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.
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MDF Annual Conference
SEPTEMBER 9TH & 10TH

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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
### Agenda for Thursday, September 8, 2022

<table>
<thead>
<tr>
<th>Time*</th>
<th>Session</th>
<th>Location</th>
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</table>
| 11:00 AM – 4:00 PM | Avidity Biosciences Tour & Lunch  
Avidity Labs | Early Arrival Coffee Bar & Networking  
Paradise Foyer | Conference Registration  
Paradise Foyer |
| 4:00 PM – 6:00 PM | Break | |
| 6:00 PM – 8:00 PM | Early Arrival Reception  
Paradise Terrace | |

### Agenda for Friday, September 9, 2022

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

REMINDER: Please allow extra time at check-in for a mandatory COVID-19 rapid test.
### Agenda for Saturday, September 10, 2022

<table>
<thead>
<tr>
<th>Time*</th>
<th>DM1 Track</th>
<th>DM2 Track</th>
<th>Caregivers &amp; Self-care Track</th>
<th>JOA Track</th>
<th>Professional Track</th>
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<tbody>
<tr>
<td></td>
<td>Paradise Ballroom</td>
<td>Dockside Room</td>
<td>Bay View Room</td>
<td>Executive Rooms 705-707</td>
<td>Garden Room</td>
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<tr>
<td>8:00 AM-8:50 AM</td>
<td>Professional Track Breakfast</td>
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<tr>
<td>9:00 AM-9:50 AM</td>
<td>Saturday Breakfast</td>
<td>Research Dataset Presentations</td>
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<tr>
<td>10:00 AM-10:50 AM</td>
<td>Stories of Inspiration from the DM Community</td>
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<td>11:00 AM-12:15 PM</td>
<td>Community Panels: Navigating Life with DM</td>
<td>DM1: Male Caregivers</td>
<td>Grief (Affected)</td>
<td>Talking About Emotions</td>
<td>Attend Open Sessions &amp; Informal Networking</td>
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<td></td>
<td></td>
<td>Garden Room</td>
<td>Sunset I</td>
<td></td>
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<tr>
<td></td>
<td></td>
<td>DM2: Female Caregivers</td>
<td>Grief (Unaffected)</td>
<td>Sunset II</td>
<td></td>
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<tr>
<td>12:15 PM-12:30 PM</td>
<td>Snack Break</td>
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<tr>
<td>12:30 PM-1:45 PM</td>
<td>The Wide World of Research &amp; Securing Funding to Move Us Closer to A Cure</td>
<td>DM2: The Brain &amp; the Muscle</td>
<td>The Wide World of Research &amp; Securing Funding to Move Us Closer to A Cure</td>
<td>Games &amp; Activities</td>
<td>Attend Open Sessions &amp; Informal Networking</td>
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<tr>
<td>1:45 PM-2:00 PM</td>
<td>Break</td>
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<tr>
<td>2:00 PM-3:00 PM</td>
<td>Networking Lunch</td>
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<tr>
<td>3:00 PM-4:00 PM</td>
<td>Industry Updates Part 2</td>
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<tr>
<td>4:00 PM-4:15 PM</td>
<td>Break</td>
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<tr>
<td>5:30 PM-6:30 PM</td>
<td>Exhibitor and Research Poster Showcase &amp; Reception</td>
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<td>6:30 PM-8:30 PM</td>
<td>Closing Dinner &amp; Dance Party!</td>
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### Agenda for Sunday, September 11, 2022

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

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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](https://www.myotonic.org/international-dm-day)
DM2 Track - Dockside Room

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

- **Understanding DM2: Disease Development & Symptom Management**
  Thuman 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 McPeek, 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

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 Muslemani, 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.

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

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**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](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](https://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
Monday 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.

Monday 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.

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

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<thead>
<tr>
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<th>Title and Affiliation</th>
<th>Talks and Topics</th>
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<tr>
<td>Tetsuo “Tee” Ashizawa, MD</td>
<td>Director of Neuroscience Research, Houston Methodist Neurological Institute</td>
<td>Stories of Inspiration</td>
</tr>
<tr>
<td>Andrew Berglund, PhD</td>
<td>Professor, Director, RNA Institute, Department of Biological Sciences, University of Albany</td>
<td>Impacts of Exercise on DM - Part 2, Future Directions of DM Research - Part 1</td>
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<tr>
<td>Luke Bolt</td>
<td>MDF Community Member, MDF Movement Committee</td>
<td>Stories of Inspiration</td>
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<tr>
<td>Kevin Brennan</td>
<td>Principal, Bluebird Strategies, MDF Advocacy Consultant</td>
<td>The Wide World of Research &amp; Securing Funding to Move Us Closer to a Cure</td>
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<tr>
<td>John Cooley, J.D.</td>
<td>MDF Community Member, MDF Support Group Facilitator</td>
<td>Navigating Life as a Male Caregiver</td>
</tr>
<tr>
<td>Thomas Cooper, MD</td>
<td>S. Donald Greenberg and R. Clarence &amp; Irene H. Fulbright Professor, Baylor College of Medicine</td>
<td>Impact of Exercise on DM1 – Part 1</td>
</tr>
<tr>
<td>John Day, MD, PhD</td>
<td>Professor of Neurology, Pediatrics &amp; Pathology, Stanford University School of Medicine</td>
<td>Stump the Doctor - DM2</td>
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<tr>
<td>Jeannine DeSoi</td>
<td>MDF Support Group Facilitator</td>
<td>Navigating Life with DM1</td>
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<tr>
<td>Missy Dixon, PhD</td>
<td>Visiting Assistant Professor, Program Director, Utah Program for Inherited Neuromuscular Disorders, Dept. of Pediatrics, University Of Utah</td>
<td>Facilitated talk about emotions (JOA); Having Difficult Conversations about DM</td>
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<tr>
<td>Elise Duchesne, PhD</td>
<td>Professor &amp; director of physiotherapy education unit, Universite Du Quebec</td>
<td>Impacts of Exercise on DM - Part 2</td>
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<tr>
<td>Tina Duong, PT, PhD</td>
<td>Physical Therapist</td>
<td>Impacts of Exercise on DM - Part 2</td>
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<tr>
<td>Belen Esparis, MD</td>
<td>Medical Director for Sleep Disorders Center, University of Pennsylvania</td>
<td>Having Difficult Conversations about DM</td>
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<tr>
<td>John Fitzpatrick</td>
<td>MDF Board of Directors</td>
<td>Navigating Life as a Male Caregiver</td>
</tr>
<tr>
<td>Lorrie Gallagher</td>
<td>MDF Community Member, MDF Movement Committee</td>
<td>Navigating Life with DM2, Movement Moments</td>
</tr>
<tr>
<td>Nicholas Johnson, MD</td>
<td>Associate Professor and Vice Chair of Research in Neurology, Virginia Commonwealth University</td>
<td>Research Dataset Presentations, DMCRN</td>
</tr>
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</table>

2022 MDF Annual Conference
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*

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

E. Khalifa  
MDF Community Member  
*Navigating Life with DM2*

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

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

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

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

Larry Lord  
Founding Member of MDF  
MDF Community Member  
*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*

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*

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

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

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

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

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

Chamindra Konersman, MD  
Associate Professor of Neurosciences, University of California, San Diego  
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MDF Support Group Facilitator  
*Navigating Life as a Female Caregiver*

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

Suzanne McDermott, PhD  
Professor, City University of New York School of Public Health  
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University Of Utah  
*Crafting a Personal Exercise Program with a Physical Therapist*

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

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

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

Hanadie 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, FRCPC
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](http://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](http://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.

---

**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.
Exhibitor and Sponsor Showcase

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

AMO Pharma
Industry sponsor

Avidity Bio
Industry sponsor

DM Research Map, Developed by MDF
Research Opportunity

DMCRN
Research Opportunity

Dyne Therapeutics
Industry sponsor

Entrada Therapeutics
Industry sponsor

Harmony Biosciences
Industry sponsor

Meet MDF
Learn about MDF

Myotonic Dystrophy Family Registry
Research Opportunity

PepGen
Industry sponsor

Rochester University
Research opportunity

Stanford University
Research opportunity

Trend Community
Survey opportunity

UCLA
Research opportunity

Virginia Commonwealth University
Research opportunity

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

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

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

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
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](http://www.myotonic.org/find-support).
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
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](https://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  
Board Member  

John W. Day, MD, PhD  
Board Member  

Belen Esparis, MD  
Board Member  

John Fitzpatrick  
Board Member  

Haley Martinelli, Esq.  
Board Member  

Thomas “Tom” McPeek  
Board Member  

Joel Revill  
Board Member  

Charles Thornton, MD  
Board Member  

MDF Staff & Consultants

To learn more about MDF staff go to [https://www.myotonic.org/staff](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](http://www.myotonic.org/volunteer-career-opportunities-mdf).
Thank You to Our Sponsors

Visit their online booths at mdf2022.pathable.co

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<tr>
<th>DIAMOND</th>
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</table>
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](https://www.myotonic.org/international-dm-day)
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.


www.amo-pharma.com

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

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

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 specialty pharmaceuticals for people living with rare conditions.
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
- RESEARCH
- INNOVATION
- INTEGRITY
- RESPONSIBILITY

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PepGen

SCIENCE WILL MAKE THE BREAKTHROUGH.
We believe science can cure every human disease.

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GRITGGENE THERAPEUTICS
CURE-DRIVEN COLLABORATIVE PLATFORM OF MODERN TECHNOLOGIES
- 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

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SITES

USA
VIRGINIA COMMONWEALTH UNIVERSITY
UNIVERSITY OF ROCHESTER
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HOUSTON METHODIST
THE OHIO STATE UNIVERSITY
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Thank you to the entire myotonic dystrophy community for your help and encouragement as we move toward our planned clinical trials in 2023!

Delivering transformative oral medications to patients with severe RNA-mediated disease

Proud sponsors of the 2022 Myotonic Dystrophy Foundation Meeting

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.

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
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.

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.

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

Learn more by visiting: www.Locanabio.com
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
Mission Bay

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