Opportunities, Stories of Inspiration

Vinod Mootha, MD
Professor, Department of Ophthalmology
University of Texas Southwestern Medical Center
Age-related Corneal Disease Mediated by Expanded CUG Repeat RNA

Samar Muslemani, MOT, MSc., PhD
Universite de Sherbrooke
DM1: Impact of Cognitive Impairments on Daily Living, Wellness Room Fireside Chat: Managing Activities of Daily Living

Mark Planco
DM1 Community Member
DM Drug Development and Approval Considerations with the FDA



Stephen Price
Caregiver
Community Discussion Panel: Practical Tactics for Self-Care for Unaffected Male Caregivers

Araya Puwanant, MD
Associate Professor in Neurology
Wake Forest University School of Medicine
Stump the Doctor: DM2, Insights Into Muscle Pathology: Imaging Analysis and Clinical Endpoints in Myotonic Dystrophy Type 2

Kaalak Reddy, PhD
Research Associate, Adjunct Assistant Professor, The RNA Institute, University at Albany
Toxic RNA Selective Screening to Identify New Drugs, Drug Targets and Genetic Modifiers for Myotonic Dystrophy Management

Jacinda Sampson, MD, PhD
Clinical Associate Professor in Neurology, Stanford Neuroscience Health Center, MDF Scientific Advisory Committee
Stump the Doctor: JOA

Charlotte Schenk
Caregiver
Community Discussion Panel: Practical Tactics for Managing Grief and Shame for Unaffected Female Caregivers

Lois Schenk
Caregiver
Community Discussion Panel: Practical Tactics for Managing Grief and Shame for Unaffected Female Caregivers

Lynn Schneider
DM2 Community Member
Community Discussion Panel: Surviving and Thriving with DM2

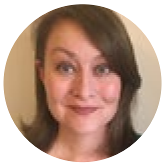
Ellen Shapiro, C-IAYT
Yoga Instructor
MDF Community Member,
Movement Committee
Wellness Room: Morning & Afternoon Chair Yoga, Movement Moments

Ruth Sheldon, MPH, MSW
Health Resources Coordinator
Myotonic Dystrophy Foundation
Mental Health & Myotonic Dystrophy: Answering Your Questions

Krzysztof Sobczak, PhD
Professor
Instytut Biologii Molekularnej i Biotechnologii, Adam Mickiewicz University
Involvement of Aberrant Splicing of the NFIX Transcription Factor in Transcriptome Changes in Muscles of DM Patients

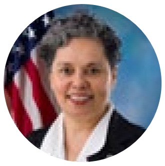
Irene Sonu, MD
Clinical Associate Professor
Stanford University
DM2: Gastrointestinal Considerations in DM2

Conference Speakers *(continued)*



Tanya Stevenson, EdD, MPH
Chief Executive Officer
Myotonic Dystrophy Foundation
Advocacy Day: Congressional Briefing, Welcome & State of the Foundation, Stories of Inspiration

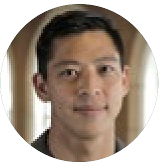
Charles Thornton, MD
Professor of Neurology, University of Rochester Medical Center, MDF Board of Directors, Scientific Advisory Committee
New Horizons in the DM Field



Diana Varela
Public Affairs Specialist
Social Security Administration
Social Security Administration Demystifies the Benefits Process

Scott Virgo
DM2 Community Member,
MDF Support Group Facilitator
Community Discussion Panel: Surviving and Thriving with DM2

Samantha Welsh
MDF Support Group Facilitator
Community Discussion Panel: Practical Tactics for Managing Grief and Shame for Unaffected Female Caregivers



Eric Wang, PhD
Associate Professor, Molecular Genetics & Microbiology, University of Florida, MDF Scientific Advisory Committee, National Advocacy Committee
Advocacy Day: Awards Reception

Industry Update Speakers

DAY 1

Andy Berglund, PhD
Professor, Director, The RNA Institute, Department of Biological Sciences University at Albany
Industry Update Introduction

Li-Jung Tai, MD, PhD
Executive Director, Clinical Development
Avidity Biosciences
An Update from Avidity Biosciences

Jeremy D. O'Connell, PhD
Co-Founder and Chief Scientific Officer
Juvena Therapeutics
Development of an Investigational Protein Drug to Treat Myotonic Dystrophy

Didier Rouy, MD, PhD
Associate Group Project Head
Sanofi
An Update on Sanofi's Gene Therapy Program for Myotonic Dystrophy Type 1

Judy Walker, MD
Chief Medical Officer
ARTHEx Biotech
ARTHEx' ATX-01 and the ArthemiR Trial

DAY 2

Ash Dugar, PhD, MBA
Senior Vice President,
Global Head of Medical Affairs
Dyne Therapeutics
Advancing FORCE™ in the Pursuit of Transformative Therapies for People Living with DM1

Ashling Holland, PhD
Director, Preclinical Development
PepGen, Inc.
PGN-EDODM1 Nonclinical Data Demonstrate Mechanistic and Meaningful Activity for Potential Treatment of Myotonic Dystrophy Type 1

David Seiden, MD
Senior Medical Director,
Clinical Development
Harmony Biosciences
Harmony Biosciences Update: Phase 2 Clinical Trial in Type 1 Myotonic Dystrophy

Heather Sadlish, PhD
Executive Director of Discovery Biology
Rgenta Therapeutics
Targeting Mismatch Repair Pathways to Treat Repeat Expansion Diseases such as DM1

Mike Snape, PhD
Chief Scientific Officer
AMO Pharma
AMO-02 in Myotonic Dystrophy - An Update

For more information from our industry partners, view MDF's Meet the DM Drug Developers series at: www.myotonic.org/meet-dm-drug-developers.

Exhibitor & Sponsor Showcase

Visit our amazing exhibitors during the conference! Tables are set up throughout the main conference floor.

AMO Pharma

Avidity Bio

Cure DM

DMCRN

Dyne Therapeutics

Global Alliance for Myotonic Dystrophy Awareness

DM Artisans Table

Myotonic Dystrophy in Motion

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Stanford University

Social Security Administration

University of Utah

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Wake Forest School of Medicine

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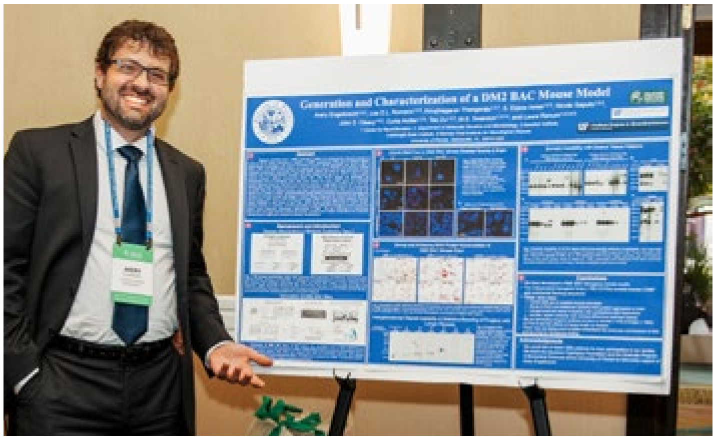


MDF Digital Academy

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**

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. Lean more and register here at: **myotonicregistry.patientcrossroads.org/**

Need additional support?
Call our Warmline at 415.800.7777

Meet the DM Drug Developer

From 2021 through 2023, 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**

Toolkits & Publications

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

Find-a-Doctor Map

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

MDF Research Fellows

We are pleased to introduce the 2022 and 2023 MDF Research Fellows, a group of exceptional predoctoral and postdoctoral scholars dedicated to advancing research in the field of myotonic dystrophy. With their diverse backgrounds and expertise, the MDF Research Fellows bring a fresh perspective and innovative approaches to tackle the challenges posed by this condition. To learn more about our MDF Research Fellows go to **www.myotonic.org/myotonic-dystrophy-research-fellows**.

Be sure to visit the Fellows' posters at the conference during the Poster Showcase to talk with them live about their research! View all the research posters online at: **www.myotonic.org/research-posters-2023-mdf-annual-conference**.

2023

Mackenzie Davenport, PhD
University of Florida

Julie Fortin, PhD
GRIMN, Quebec, CA

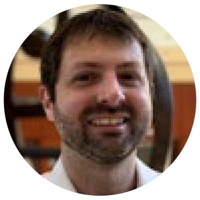
Tatiana Koike, PhD
Université de Montreal

Jiss Louis, PhD
The RNA Institute
University at Albany

Emma Shea
University of Florida

2022

Lily Cisco
University of Rochester



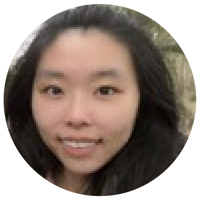
Avery Engelbrecht
University of Florida

Jesus Frias
The RNA Institute
University at Albany

Christina Heil, PhD
University of Rochester

Preeti Kumari, PhD
Massachusetts
General Hospital

Larissa Nitschke, PhD
Baylor College
of Medicine



Xiaomeng Xing
University of
Nottingham

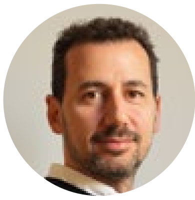
Keep in touch! The MDF monthly e-newsletter, the MDF Dispatch, provides you with up-to-date information on research advances, DM daily living strategies, upcoming events and stories from community members. It is the best way to stay current on news in the DM community.

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

MDF Early Career Researchers

We are excited to introduce the MDF Early Career Researchers, a group of promising early career scholars dedicated to advancing knowledge and innovation in the field of myotonic dystrophy. These talented individuals have shown great potential and passion for studying this complex neuromuscular disorder early in their careers. With their fresh perspectives and drive for discovery, the MDF Early Career Researchers are poised to contribute novel insights and advancements in understanding the mechanisms, diagnosis, and treatment of myotonic dystrophy. To learn more about our MDF Early Career Researchers go to:
www.myotonic.org/myotonic-dystrophy-research-grant-recipients.

Dylan Farnsworth, PhD
The RNA Institute
University at Albany



Matteo Garibaldi, MD, PhD
Sapienza University
of Rome

Melissa Hale, PhD
Virginia Commonwealth
University

Advocate for the DM Community

Your voice matters!

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:

INTERNATIONAL
Myotonic
Dystrophy
Awareness Day
SEPTEMBER 15

Keep up the momentum from MDF Advocacy Day!

- Attend a Fireside Chat with Kevin Brennan, MDF's advocacy consultant, this Saturday, September 9th at 2:45 pm in the Wellness Room!
- Follow up with your senators with in-person meetings, phone calls, letters, or emails! Find instructions, talking points, and letters templates at **www.myotonic.org/advocate**.

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**.

2023 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**.

Geography-based Support Groups & Facilitators

Arizona, USA

Teresa Cummings

Atlanta, GA, USA

Chuck Hunt

Canada
Support Group

Alexandra LeBoeuf

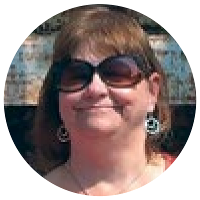
Canada
Support Group

Julie LeBoeuf

Chicago, IL, USA

Rob Besecker

Dallas, TX, USA



Sherry Morris

Florida, USA

Kristen McClintock

Kansas City
Region, USA

John Cooley

Kansas City
Region, USA

Patricia Gibson

Massachusetts, USA

Jeannine DeSoi

Massachusetts, USA

Bill Nuttall

Michigan, USA

Suzanne Perkins

Michigan, USA

Scott Virgo

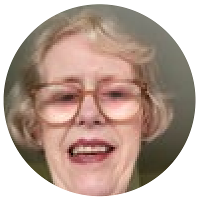
Mountain West
Region, USA

Kay Hayes

New York City Area

Janis Jaffe

New York City Area

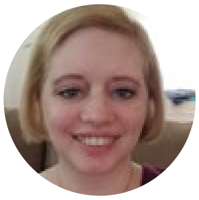


Susan "Glenda" Winson

New York City Area

Guillermo Zubillaga

N. & S. Carolina, USA



Mindy Kim

Ohio, USA

Carolyn Valek

Portland, OR, USA

Mark Coplin

Geography-based Support Groups & Facilitators *(cont.)*

Rochester, NY, USA

Emily Jones

San Diego, CA, USA

Rashid Kassir

S. California, USA

Loraine Dressler

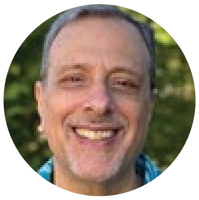
Virginia, USA

Jodie Howell, LPN, CCRP

Virginia, USA

Samantha Welsh

Washington State Region, USA



Jonathan Freedman

Washington State Region, USA

Cindy Hubert

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

Virtual Support Groups & Facilitators

f = Facebook Moderator

Caregivers of Children Living with CDM

Sarah Berman

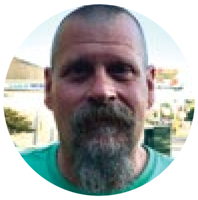
DM1 & Caregivers Facebook Group

Erin & Nathan Beucler

Caregivers of Children Living with CDM

Loraine Dressler

Caregiver Call-in Support Group



Ted Salwin

DM2 Caregivers Support Group

Kim McPeek

DM2 Virtual Support Group

Tom McPeek

DM2 Virtual Support Group

Haley Martinelli

Grupo de Soporte Virtual en Español

David Kugler

Grupo de Soporte Virtual en Español

Araceli Mera

Telefonische Gesprächsgruppe DM1 / DM2

Anke Klein

Telefonische Gesprächsgruppe DM1 / DM2

Bernhard Rogg

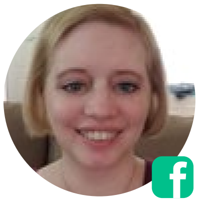
Juvenile-onset Adult (JOA) Warriors

Carolyn Valek

Juvenile-onset Adult (JOA) Warriors

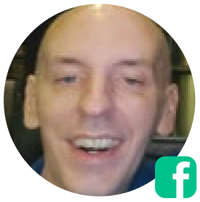
Ann Woodbury

Adult Facebook Chat, DM Virtual Happy Hour



Mindy Kim

Adult Facebook Chat



Bill Nuttall

DM2 Facebook Group

Kim Reynolds

Contribute to Community, Care, and a Cure!

In 2022, 87% of MDF's budget was dedicated to driving DM Research, Care, and Advocacy programs. With your support, we provide assistance and information to our community, fund groundbreaking research, raise awareness, and advocate with legislators and federal agencies. Help change the future of DM - donate now!

DONATE VIA PHONE
415.800.7777

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

DONATE ONLINE
www.myotonic.org/donate

2023 MDF Scientific Advisory Committee

The MDF 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: **www.myotonic.org/scientific-advisory-committee**.

Guillaume Bassez, MD, PhD
Institut de Myologie

Andy Berglund, PhD
The RNA Institute University at Albany

Kathie Bishop, PhD
Acadia Pharmaceuticals

Thomas A. Cooper, MD
Baylor College of Medicine

John W. Day, MD, PhD
Stanford University

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

Douglas Kerr, MD, PhD, MBA
Generation Bio

Darren Monckton, PhD
University of Glasgow

Laura Ranum, PhD
University of Florida

Jacinda Sampson, MD, PhD
Stanford University

Charles Thornton, MD
University of Rochester

Eric Wang, PhD
University of Florida

2023 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 Scientific Advisory Committee. To learn more about MDF Board of Directors go to **www.myotonic.org/board-directors**.

Jeremy Kelly
Board Chair & Lifetime Trustee

Martha Montag Brown
Vice-Chair

Elizabeth Florence, Esq.
Secretary

David Herbert
Treasurer

David Berman, MBA

John W. Day, MD, PhD

Belen Esparis, MD

John Fitzpatrick

Haley Martinelli, Esq.

Thomas "Tom" McPeek

Joel Revill

Charles Thornton, MD

2023 MDF Staff & Consultants

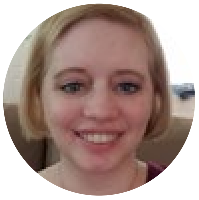
To learn more about MDF staff go to **www.myotonic.org/staff**.

Kate Beck
Director of Development

Kevin Brennan
Advocacy Consultant

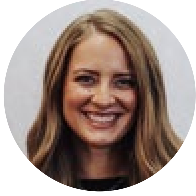
Mindy Buchanan
Director of Programs

Kleed Cumming
Director of Communications & Technology



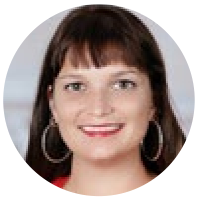
Mindy Kim
Registry Outreach Specialist

Sofia Olmos, PhD
Myotonic Dystrophy Family Registry Coordinator

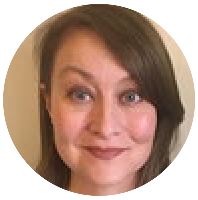


Emily Romney, MPA
Special Projects Manager

Ruth Sheldon, MPH, MSW
Health Resources Coordinator



Nadine Skinner, PhD, MPA
Research Coordinator



Tanya Stevenson, EdD, MPH
Chief Executive Officer

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GRITTGENE THERAPEUTICS

sanofi

LUPIN NEUROSCIENCES

astellas GENE THERAPIES

Visit their online booths at **https://app.swapcard.com/**.

Global Alliance for DM Awareness

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

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Algeria

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Australia

ABMM Telethon.be
Belgium

NMD4C
Canada

MDC DMC MUSCULAR DYSTROPHY CANADA DYSTROPHIE MUSCULAIRE CANADA
Canada

MUSKELSVINDFONDEN plads til forskellel
Denmark

EURO-DYMA
Europe

AFM TELETHON INNOVER POUR GUÉRIR
France

DGM Deutsche Gesellschaft für Muskelkranke e.V.
Germany

MDA ΕΛΛΑΣ
Greece

fm
Italy

CENTRO CLINICO NeMO
Italy

DM family
Japan

MD
Netherlands

SPIERZIEKTEN NEDERLAND
Netherlands

THE UNIVERSITY OF AUCKLAND NEW ZEALAND
New Zealand

INCLIVA | VLC
Spain

VNIVERSITAT DE VALÈNCIA
Spain

ASRIMM
Switzerland

LUPIN NEUROSCIENCES
Switzerland

AMO PHARMA
United Kingdom

Cure DM Research • Support • Awareness
United Kingdom

Myotonic Dystrophy SUPPORT GROUP
United Kingdom

Muscular Dystrophy UK Fighting muscle-wasting conditions
United Kingdom

NHS St George's University Hospitals NHS Foundation Trust Outstanding care every time
United Kingdom

TREAT-NMD Neuromuscular Network
United Kingdom

UK Myotonic Dystrophy Patient Registry Newcastle University John Walton
United Kingdom

NHS University College London Hospitals NHS Foundation Trust
United Kingdom

AskBio
USA

astellas GENE THERAPIES
USA

ATHENS ACADEMY
USA

AVIDITY BIOSCIENCES
USA

Dyne THERAPEUTICS
USA

entrada THERAPEUTICS
USA

EXPANSION THERAPEUTICS
USA

GRITTGENE THERAPEUTICS
USA

HARMONY BIOSCIENCES
USA

HOUSTON Methodist LEADING MEDICINE
USA

Juvena THERAPEUTICS Decoding Secreted Proteins
USA

K-STATE
USA

SCHOOL OF MEDICIN DEPARTMENT OF NEUROLOGY
USA

The Jackson Laboratory Leading the search for tomorrow's cures
USA

MDA Muscular Dystrophy Association
USA

Myotonic Dystrophy FOUNDATION
USA

Myotonic Dystrophy Family Registry
USA

USA

Paul D. Wellstone Muscular Dystrophy Research Centers
USA

PepGen
USA

Stanford MEDICINE
USA

UF Center for NeuroGenetics College of Medicine UNIVERSITY of FLORIDA
USA

THE RNA INSTITUTE COLLEGE OF ARTS & SCIENCES UNIVERSITY AT ALBANY
USA

UNIVERSITY OF IOWA HEALTH CARE
USA

U HEALTH UNIVERSITY OF UTAH
USA

VCU School of Medicine
USA

Wake Forest School of Medicine
USA

IDMC-13
Worldwide

Myotonic Dystrophy *IN MOTION*

www.myotonic.org

Learn more about the MDF's care initiative to unify, inspire, and encourage the international DM community to start moving!

Stop by the booth to find out more about the impact of movement and exercise in your daily life, and check out Myotonic Dystrophy in Motion (MDIM) resources.

Luke Bolt sported his Myotonic Dystrophy In Motion cycling jersey on his recent Seattle to Portland ride.

Get moving! Learn more here at:
https://www.myotonic.org/in-motion

Thank you to MDF's National Advocacy Committee!

Founded in 2022, the National Advocacy Committee leads the charge in executing MDF's advocacy campaign to increase DM awareness, grow DM research funding, and advance policies to accelerate the approval of drugs to treat and eventually cure DM.

Martha Montag Brown
(MDF, Vice-Chair)
California

Rob Besecker
Illinois

Rebecca Coplin
Oregon

Belen Esparis, MD
Pennsylvania

Charles Hunt
Georgia

Emily Jones
New York

Mindy Kim
North Carolina

Eric Wang, PhD
Florida

MYOTONIC DYSTROPHY CLINICAL RESEARCH NETWORK

CONTACT:

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NETWORK COORDINATOR
RUBY.LANGESLAY@VCUHEALTH.ORG

●

JENNIFER RAYMOND,
NETWORK COORDINATOR
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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 RESEARCH 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

UT SOUTHWESTERN

VIRGINIA COMMONWEALTH UNIVERSITY

EUROPE

CENTRO CLINICO NEMO

INSTITUT DE MYOLOGIE – PARIS

FRIEDRICH-BAUR INSTITUT - LMU MUNICH

RADBOUD UNIVERSITY – NL

ST. GEORGE'S, UNIVERSITY OF LONDON

UNIVERSITY COLLEGE LONDON

NEW ZEALAND

UNIVERSITY OF AUCKLAND

Our Motivation is

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Loraine

DM1 Advocate and her family

We are grateful for the important contributions of MDF and the patients and families that have participated in MARINA™ and other clinical studies of DM1.

Avidity Biosciences' commitment to innovative science is matched only by our passion to see patients' lives changed.

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 Myotonic Dystrophy Type 1 (DM1)

PepGen is advancing the next generation of oligonucleotide therapeutics, revolutionizing the treatment of severe neuromuscular disorders. Our enhanced delivery oligonucleotides (EDOs) are engineered to optimize delivery to tissues affected by DM1. Our mission is to deliver transformative therapies to improve the lives of people living with neuromuscular diseases, their families and the broader healthcare community.

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.

Contact

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

Email: community@pepgen.com

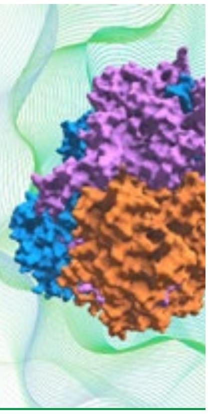
Visit our website to learn about our approach, our pipeline, our work with the DM community and upcoming clinical trials

AMO Pharma is committed to research that can lead to better health and a brighter future for people affected by myotonic dystrophy

AMO PHARMA

New Medicines. Better Lives.

www.amo-pharma.com




Where Empathy and Innovation Meet

Fund the Future of DM Research!

Fund-A-Fellow today!

The MDF Fund-a-Fellow program provides vital funding and resources to bright young DM scholars. By supporting their groundbreaking research, we aim to accelerate progress in understanding and treating DM, ultimately paving the way for improved care and, one day, a cure. Join us in providing hope by investing in our Fund-A-Fellow program.

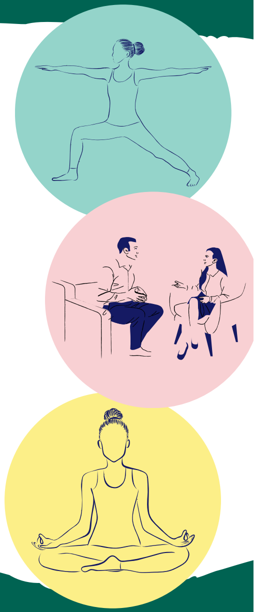
Learn more and donate at:
www.myotonic.org/fellows

Myotonic Dystrophy FOUNDATION

The Wellness Room

Brought to you by
Harmony Biosciences

Join us in Anacostia Ballroom F throughout Friday & Saturday!



Take advantage of a delightful array of activities designed to rejuvenate attendees:

- **Movement & Mindfulness Moments**
- **Fireside Chats**
- **Quiet Space**

Find more details on page 16.

Myotonic Dystrophy FOUNDATION

Questions About Mental Health & DM?

Join us on Saturday, September 9th for

Mental Health & DM: Answering Your Questions

Submit your questions at the Mental Health Research Poster (#62) by 2:45 PM on Saturday!

Don't miss your chance to join the conversation and get answers and insights from our panel of experts!

Myotonic Dystrophy FOUNDATION

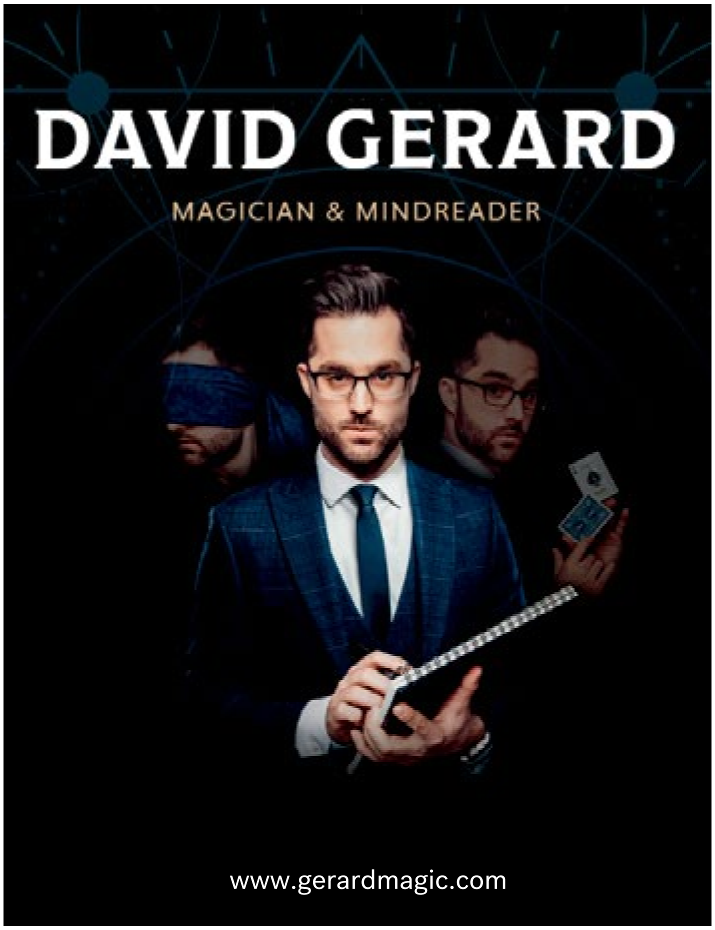
MYOTONIC DYSTROPHY FOUNDATION

2023 GALA

SAVE THE DATE
Saturday, November 11, 2023
Mauna Kea Resort, Hawaii

e ha 'awi I ka mana 'olana

TO GIVE HOPE



DAVID GERARD

MAGICIAN & MINDREADER

www.gerardmagic.com

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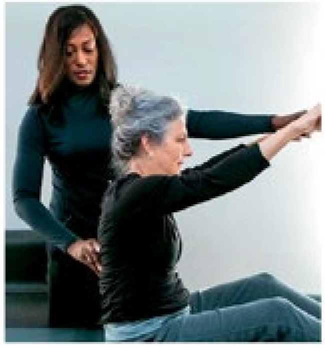
pnεũma
PILATES & WELLNESS

Lorna Jarrett DHSc (candidate) MS, NCPT specializes in pilates programming and has movement certifications and education to help her to establish safe and effective classes. Join her online Thursdays at 3:00pm CST or in the studio.



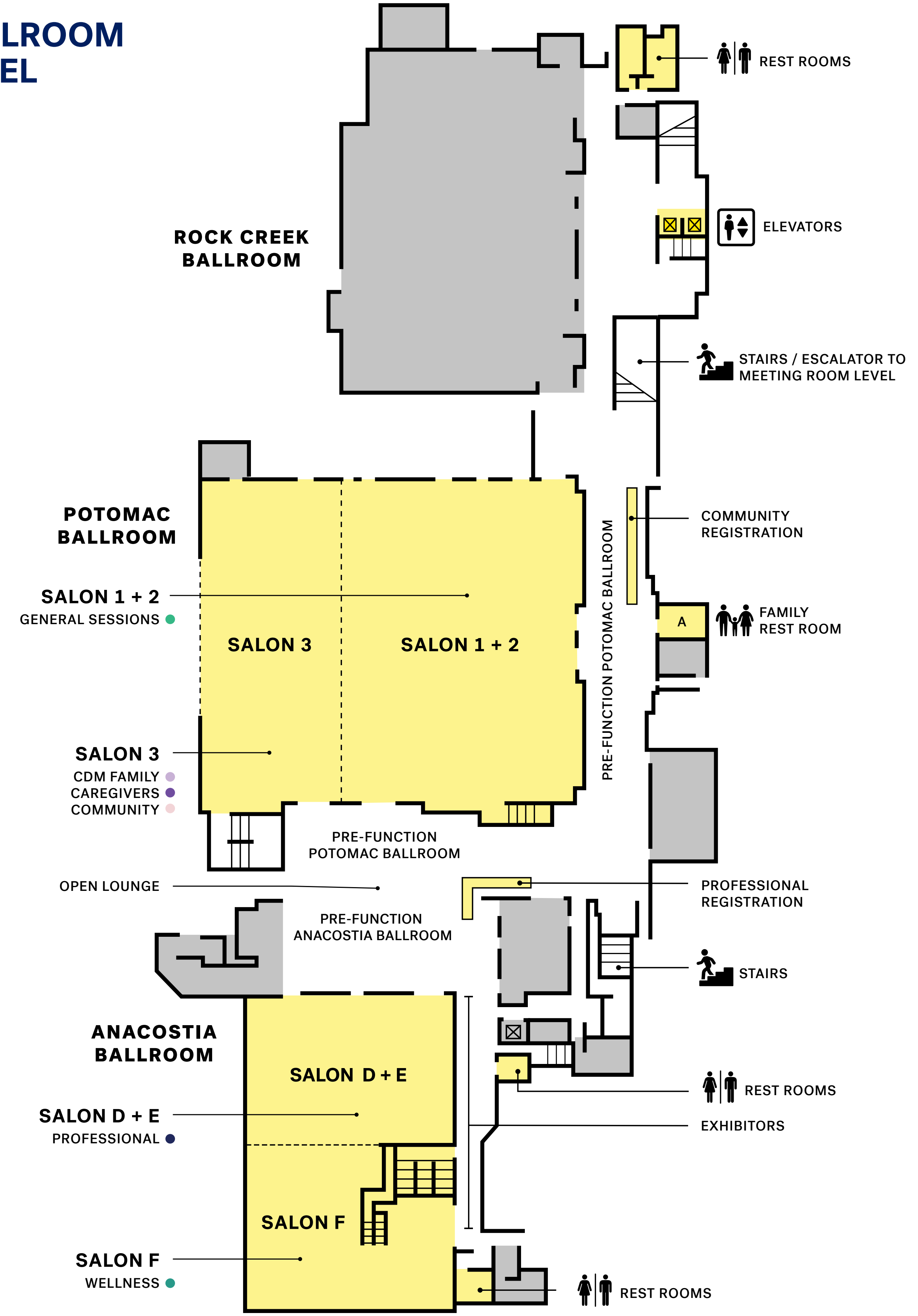
Participate in Myotonic Dystrophy Seated & Standing Pilates to develop your core through exercises and breathwork to keep you centered and balanced and to keep your heart, lungs and circulatory system healthy.

admin@pneumaeverydaywellness.org

pneumaeverydaywellness.org/

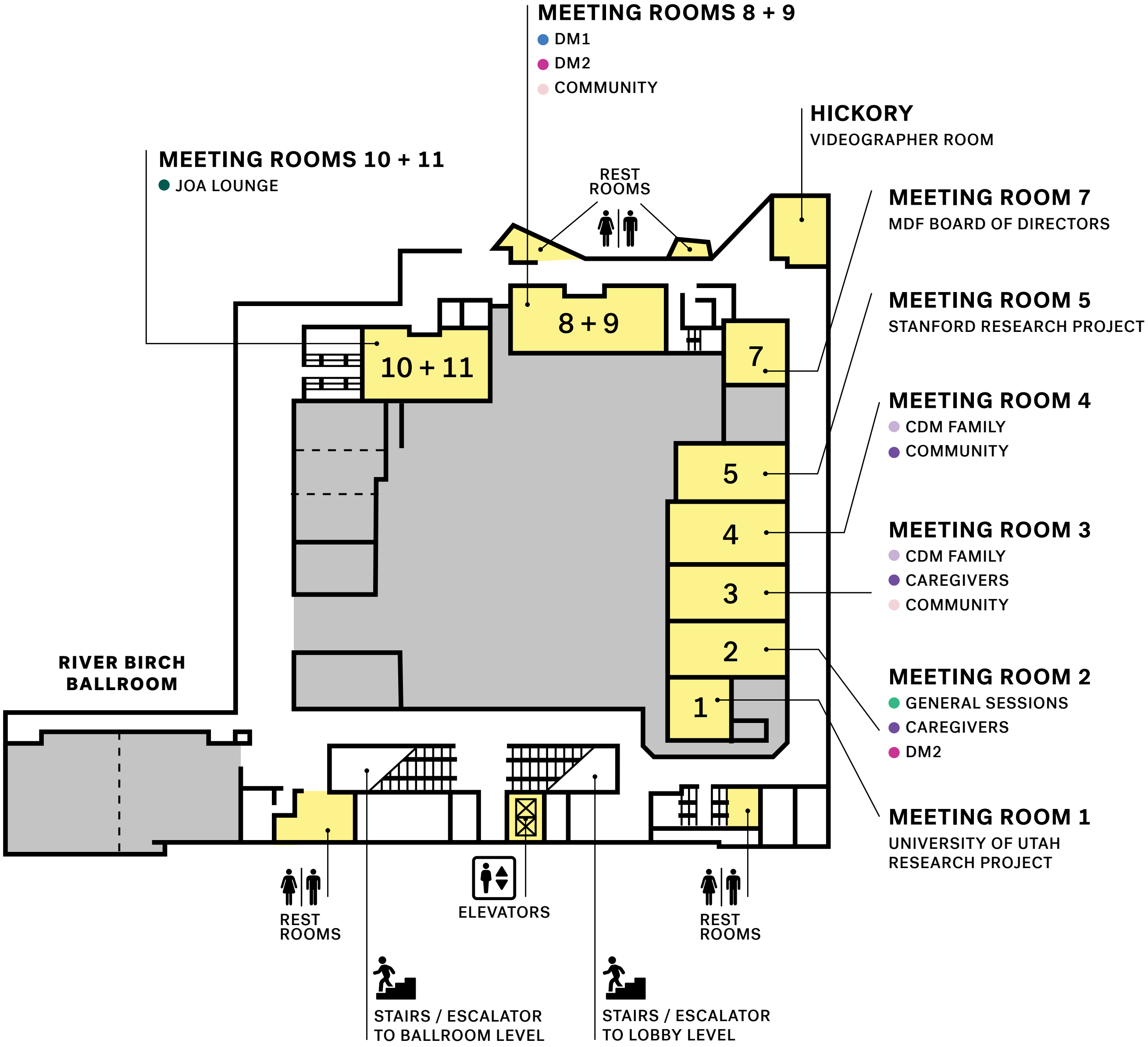
MDF Conference Area Map | Ballroom Level

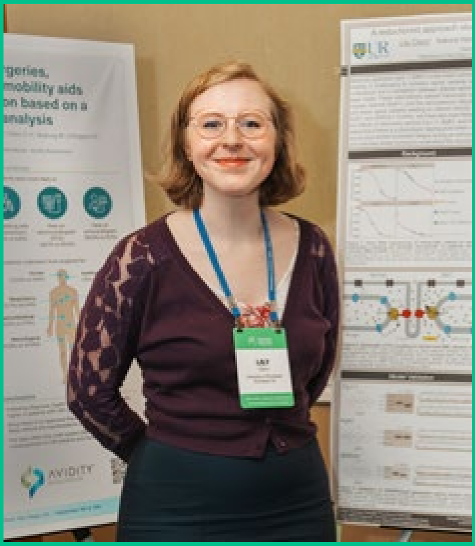
BALLROOM LEVEL



MDF Conference Area Map | Meeting Room Level

MEETING ROOM LEVEL





Myotonic
Dystrophy
FOUNDATION

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