



2022 MDF Annual Conference

Paradise Point, San Diego, CA & Virtually!
September 9th & 10th

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





Our Vision

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

Our Mission

The mission of the Myotonic Dystrophy Foundation is Community, Care, and a Cure.

We support and connect the myotonic dystrophy community.

We provide resources and advocate for care.

We accelerate research toward treatments and a cure.

Myotonic Dystrophy Foundation

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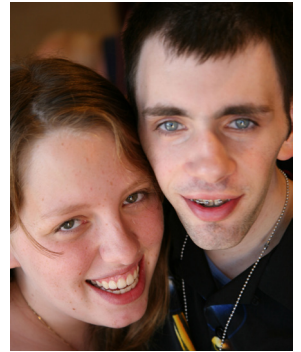
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SEPTEMBER 9TH & 10TH

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A Letter from the CEO



Welcome to the 2022 MDF Annual Conference!

On behalf of the MDF Board, staff, and countless volunteers who have helped make this year's conference possible, I am thrilled to welcome you, and over 500 others, to our first ever hybrid conference! For the fourteenth year in a row, MDF is delighted to bring together our powerful community of DM families, researchers, clinicians, industry partners, and allies. A special welcome to the over 130 people attending the conference for the very first time!

With input from hundreds of community members, we have organized an agenda to help meet the diverse needs and interests of our community, including five independent tracks: DM1, DM2, Caregiver/Self-care, JOA, and DM Professionals. General Sessions are open to everyone in all tracks and will provide updates about recent activities of the Foundation, advancements in research and research opportunities, inspirational stories, highlights of treatment advances, special awards, and more. Stump-the-Doctor sessions return this year and six different community panels are offered! New this year, and in line with MDF's new *Myotonic Dystrophy In Motion* initiative, Movement Moments will provide short movement activities for all conference attendees. We hope you will also take advantage of the many different networking opportunities built into the program to ensure you have the chance to meet, hug, and laugh with old and new friends alike!

The conference is an amazing opportunity to learn about the latest in research toward treatments and a cure for DM. Be sure to join our popular Industry Updates sessions highlighting the latest news from 10 companies working in DM drug development. Saturday afternoon, attend the "Wide World of Research" or "DM2: the Brain & the Muscle" to hear some of the latest in DM1 and DM2 research, including information about registries, biobanks, and clinical trials. Don't forget to check out the exhibitor booths hosted by our clinic and lab partners this year – some research will even be taking place onsite! We hope you will also explore the several dozen DM-focused research posters displayed around the ballroom and talk with their authors during Saturday evening's poster/exhibitor reception. You might even encounter one of MDF's 13 current Research Fellows attending this year! The entire DM ecosystem is working together toward a cure – right here at conference.

It is with deep appreciation that I thank you for taking into consideration the health and safety of our community by testing for the coronavirus prior to conference participation and taking all necessary precautions to help prevent the spread of COVID-19. We know it can be inconvenient and sometimes frustrating but we are grateful for this small sacrifice that will allow us to be together this week.

We are excited that ALL attendees have an online conference profile and may access the virtual conference platform to attend sessions online at any time. Login to mdf2022.pathable.co from the comfort of your own home, your hotel room, or one of the gorgeous outdoor spaces at the hotel! Please reach out to the MDF team if you need anything. I look forward to meeting you all and hope you truly enjoy the next few days.

Tanya Stevenson, EdD, MPH
Chief Executive Officer

REMINDER: Please allow extra time at check-in for a mandatory COVID-19 rapid test.

Agenda for Thursday, September 8, 2022

Time*	Session		
11:00 AM - 4:00 PM	Avidity Biosciences Tour & Lunch <i>Avidity Labs</i>	Early Arrival Coffee Bar & Networking <i>Paradise Foyer</i>	Conference Registration <i>Paradise Foyer</i>
4:00 PM - 6:00 PM	Break		
6:00 PM - 8:00 PM	Early Arrival Reception <i>Paradise Terrace</i>		

Agenda for Friday, September 9, 2022

Time*	DM1 Track <i>Paradise Ballroom</i>	DM2 Track <i>Dockside Room</i>	Caregivers & Self-care Track <i>Bay View Room</i>	JOA Track <i>Executive Rooms 705-707</i>	Professional Track <i>Garden Room</i>
8:00 AM – 8:50 AM	Professional Track Breakfast <i>Garden Room</i>				
9:00 AM- 9:50 AM	First Timers’ Tea <i>Paradise Ballroom</i>				Considerations & Open Discussions of Exercise Impact on Clinical Trials & Everyday Health
10:00 AM- 10:50 AM	Friday Breakfast <i>Paradise Ballroom</i>				
11:00 AM- 11:45 AM	Welcome & State of the Foundation <i>Paradise Ballroom</i>				
11:45 AM- 12:00 PM	Break				
12:00 PM- 1:15 PM	Understanding DM1: Disease Development & Symptom Management	Understanding DM2: Disease Development & Symptom Management	Making Daily Life Easier with an Occupational Therapist	Get-to-Know- You-Games	Future Directions of DM Research - Part 1
1:15 PM- 1:30 PM	Break				
1:30 PM- 2:30 PM	Networking Lunch <i>Paradise Ballroom</i>			MDF Research Fellows Lunch (Private) <i>Bay View Room</i>	
2:30 PM- 3:30 PM	Industry Updates Part 1 <i>Paradise Ballroom</i>				
3:30 PM- 3:45 PM	Break				
3:45 PM- 5:00 PM	Disability Rights & Resources <i>Paradise Ballroom</i>		Physical Therapy, Mobility & Adaptive Sports	Bag-o-Stuff & Icebreaker Games	Future Directions of DM Research - Part 2
5:00 PM- 5:15 PM	Break				
5:15 PM- 6:15 PM	DM1 Networking	DM2 Networking	Caregiver Networking	Pizza Party & Movie Night	DM Professionals Networking
	Parents & Guardians Networking <i>Executive Suite 701-703</i>				
6:15 PM- 6:30 PM	Break				
6:30 PM- 8:30 PM	Welcome Reception <i>Paradise Ballroom</i>			MDF Scientific Advisory Committee Meeting (Private) <i>Bay View Room</i>	

REMINDER: Please allow extra time at check-in for a mandatory COVID-19 rapid test.

Agenda for Saturday, September 10, 2022

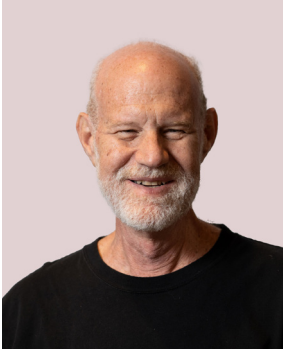
Time*	DM1 Track <i>Paradise Ballroom</i>	DM2 Track <i>Dockside Room</i>	Caregivers & Self-care Track <i>Bay View Room</i>	JOA Track <i>Executive Rooms 705-707</i>	Professional Track <i>Garden Room</i>	
8:00 AM- 8:50 AM	Professional Track Breakfast					
9:00 AM- 9:50 AM	Saturday Breakfast <i>Paradise Ballroom</i>				Research Dataset Presentations	
10:00 AM- 10:50 AM	Stories of Inspiration from the DM Community <i>Paradise Ballroom</i>					
11:00 AM- 12:15 PM	Community Panels: Navigating Life with DM	DM1	Male Caregivers <i>Garden Room</i>	Grief (Affected) <i>Sunset I</i>	Talking About Emotions	Attend Open Sessions & Informal Networking
		DM2	Female Caregivers <i>Bay View Room</i>	Grief (Unaffected) <i>Sunset II</i>		
12:15 PM- 12:30 PM	Snack Break					
12:30 PM- 1:45 PM	The Wide World of Research & Securing Funding to Move Us Closer to A Cure	DM2: The Brain & the Muscle	The Wide World of Research & Securing Funding to Move Us Closer to A Cure	Games & Activities	Attend Open Sessions & Informal Networking	
1:45 PM- 2:00 PM	Break					
2:00 PM- 3:00 PM	Networking Lunch <i>Paradise Ballroom</i>					
3:00 PM- 4:00 PM	Industry Updates Part 2 <i>Paradise Ballroom</i>					
4:00 PM- 4:15 PM	Break					
4:15 PM- 5:30 PM	Stump the Doctor: DM1	Stump the Doctor: DM2	Meaningful Communication and DM: A Panel Discussion	Stump the Doctor: JOA	Break & Informal Networking	
5:30 PM- 6:30 PM	Exhibitor and Research Poster Showcase & Reception <i>Paradise Foyer & Porte Cochere</i>					
6:30 PM- 8:30 PM	Closing Dinner & Dance Party! <i>Paradise Ballroom</i>					

Agenda for Sunday, September 11, 2022

Time*	Special Event for CDM Families!
9:00 AM- 11:00 AM	Congenital Myotonic Dystrophy (CDM) Family Networking Breakfast Sponsored by AMO Pharma. <i>Executive Suite 713-715</i>

***All times Pacific Daylight Time (Pacific).**
For virtual attendees, the online platform, *Pathable*, should adjust the
online program agenda to read in local time zones.

Joining Virtually



Join the Conference Virtually at mdf2022.pathable.co
Use the checklist below for the best virtual conference experience possible!

Suggested setup:

- ☐ **Google Chrome** or **Microsoft Edge** web browser
- ☐ Laptop or desktop computer
- ☐ At least 1.5 mbps internet connection (check your internet speed: <https://www.speedtest.net/> or <https://fast.com/>)
- ☐ Webcam, microphone + headphones or speakers
- ☐ Zoom video conferencing app (you don't need an account but do need Zoom downloaded)
 - Download Zoom <https://zoom.us/>
 - Camera and lighting tips <https://support.pathable.com/hc/en-us/articles/360055411231-Presentation-Pro-Tips-and-Best-Practices>



If the above options aren't available, check out these alternatives:

- ☐ Smartphone or tablet
- ☐ Other internet browsers (Safari, Firefox, etc.)
- ☐ Slower internet speeds - we suggest keeping your camera turned off if your internet is less than 1.5 mbps



Extra credit setup:

- ☐ Build your personal conference agenda:
 - In the conference website, go to **Agenda > Full Agenda**
 - Select the + next to sessions you want to attend to add them to **My Agenda**
 - Review your schedule under **Agenda > My Agenda**
- ☐ Get to know attendees, speakers, sponsors and exhibitors
 - View everyone attending the conference under the **Attendees** tab
 - Learn more about individuals by clicking their name and reading their profile
 - Schedule video meetings, or send messages to get in touch with other attendees!



At the Conference:

- ☐ When it's time, join sessions you have selected by going to **Agenda > My Agenda** and clicking **Join Live Meeting**
- ☐ Chat with others in the chat box on the right side of any session page
- ☐ Take notes during sessions by clicking **Notes** on any session page (download these later by exporting from the **Agenda** page)
- ☐ Visit the exhibitor and sponsor booths by visiting **Exhibitors > Exhibitors by Type**
 - Read about exhibitors and sponsors just as you would a session



Enjoy the Conference!

Conference Tracks

DM1 Track - Paradise Ballroom

This track was designed with community input from individuals living with DM1.

- **Understanding DM1: Disease Development & Symptom Management**

Chamindra Konersman, MD

Learn from a leading expert about the causes and genetics underlying DM1, how and when in life it can affect different systems of the body, tips to help self-manage symptoms, and best practices for working with a care team to ensure the best quality of life. Learn too, about the latest research in DM1 and how you can get involved.

- **DM1 Networking**

Join a facilitated conversation for individuals living with DM1.

- **Navigating Life with DM1**

Mindy Kim, Bill Nuttall, Jeannine DeSoi, Dean Sage

Individuals living with DM1 share stories and ideas for navigating daily life. Professionals are welcome to attend community panels and are asked to take seats only if space allows and refrain from making comments or posing questions during the open discussion as this time is for individuals with DM1 to share.

- **Grief Support Group for Individuals Living with DM**
Jane Mello-Reynolds, LCSW

Individuals living with DM – types 1 and 2 – are invited to take part in a bereavement support group that explores coping with the loss of a loved one while facing their own challenges living with the condition. The facilitator's hope is to provide a safe and confidential environment to express concerns, fears, and emotions. Whether you share memories of struggles and challenges, laugh at anecdotal stories, or cry over your losses, you will be provided a secure venue to find other individuals with shared experiences to connect. This session is only available in-person and will not be recorded. *This session takes place in Executive Suite 707.*

- **Stump the Doctor: DM1**

Charles Thornton, MD

Meet a leading expert in myotonic dystrophy type 1 and ask your most persistent, challenging questions. Can you stump the doctor? This session will be live-streamed to accommodate in-person and virtual attendees, but it will not be recorded due to privacy considerations for attendees whose questions may be personal.



Celebrate myotonic dystrophy families and raise awareness for community, care, and a cure on September 15th! Wear green, attend MDF's LIVE webinar advocacy event, and post on social media using #myotonicDystrophy and @myotonicstrong.

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

Together, we can change the future of myotonic dystrophy!

Get involved and join the movement at <https://www.myotonic.org/international-dm-day>

Conference Tracks

DM2 Track - Dockside Room

This track was designed with community input from individuals living with DM2.

- **Understanding DM2: Disease Development & Symptom Management**

Thurman Wheeler, MD

Learn from a leading expert about the causes and genetics underlying DM2, how and when in life it can affect different systems of the body, and how best to self-manage and work with a care team to ensure the best quality of life. Learn, too, about the latest research in DM2 and how you can get involved. This session is open to individuals with DM2 and their families.

- **DM2 Networking**

Join a facilitated conversation for individuals living with DM2.

- **Navigating Life with DM2**

Tom McPeck, Lorrie Gallagher, E. Khalifa, Elyse Posner

Individuals living with DM2 share stories and ideas for navigating daily life, and welcome caregivers and families to attend and participate. Professionals are welcome to attend community panels and are asked to take seats only if space allows and refrain from making comments or posing questions during the open discussion as this time is for individuals with DM2 to share.

- **Grief Support Group for Individuals Living with DM**
Jane Mello-Reynolds, LCSW

Individuals living with DM – types 1 and 2 – are invited to take part in a bereavement support group that explores coping with the loss of a loved one while facing their own challenges living with the condition. The facilitator's hope is to provide a safe and confidential environment to express concerns, fears, and emotions. Whether you share memories of struggles and challenges, laugh at anecdotal stories, or cry over your losses, you will be provided a secure venue to find other individuals with shared experiences to connect. This session is only available in-person and will not be recorded. *This session takes place in Executive Suite 707.*

- **DM2: The Brain & the Muscle**

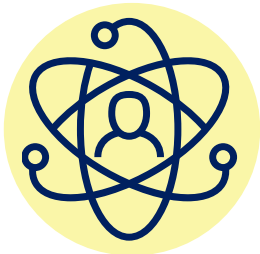
Araya Puwanant, MD

This session will start with a short review of the CNS manifestations in DM2 followed by a discussion of pilot data in everyday terms from a recent DM2 brain imaging and cognitive study. It will also include a discussion of how brain abnormalities could potentially affect motor function.

- **Stump the Doctor: DM2**

John Day, MD, PhD

Meet a leading expert in myotonic dystrophy type 2 and ask your most persistent, challenging questions. Can you stump the doctor? This session will be live-streamed to accommodate in-person and virtual attendees, but it will not be recorded due to privacy considerations for attendees whose questions may be personal.



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 from anywhere at [mdf2022.pathable.co](https://www.mdf2022.pathable.co)

Free Childcare at the 2022 MDF Annual Conference

Playtime Pros provide dedicated childcare Friday and Saturday from 9 AM to 7 PM each day in Executive Suites 709 & 711. Space is limited and pre-registration with the vendor is required. Register your child at <https://www.childrenspath.net/playtime-pros-registration> or call (619) 309-8032 by Sept. 6th.



Conference Tracks

Caregivers & Self-care Track - Bay View Room

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

- **Making Daily Life Easier with an Occupational Therapist**
Samar Muslemeni, OT, MSc

A leading clinician and researcher in occupational therapy (OT) will define this form of healthcare and advise on how it can be used by individuals with DM1 and DM2 and their families. She will also discuss the ways that adaptation, task alteration, devices, or environmental changes can help individuals with DM, both children and adults, make their daily life easier. She will specifically address common issues and solutions in different activities such as moving, toileting, and eating for adults and children.

- **Physical Therapy, Mobility & Adaptive Sports**
Melissa McIntyre, DPT

Join a leading physical therapist whose clinical care and research focus centers on individuals with neuromuscular disorders. In this session, we will discuss the basics of physical therapy (PT) and what it can do for DM populations – both type 1 and type 2, adult and pediatric. Additionally, this session will review adaptive sports and novel adaptive equipment.

- **Caregiver Networking**

Join a facilitated conversation for DM caregivers, including family or other caregivers who do not have DM.

- **Navigating Life as an Unaffected Male Caregiver**
John Cooley, JD; Ted Salwin; John Fitzpatrick

Hear from a panel of spouses and other male caregivers, and share your own experiences. Men only, please. Professionals are welcome to attend community panels and are asked to take seats only if space allows and refrain from making comments or posing questions during the open discussion as this time is for unaffected male caregivers to share.

DM caregivers are not alone. Meet online at MDF's Facebook group anytime at www.facebook.com/groups/myotonicdystrophycaregivers/ or contact the monthly virtual support group facilitator Ted Salwin for meeting details: tedsalwin@hotmail.com.

- **Navigating Life as an Unaffected Female Caregiver**
Suzanne Perkins, PhD; Kim McPeck;

Emily Jones; Julie LeBoeuf

Hear from a panel of spouses and other female caregivers, and share your own experiences. Women only, please. Professionals are welcome to attend community panels and are asked to take seats only if space allows and refrain from making comments or posing questions during the open discussion as this time is for unaffected female caregivers to share.

- **Meaningful Communication and DM:**
A Panel Discussion

Missy Dixon, PhD; Belen Esparis, MD; and
Lori Planco, BSBA

One of the most challenging interpersonal aspects of a DM diagnosis is communicating about it to other people. To communicate well, we must identify what we wish to accomplish (change, alter, or enhance) in our interpersonal interactions with ourselves and others before we begin to talk, write, type, or text about DM. Learning different ways of communicating may help you feel understood by the people in your life, and may help you to successfully self-advocate or advocate for others. This panel session will focus on understanding what we want to accomplish in our different relationships when we communicate about DM, potential barriers to communication, and choosing appropriate and effective communication strategies to foster meaningful conversations about DM with others.



Myotonic Dystrophy Family Registry

If you have been affected by myotonic dystrophy as a patient or caregiver you can join the Myotonic Dystrophy Family Registry. Our goal is to use the basic data collected here in the registry to amplify the voice of those affected by myotonic dystrophy, no matter where they are in the world. Every contribution creates a more complete picture of the DM community.

Learn more here:

<https://myotonicregistry.patientcrossroads.org/>



Conference Tracks

JOA Track - Executive Suites 701 & 703 aka the JOA Warriors Lounge

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.

- **Getting to Know You**

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

- **Bag-o-Stuff & Icebreaker Games**

We will work in teams to create an original skit from a bag of different objects. Bring all the creativity you can muster for this session!

- **Pizza Party & Movie Night**

Join us in the lounge for pizza and a movie! We will be watching "Inside Out," the Pixar Disney animated classic that critics have hailed as a great exploration of the main characters' struggle to deal with overwhelming emotions.

- **Let's Talk About Feelings**

Missy Dixon, PhD

Missy Dixon, PhD, will stop by to talk with the group about how feelings affect our bodies and minds. She will also lead an interactive discussion about strategies for learning to manage overwhelming emotions.

- **Yoga & Games**

Ellen Shapiro, RYT-500

Join community favorite Ellen Shapiro for a fun, guided yoga session. No yoga experience or equipment is necessary. After the 20-minute yoga session, get ready to play games, talk, or relax in the lounge.

- **Stump the Doctor: JOA**

Jacinda Sampson, MD

Meet a leading expert in DM and ask your most persistent, challenging questions. Can you stump the doctor? This session will be live-streamed to accommodate in-person and virtual attendees, but it will not be recorded due to privacy considerations for attendees whose questions may be personal. This session is only open to JOA community members.

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 kwwasw@yahoo.com.



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 2022 MDF Annual Conference.

Conference Tracks

Professionals Track - Garden Room

This track was designed with expert guidance from members of MDF's Scientific Advisory Committee. Attendees must have a professional registration.

- **Professional Track Breakfast**

Make your way to the meeting room and prepare for the Professional Track with breakfast and coffee. Only available for Professional Track registrants.

- **Considerations & Open Discussions of Exercise Impact on Clinical Trials & Everyday Health**

Thomas A. Cooper, MD;

Mark Tarnopolsky, MD, PhD, FRCP(C);

Elise Duchesne, PhD; Andrew Berglund, PhD;

Tina Duong MPT, PhD

The Professional Track begins with a session on DM and exercise. Thomas A. Cooper, MD, presents, "Endurance exercise leads to beneficial molecular and physiological effects in the HSALR mouse model of myotonic dystrophy type 1". Mark Tarnopolsky, MD, PhD, FRCP(C) presents "Exercise and nutritional strategies for myotonic dystrophy". Elise Duchesne, PhD, presents "Strength training effectively alleviates skeletal muscle impairments in myotonic dystrophy type 1". Andrew Berglund, PhD, presents "Analysis of individual transcriptomic response to strength training for myotonic dystrophy type 1 patients reveals rescue at the molecular level". The session will end with a presentation by Tina Duong MPT, PhD.

- **Future Directions of DM Research - Part 1**

John Lueck, PhD; Andrew Berglund, PhD

First in our lineup of presentations on future directions in DM research, John Lueck, PhD, presents "Generation of Bi-channelopathy DM Mice and Drug Repurposing". Then, Dr. Berglund returns to the podium to present on screening and natural products.

- **Future Directions of DM Research - Part 2**

Laura Ranum, PhD; Eric Wang, PhD

Opening the afternoon sessions on Future Directions of DM Research is Laura Ranum, PhD, presenting "A BAC transgenic mouse model for DM2". Afterward, Eric Wang, PhD, will ask the question: "is gene therapy in the cards for myotonic dystrophy?"

- **DM Professionals Networking**

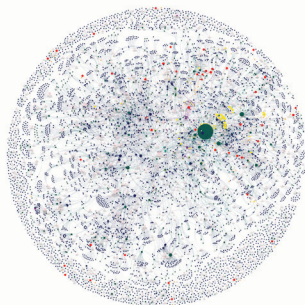
A networking event for clinicians, scientists, and other DM professionals. Attendees must have a professional registration.

- **Research Dataset Presentations**

Suzanne McDermott, PhD; Nicholas Johnson, MD; Jeffrey Statland, MD

Suzanne McDermott, PhD, presents, "Groups of Signs and Symptoms in adult-onset myotonic dystrophy type 1". Nicholas Johnson, MD, and Jeffrey Statland, MD, present "The Myotonic Dystrophy Clinical Research Network (DMCRN): observational studies as the foundation for clinical trials".

MDF invites all DM Professionals to attend Saturday's general & community sessions as guests and listeners. Please understand that these sessions primarily serve individuals living with DM & their families, so please respect their access to seating and space, and refrain from comments and questions during Q&A.



Discover the Myotonic Dystrophy Research Map

MDF is proud to serve as the nexus of care, research and drug development within the myotonic dystrophy community.

To increase collaboration and general knowledge across the DM ecosystem, MDF has compiled publicly available research-related information on myotonic dystrophy into a single comprehensive database. To capture the interconnected nature of progress in the DM research ecosystem, we have illustrated the data in a visual network map – an intuitive, holistic, and explorable knowledge-building tool built for professionals and community members alike.

Start exploring at: <https://www.myotonic.org/myotonic-dystrophy-research-map>

Conference Tracks

General Sessions

General sessions are for everyone!

- **Avidity Biosciences Lab Tour**

Take a tour of the new Avidity Biosciences facilities. Due to limited capacity, this activity requires a separate sign up before the Conference. Attendees will gather at 11am for lunch in the Dockside Room, then board the mini-coach to the lab for a one-hour tour. Two groups will depart separately.

- **Early Arrival Coffee Bar & Networking**

Join the MDF Board of Directors and other early arrivals for coffee and conversation *in the Bay View Room*.

- **Early Arrival Reception**

Join us on the eve of the MDF Annual Conference for hors d'oeuvres, one drink ticket, and no host bar reception for early arrivals.

- **First Timers' Tea**

For those who have never attended a MDF Conference before, we invite you to join this special orientation and networking event to introduce you to the Conference, MDF, and the DM community. This first-timer event is designed to help improve your Conference experience.

- **Friday Breakfast**

Join friends, family, and other attendees for breakfast!

- **Welcome & State of the Foundation**

MDF leadership and Board of Directors are eager to share exciting updates about the state of the Foundation, International Myotonic Dystrophy Awareness Day, and the MDF strategic plan.

- **Networking Lunch**

Get to know your fellow attendees over lunch.

- **Industry Updates Part 1**

Representatives from biotech and pharma companies will provide updates on their drug development efforts in the DM field. Presenters include Avidity Biosciences, Dyne Therapeutics, Harmony Biosciences, GrittGene Therapeutics, and AMO Pharma.

- **Disability Rights & Resources**

- Christopher Knauf, Esq**

Many individuals living with DM – types 1 and 2 – and their families may find themselves in situations where they need to know their rights, especially rights related to employment, education, public accommodation, genetic counseling, and healthcare. This workshop will provide an overview of key laws such as the ADA, IDEA, ACA, GINA, which may protect you or your family. It will cover some common situations when these laws can be used and address processes for activating those rights, including when to consider engaging a lawyer or other advocate. Learn, too, about organizations, agencies, and other resources to help empower yourself and your family. This session is open to individuals living with any form of DM and their families.

- **Welcome Reception**

Celebrate the end of a wonderful Day 1 at the MDF Annual Conference with hors d'oeuvres, one drink ticket, and no host bar. *There will be no evening meal provided on Friday.*

- **Saturday Breakfast**

Join friends, family, and other attendees for breakfast!

- **Stories of Inspiration from the DM Community**

A celebration of the DM community, including the 4th Annual Kayla Vittek Memorial Award for Outstanding Community Advocate presented by Lisa Harvey-Duren, as well as the celebration of the Above and Beyond Award and Lifetime Achievement Award Recipients.

- **Networking Lunch**

Get to know your fellow attendees over lunch.

- **Industry Updates Part 2**

Representatives from biotech and pharma companies will provide updates on their drug development efforts in the DM field. Presenters include Juevena Therapeutics, PepGen, ARTHEX Biotech, Entrada Therapeutics, and NeuBase Therapeutics.

- **Research Poster and Exhibitor Showcase & Reception**

Enjoy a drink and hors d'oeuvres while you discover the DM Research Posters and Exhibitors Showcased at the MDF Annual Conference. Talk with the research poster authors or review most posters online at mdf2022.pathable.co/research-poster-showcase.

- **Closing Dinner & Dance Party!**

Join us for a dance party! Our DJ is playing everyone's favorite dance numbers so we can see your cool moves!

- **Congenital Myotonic Dystrophy (CDM) Family Networking Breakfast, Sponsored by AMO Pharma**

All families with children with CDM are invited to *Executive Suite 713-715* to network, chat, and enjoy breakfast together.

MDF is pleased to provide the following meals:

- Thursday evening reception (*hors d'oeuvres & beverages*)
- Friday breakfast
- Friday lunch
- Friday evening reception (*hors d'oeuvres & beverages*)
- Saturday breakfast
- Saturday snack break
- Saturday lunch
- Saturday Exhibitor/Poster reception (*hors d'oeuvres & beverages*)
- Saturday dinner



Movement Moments



For the first time at the MDF Annual Conference, and in line with MDF's new initiative Myotonic Dystrophy In Motion, Movement Moments will provide short movement demonstrations for all conference attendees, each lasting a few minutes. The hope with Movement Moments 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 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 the 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.

Leslie Krongold, EdD, who lives with DM1, is a longtime leader and advocate in the DM exercise movement. Leslie and Lorrie Gallagher, who lives with DM2, are both passionate about movement. Along with instructor Natalie Graniela, Leslie and Lorrie will demonstrate the **GYROKINESIS®** exercise method, a movement practice based on slow and fluid movements that can help with balance. **GYROTONIC®** and **GYROKINESIS®** are registered trademarks of Gyrotonic Sales Corp and are used with their permission.

Friday Afternoon Movement Moment: ENDURANCE EXERCISE

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. Mindy Kim, a tap dancer and teacher who lives with DM1, will show off some of her skills and illustrate how we can groove without getting out of our chairs by doing a tap dance warmup routine.



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.

Saturday Afternoon Movement Moment: STRENGTH

Resistive exercises are activities that make your muscles contract to increase their strength and endurance.

Luke Bolt is a competitive bicyclist who lives with DM1. He helped start the Myotonic Dystrophy In Motion initiative. Watch or follow along his easy exercise band routine.



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, PhD, PT; Kate Eichinger, PhD, PT, DPT, NCS; Lorrie Gallagher; Mindy Kim; Leslie Krongold, EdD; and Ellen Shapiro.

Conference Speakers



Tetsuo "Tee" Ashizawa , MD
Director of Neuroscience Research,
Houston Methodist Neurological
Institute
Scientific Advisory Committee
Stories of Inspiration



Jeannine DeSoi
MDF Support Group Facilitator
Navigating Life with DM1



Andrew Berglund, PhD
Professor, Director, RNA Institute,
Department of Biological Sciences,
University of Albany
Scientific Advisory Committee
Impacts of Exercise on DM - Part 2
Future Directions of DM Research - Part 1



Missy Dixon, PhD
Visiting Assistant Professor, Program Director,
Utah Program for Inherited Neuromuscular
Disorders, Dept. of Pediatrics,
University of Utah
Facilitated talk about emotions (JOA);
Having Difficult Conversations about DM



Luke Bolt
MDF Community Member
MDF Movement Committee
Stories of Inspiration



Elise Duchesne, PhD
Professor & director of physiotherapy
education unit, Universite Du Quebec
Impacts of Exercise on DM - Part 2



Kevin Brennan
Principal, Bluebird Strategies
MDF Advocacy Consultant
The Wide World of Research & Securing
Funding to Move Us Closer to a Cure



Tina Duong, PT, PhD
Physical Therapist | Clinical Research
Manager, Stanford University
MDF Movement Committee
Impacts of Exercise on DM - Part 2



John Cooley, J.D.
MDF Community Member
MDF Support Group Facilitator
Navigating Life as a Male Caregiver



Belen Esparis, MD
Medical Director for Sleep Disorders
Center, University of Pennsylvania
MDF Board of Directors
MDF National Advocacy Committee
Having Difficult Conversations about DM



John Fitzpatrick
MDF Board of Directors
Navigating Life as a Male Caregiver



Thomas Cooper, MD
S. Donald Greenberg and R. Clarence
& Irene H. Fulbright Professor,
Baylor College of Medicine
Scientific Advisory Committee
Impact of Exercise on DM1 - Part 1



Lorrie Gallagher
MDF Community Member
MDF Movement Committee
Navigating Life with DM2
Movement Moments



John Day, MD, PhD
Professor of Neurology, Pediatrics &
Pathology, Stanford University School
of Medicine
MDF Board of Directors
Scientific Advisory Committee
Stump the Doctor - DM2



Nicholas Johnson, MD
Associate Professor and Vice Chair
of Research in Neurology, Virginia
Commonwealth University
Scientific Advisory Committee
Research Dataset Presentations
DMCRN

Conference Speakers (continued)



Emily Jones
MDF Community Member
MDF Support Group Facilitator
MDF National Advocacy Committee
Navigating Life as a Female Caregiver
Stories of Inspiration



John Lueck, PhD
Assistant Professor, Department of
Pharmacology and Physiology, University
of Rochester Medical Center
Generation of Bi-channelopathy DM Mice
and Drug Repurposing



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



Suzanne McDermott, PhD
Professor, City University of New York
School of Public Health
Groups of Signs and Symptoms in adult-onset myotonic dystrophy type 1



E. Khalifa
MDF Community Member
Navigating Life with DM2



Melissa McIntyre, DPT
University Of Utah
Crafting a Personal Exercise Program with a Physical Therapist



Mindy Kim
MDF Support Group Facilitator
MDF Movement Committee
Navigating Life with DM1



Thomas McPeck
MDF Board of Directors
MDF Support Group Facilitator
Navigating Life with DM2



Christopher Knauf, JD
Director of Litigation, Disability Rights Legal
Center
Disability Rights & Resources



Kim McPeck
MDF Community Member
MDF Support Group Facilitator
Navigating Life as a Female Caregiver



Chamindra Konersman, MD
Associate Professor of Neurosciences,
University of California, San Diego
Understanding DM1: Disease
Development & Symptom Management



Jane Mello-Reynolds, LCSW
Oncology Social Worker, UCSD Moores
Cancer Center
Grief Support Group for Individuals with DM



Julie LeBoeuf
MDF Community Member
MDF Support Group Facilitator
Navigating Life as a Female Caregiver



Darren Monckton, PhD
Professor, University of Glasgow
Scientific Advisory Committee
Stories of Inspiration



Larry Lord
Founding Member of MDF
MDF Community Member
Stories of Inspiration



Samar Muslemani, OT
PhD Student & Occupational Therapist,
Universite De Sherbrooke
Making Daily Life Easier with an Occupational Therapist

Conference Speakers (continued)



Bill Nuttall
MDF Support Group Facilitator
Navigating Life with DM1



Suzanne Perkins, PhD
MDF Community Member
MDF Support Group Facilitator
Navigating Life as a Woman Caregiver



Lori Planco
MDF Community Member
Having Difficult Conversations about DM



Allan Posner
MDF Community Member
Navigating Life as a Male Caregiver



Elyse Posner
MDF Community Member
Navigating Life with DM2



Araya Puwanant, MD
Assistant Professor in Neurology,
Wake Forest University
DM2: The Brain & The Muscle



Laura Ranum, PhD
Professor of Genetics/Research Director
Center for NeuroGenetics,
University Of Florida
Scientific Advisory Committee
Future Directions of DM Research - Part 2



Dean Sage
MDF Community Member
Navigating Life with DM1



Ted Salwin
MDF Community Member
MDF Support Group Facilitator
Navigating Life as a Male Caregiver



Ellen Shapiro, RYT - 500
Yoga Instructor
MDF Community Member
MDF Movement Committee
*JOA Movement Session
Movement Moments*



Jacinda Sampson, MD, PhD
Clinical Associate Professor in Neurology,
Stanford Neuroscience Health Center
Scientific Advisory Committee
*The Wide World of Research & Securing
Funding to Move Us Closer to a Cure,
Stump the Doctor - JOA*



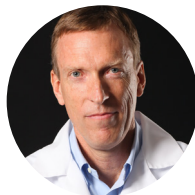
Jeffrey Statland, MD
Neurologist, University of Kansas
Medical Center
Research Dataset Presentations



Tanya Stevenson, EdD, MPH
Chief Executive Officer,
Myotonic Dystrophy Foundation
Welcome & State of the Foundation



Mark Tarnopolsky, MD, PhD, FRCP(C)
Professor of Pediatrics and Medicine
CEO and CSO, Exerkine Corporation,
McMaster University Medical Center
Impacts of Exercise on DM - Part 1



Charles Thornton, MD
Professor of Neurology, University of Rochester
MDF Board of Directors
Scientific Advisory Committee Chair
*Welcome & State of the Foundation
Stump the Doctor - DM1
DMCRN*



Debbie Wais, LCSW
Social Worker, UCSD Medical Center
*Grief Support Group for Unaffected
Caregivers*



Eric Wang, PhD
Assistant Professor of Molecular Genetics
and Microbiology, University Of Florida
Scientific Advisory Committee
MDF National Advocacy Committee
Future Directions of DM Research - Part 2



Thurman Wheeler, MD
Assistant Professor, Massachusetts
General Hospital
*Understanding DM2: Disease
Development & Symptom Management*

Industry Update Speakers

In speaking order:

DAY 1



Dr. William Jacobson, BCMA
Senior Director, Clinical Development
Harmony Biosciences
*Meet Harmony Biosciences & Learn
about our Clinical Trial in Type 1
Myotonic Dystrophy*
www.harmonybiosciences.com



Li Tai, MD, PhD
Executive Director, Clinical Development
Avidity Biosciences
An Update from Avidity Biosciences
www.aviditybiosciences.com



Ash Dugar, PhD, MBA
Senior Vice President,
Global Head of Medical Affairs
Dyne Therapeutics
*DYNE-101: Moving from Bench
to Clinic to Deliver a Potentially
Transformative Therapy for DM1*
www.dyne-tx.com



Dr. Gor Sarkisyan
Co-Founder and CSO
GrittGene Therapeutics
*Multifaceted Approach,
Uncompromised Results,
Precision Treatment*
www.grittgene.com



Mike Snape, PhD
Chief Scientific Officer
AMO Pharma Ltd.
*An Update on the REACH CDM and
REACH CDM X Studies of AMO-02 in
Children and Adolescents*
www.amo-pharma.com

DAY 2



Jane Larkindale, DPhil
Vice President, Clinical Science
PepGen
*Unlocking the Potential of
Oligonucleotide Therapeutic
Candidates for Myotonic Dystrophy
through Enhanced Delivery*
www.pepgen.com



Hanadi Yousef, PhD
CEO and Co-Founder
Juvena Therapeutics, Inc.
*Human Pluripotent Stem Cell-derived
Protein Therapy that Promotes Muscle
Regeneration for Treating DM1*
www.juvenatherapeutics.com



Michael S. Oldham, MD, MPH
Executive Medical Director
Entrada Therapeutics
*Introducing Entrada & Our Approach to
Treating Myotonic Dystrophy Type 1*
www.entradatx.com



Dietrich A. Stephan, PhD
Chairman & CEO
NeuBase Therapeutics
An Update from NeuBase Therapeutics
www.neubasetherapeutics.com



Judy Walker, MD, FRCP
Chief Medical Officer
ARTHEx Biotech
*Arthex' ATX-01: A Novel Approach
to Myotonic Dystrophy*
www.arthexbiotech.com

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



Tips to Help Maximize Your Conference Experience

- 1. Divide and conquer.** If you are attending the Conference with multiple family members or friends, consider attending different sessions when multiple sessions occur at the same time.
- 2. Use the breaks.** Feedback from last year suggests that attendees wanted more time between sessions, and more frequent breaks, so there is a lot of room between sessions. Use these breaks to stretch, check out other Conference activities, connect with a new friend, or just rest. Need a break but don't want to miss a session? View the session online at mdf2022.pathable.co
- 3. Take notes.** Sessions are jam-packed with information, and it would be impossible for anyone to memorize everything that's discussed at the Conference. Take notes so you can revisit things again after the Conference.
- 4. Don't worry - almost everything is being recorded.** All sessions except for the discussion portion of the community panels and the Stump-the-Doctor sessions, will be recorded and posted to MDF's Digital Academy after the Conference. So if you miss a session, or you have to choose one session over another that looks equally interesting, don't worry - you will see it soon!



Presentation Abstracts Available Online!

For the first time ever, 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.

<https://www.myotonic.org/professional-session-abstracts-2022-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.

<https://www.myotonic.org/research-posters-2022-mdf-annual-conference>



Exhibitor and Sponsor Showcase

Visit our amazing exhibitors during the conference! Tables are set up in hallways around the conference area.



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**DM Research Map,
Developed by MDF**
Research Opportunity



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Meet MDF
Learn about MDF



All conference registrants can visit
VIRTUAL exhibitor booths online by visiting
MDF2022.Pathable.Co, then selecting Exhibitor.



**Myotonic Dystrophy
Family Registry**
Research Opportunity



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Continue Learning With MDF



MDF Digital Academy



View hours of educational and inspirational videos by DM experts wherever and 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!

<https://www.myotonic.org/digital-academy>

Ask-the-Expert



MDF's 2021-2022 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: <https://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 MDRF enables MDF and others to review community-reported statistics and data about their experience of living with DM to aid advocacy and education efforts. Learn more and register here: <https://myotonicregistry.patientcrossroads.org/index.php>

Meet the DM Drug Developer



Throughout 2021 and 2022, 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:

<https://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 publications, toolkits, and clinical care recommendations that guide health care

providers and families in the care and management of DM. Publications include clinical care guidelines for DM1, DM2, congenital DM, anesthesia, cardiology, and more. View the full range of toolkits and publications here:

<https://www.myotonic.org/toolkits-publications>

Support Programs



Due to the variable nature of DM, one can sometimes feel isolated, frustrated, and confused. Many families benefit from connection and community with others who share their experiences. MDF facilitates local support groups, virtual meetings for special populations, and even social media communities to ensure that no one affected by DM ever feels alone. Check out MDF's support programs here:

<https://www.myotonic.org/find-support>

MDF Research Fellows

Introducing the 2021 and 2022 MDF Pre- and Postdoctoral Research Fellows! Trainees applied for support for projects focused on basic, translational, or clinical research in myotonic dystrophy. This program not only supports meritorious research, but also helps continue to build a critical mass of researchers engaged in working on Myotonic Dystrophy. To learn more about our MDF Research Fellows go to <https://www.myotonic.org/myotonic-fellows-grant-recipients>. Be sure to visit the Fellows' posters at the conference during Showcase hours to talk with them live about their research! View all the research posters online at mdf2022.pathable.co.

2022



Lily Cisco
University of Rochester
Medical Center



Avery Engelbrecht
University of Florida



Jesus Frias
The RNA Institute



Christina Heil, PhD
University of Rochester
Medical Center



Preeti Kumari, PhD
Massachusetts
General Hospital



Larissa Nitschke, PhD
Baylor College
of Medicine



Zoe Scherzer
University of Florida

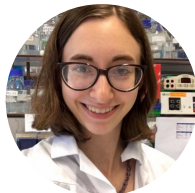


Xiaomeng Xing
University of
Nottingham

2021



Kamyra Simone Edokpolor
Emory University
School of Medicine



Maya Gosztyla
UC San Diego



Benjamin M. Kidd
University of Florida



Rong-Chi Hu
Baylor College of Medicine



Subodh Kumar Mishra, PhD
The RNA Institute,
University of Albany

MDF Support Group Facilitators

The Myotonic Dystrophy Foundation is the world's largest myotonic dystrophy (DM) patient advocacy organization, connecting people living with DM in 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 at www.myotonic.org/find-support.



Rob Besecker, MBA
Chicago, IL, USA



John Cooley
Kansas City Area, USA



Mark Coplin
Portland, OR, USA



Teresa Cummings
Arizona, USA



Jeannine DeSoi
Boston, MA, USA



Loraine Dressler
Orange County, CA, USA



Sarah Francheteau-Berman
Parents/Guardians
of Children with DM



Jonathan Freedman
Seattle, WA, USA



Patricia Gibson
Kansas City Region, USA



Kay Hayes
Denver, CO, USA



Cindy Hubert
WA State Region, USA



Chuck Hunt
Atlanta, GA, USA



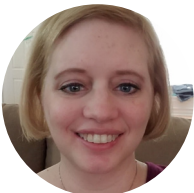
Janis Jaffe
New York City Area



Emily Jones
Rochester, NY, USA



Rashid Kassir
San Diego, CA USA



Mindy Kim
North Carolina, USA &
Adult Facebook Chat



Anke Klein
International
German Speakers



Leslie Krongold, EdD
DM1 50+ & Healthier
Living Discussion Group



Carrie Lahnovych
Rochester, NY



Julie LeBoeuf
Canadian
Support Group



Alexandra LeBoeuf
Canadian Support
Group



Kristen McClintock
Florida, USA



Kim McPeck
DM2 Caregivers
Support Group



Tom McPeck
DM2 Virtual
Support Group



Sherry Morris
Dallas, TX, USA

Support Group Facilitators (continued)



Bill Nuttall
Adult Facebook Chat



Suzanne Perkins
Michigan, USA



Bernhard Rogg
International
German Speakers



Ted Salwin
Indianapolis, IN, USA



Carolyn Valek
Worthington, OH, USA
USA & Juvenile-onset
Adult (JOA) Warriors



Patrick Welker
Dallas, TX USA



Susan "Glenda" Winson
New York City Area



Ann Woodbury
Utah & Mountain West
Region, USA



Guillermo Zubillaga
New York City Area

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:

<https://www.myotonic.org/scientific-advisory-committee>.



Tetsuo Ashizawa, MD
Houston Methodist
Neurological Institute



Guillaume Bassez, MD, PhD
Institut de Myologie



Andy Berglund, PhD
RNA Institute
University of 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, FAANA
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

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 <https://www.myotonic.org/board-directors>.



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Board Chair & Lifetime Trustee



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Board Member



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Charles Thornton, MD
Board Member

MDF Staff & Consultants

To learn more about MDF staff go to <https://www.myotonic.org/staff>.



Kate Beck
Special Projects
Manager



Kleed Cumming
Communications &
Technology Manager



Allison Formal, MBA
Senior Science Advisor



Mike Knaapen
Program Director



Mounica Lakshmi Dugginapeddi, MPH
Community Programs Coordinator



Nadine Skinner, PhD, MPA
Research Coordinator



Tanya Stevenson, EdD, MPH
Chief Executive Officer

MDF is growing! Find out how you can become part of the team as a staff member or volunteer at www.myotonic.org/volunteer-career-opportunities-mdf

Thank You to Our Sponsors

Visit their online booths at mdf2022.pathable.co

DIAMOND



PLATINUM



GOLD



SILVER



BRONZE



Our Global Alliance

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: <https://www.myotonic.org/international-dm-day>



Myotonic Dystrophy

IN MOTION

www.myotonic.org

This is a new MDF care initiative to unify, inspire, and encourage the international DM community to start moving!

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



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



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 here: <https://www.myotonic.org/news-and-events/newsletter-archive>

Support MDF at the Community Raffle!



Be sure to buy your raffle tickets for a chance to win a vacation in Mammoth Lakes, CA, an iPad, jewelry, and *more!*

Visit the MDF Info booth for more details!



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



New Medicines. Better Lives.

www.amo-pharma.com

Stanford Myotonic Dystrophy Biobank

The Stanford Myotonic Dystrophy Biobank is established to coordinate collection, preservation, and sharing of samples with the international research community. If you are interested in learning more, please contact the team at:

StanfordBiobank@lists.stanford.edu

Phone: (650) 497-9807



Dr. John W. Day, MD, PhD.
Professor of Neurology,
Pediatrics and Pathology



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

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

We're proud to support the 2022 MDF Annual Conference.

Learn more at vrtx.com

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Lupin Neurosciences is committed to improving the lives of people affected by underserved neurological disorders who are often neglected.

Our mission is to expand patient access to science-based therapies and solutions through building a global portfolio of speciality pharmaceuticals for people living with rare conditions.

Lupin Neurosciences is a Specialty Pharma Division of Lupin Atlantis Holdings SA
EU-LUP-2207-00001 July 2022
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OUR MISSION:

Empowering Oligonucleotide Therapeutics

PepGen is advancing the next generation of oligonucleotide therapeutics with the goal of transforming the treatment of severe neuromuscular and neurologic diseases.

OUR CORE VALUES



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RESPONSIBILITY

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www.PepGen.com
community@pepgen.com

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- **Cure Driven**
 - DM2 treatment and cure is our goal
- **Collaborative Platform**
 - Working with scientists, clinicians and field experts to develop DM2 cure
- **Modern Technologies**
 - Regenerative Medicine, Gene Therapies, Small Molecule Pharmacology, AI and Computational Biology, and a personal touch

MYOTONIC DYSTROPHY CLINICAL RESEARCH NETWORK

RESEARCH STUDIES OPEN TO ACCRUAL

- **ESTABLISHING
BIOMARKERS AND
CLINICAL ENDPOINTS IN
MYOTONIC DYSTROPHY
TYPE 1: END-DM1**
- **ASSESSING PEDIATRIC
ENDPOINTS IN DM1:
ASPIRE-DM1**

CONTACT:

**JESSICA ST. ROMAIN,
NETWORK MANAGER**

jessica.stromain@vcuhealth.org

**RUBY LANGESLAY,
NETWORK COORDINATOR**

ruby.langeslay@vcuhealth.org

SITES

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UNIVERSITY OF ROCHESTER

UNIVERSITY OF FLORIDA

HOUSTON METHODIST

THE OHIO STATE UNIVERSITY

UNIVERSITY OF KANSAS

UNIVERSITY OF IOWA

UNIVERSITY OF COLORADO, DENVER

UNIVERSITY OF CALIFORNIA, LOS ANGELES

STANFORD UNIVERSITY

EUROPE

CENTRO CLINICO NEMO

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to patients with severe RNA-mediated disease

Proud sponsors of the
2022 Myotonic Dystrophy Foundation Meeting

neubase

Thank you to the entire myotonic
dystrophy community for your help and
encouragement as we move toward our
planned clinical trials in 2023!

SLEEP IN RARE

REAL-WORLD EVIDENCE ACCELERATOR



JUSTICE RICKENBACH
COMMUNITY AMBASSADOR

This evidence accelerator aims to deepen our understanding of the degree to which sleep disorders impact people living with rare disease. If you or someone you care for in the myotonic dystrophy community is impacted by excessive daytime sleepiness and fatigue, we invite you to take a brief survey. **You will receive a \$10 Amazon gift card for your participation.**

For more information visit:
trend.community/news/sleep-in-rare-evidence-accelerator or email us at evidence@trend.community



MYOTONIC DYSTROPHY FOUNDATION GALA 2022

THURSDAY, OCTOBER 20TH, 2022
SONY HALL
NEW YORK, NEW YORK

COME BE
A PART OF IT

[WWW.MYOTONIC.ORG/GALA](https://www.myotonic.org/gala)



We thank Harmony Biosciences and Avadel for amplifying the voices of people living with rare disease by co-sponsoring this initiative.

Thank you to all of our generous sponsors!
Visit exhibitors online at mdf2022.pathable.co.



The muscle to *keep life moving*[™]

Dyne Therapeutics is a clinical-stage muscle disease company focused on advancing innovative life-transforming therapeutics for people living with genetically driven diseases.

Please join us at the conference on September 9th and 10th, visit our booth and listen to our SVP, Global Medical Affairs Ash Dugar, Ph.D. provide an update on our Clinical Trial of DYNE-101.

We are proud to sponsor the 2022 Myotonic Dystrophy Foundation Annual Conference

www.Dyne-tx.com

We approach rare diseases differently.

At Avidity Biosciences, we're developing a brand-new class of therapies to find treatments for people with rare muscle diseases, including myotonic dystrophy type 1 (DM1). Our commitment to innovative science is matched only by our passion to change people's lives for the better.

We've used the latest advances in science, medicine, and engineering to create a promising new way to deliver powerful therapeutics precisely to their targets. AOC 1001 is being developed to target the root cause of DM1. We are currently evaluating AOC 1001 in the MARINA™ and MARINA-OLE™ clinical trials.

Karin,
DM1
advocate

CLINICAL TRIALS


MARINA™

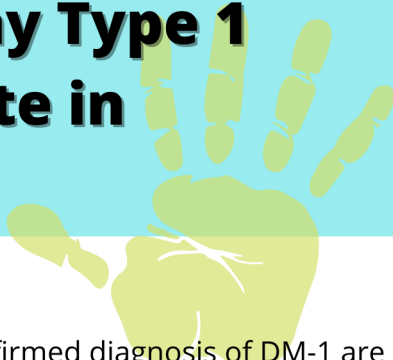
MARINA-OLE™



AVIDITY®
BIOSCIENCES

Connect with us
Patients@Aviditybio.com

Looking for individuals with Myotonic Dystrophy Type 1 (DM-1) to participate in research study




WHO?

Individuals ages 3 years and up with a confirmed diagnosis of DM-1 are invited to participate in this 1 year research study with the Stanford Neuromuscular Team.

WHAT?

This study will assess several new methods of measuring handgrip myotonia and gait parameters in DM1. The new devices include a motion capture glove, an ankle/wrist monitor, and a high speed 3D video platform to capture movement. This is a Clinical Trials readiness study with the long-term aims to incorporate these outcomes into clinical trials of DM1 therapies.

HOW?



Interested individuals should contact research coordinator Christina Frater at cfrater@stanford.edu.

For general information about participant rights, contact 1-866-680-2906.

**Look for us in the
Porte Cochere Room at the Conference**

**Stanford
University**

ADVERTISEMENT

Over 76% of people living with type 1 myotonic dystrophy indicate they are impacted by impaired sleep or daytime sleepiness.

“Long before you ever get to the muscle loss, you have the inability to stay focused and alert all day. The days are spent on my couch because I’m too sleepy to get out and do things.”

– Susannah, living with myotonic dystrophy

Reference: Treating sleep disorders in DM1. Myotonic Dystrophy Foundation. Accessed February 2, 2022.
<https://www.myotonic.org/treating-sleep-disorders-dm1>

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March 2022



Do you or someone you love have type 1 myotonic dystrophy?

Harmony Biosciences is currently enrolling a clinical trial for patients with type 1 myotonic dystrophy who are experiencing excessive daytime sleepiness.

Scan here to learn more about the trial



**Mapping Secreted Proteins
to Unmet Medical Needs**

We are biopharma company unlocking the therapeutic potential of stem-cell secreted proteins and accelerating their development into life-saving therapies.



Advanced lead protein
therapeutic candidate
for Myotonic Dystrophy
Type 1 (DM1)

<https://www.juvenatherapeutics.com/>

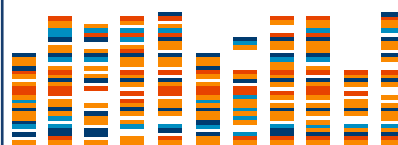
Locanabio is pleased to support the MDF and contribute to finding therapies for patients suffering from myotonic dystrophies.

“We are **excited to leverage our RNA-targeted platform, CORRECTx™, to advance a therapeutic candidate** for the treatment of myotonic dystrophy type 1, or DM1, towards the clinic.



Ranjan Batra, Ph.D.
Senior Vice President of
Research & Development

Our technology is well suited to targeting the mutant RNA allele in repeat expansion disorders such as DM1 and has the **potential to offer a one-time treatment with durable benefit.**



Learn more by visiting:
www.Locanabio.com



Video Capture for Motion Analysis

Want to see a 3D model of how you move?

Participate in a new study with Stanford Neuromuscular clinicians assessing movement patterns through digital video technologies.

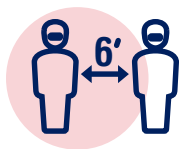
Interested? Check us out at the MDF Conference!

Stanford
University

COVID-19 Precautions



Before you can join any conference activities, collect a COVID-19 rapid test from the table at the hotel check-in. You will be given a free rapid test to take to your room to confirm that you are negative for COVID-19. Rapid tests take approximately 15-30 minutes to complete.



Please budget time to take your test before checking in at the conference registration table or attending any conference activities. Please be current on vaccines, wear a mask, wash your hands, and take all other precautions recommended by the CDC, State of California, and local authorities.

Paradise Point Map



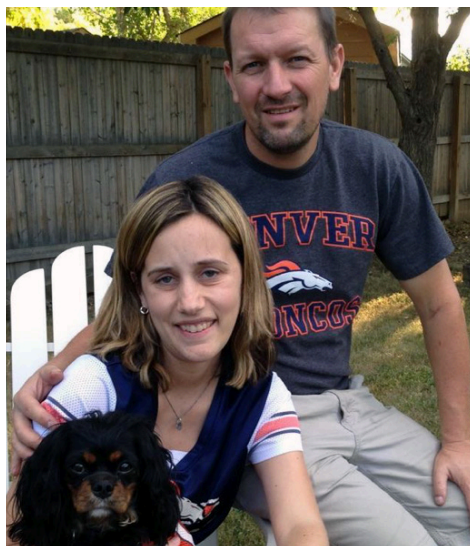
Paradise Point MDF Conference Area Map



Enjoy the Fresh Air at Paradise Point



Paradise Point Resort has many outdoor spaces that in-person attendees can enjoy. WiFi is available in all guest rooms, outdoor common areas such as the pool and beaches, and restaurants and outlets. During the conference, attendees are welcome to take their meals outside. Tables and chairs will be available for attendee use anytime in the Porte Cochere area and on the Bayview and Dockside decks. The Early Arrival Reception Thursday will take place at the Paradise Terrace, and the Exhibitor & Research Poster Showcase will also take place outdoors. We invite you to utilize outdoor spaces as your health and comfort allow. If you want to watch a conference session from an outdoor space or your hotel room, visit mdf2022.pathable.co



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