

MDF 2020 Virtual Conference

SEPTEMBER 25 & 26



PRESENTED IN PARTNERSHIP WITH



Welcome!

The Myotonic Dystrophy Foundation (MDF) is pleased to welcome you to our 2020 Virtual Conference!

This event has been designed as an educational and community-building experience specifically for individuals and families living with myotonic dystrophy (DM). Conference sessions were developed based on direct feedback from the DM community via the MDF 2020 mid-year survey, 2019 conference evaluations, support group participants, and callers to the MDF Warmline.

Our program this year includes three different conference tracks with over 20 fantastic sessions! We continue our traditions of a 1st Timers' Tea, JOA Stump-the-Doctor session, yoga, dance party, happy hour, and of course dozens of DM experts presenting on current research, care, and advocacy. We hope there is something for everyone, whether you are new to the DM community, have been with us from the beginning, or joined the MDF family only a few years ago.

The virtual format was not how any of us imagined we would be attending the annual conference this year, but I am truly inspired that hundreds of people from around the world have prioritized gathering together during such an incredibly challenging time. Throughout our 14-year history MDF has ensured access to education, community connection, resources and research – we refuse to let a global pandemic stop us from doing that now!

We hope you will take advantage of the many opportunities to connect with friends and family in the new virtual conference environment. The conference website will allow you to schedule one-to-one and group video meetings with other conference attendees, participate in live chats and polls, interact with our sponsors and exhibitors in their virtual booths, as well as engage in networking sessions throughout the event.

On behalf of MDF's Board of Directors and staff, I hope you enjoy our first virtual conference and we look forward to seeing you in person in San Diego next year.

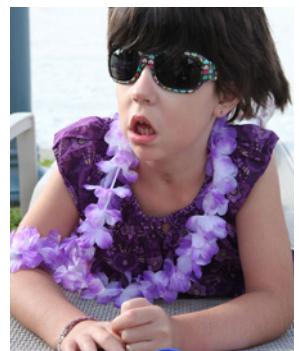
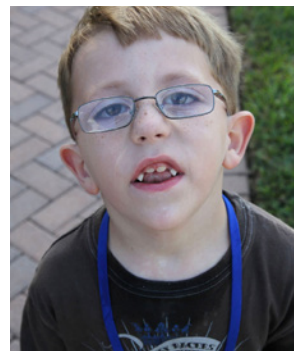
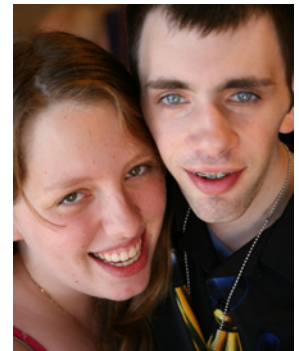
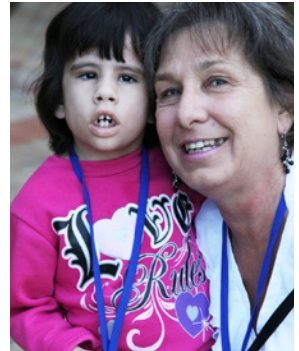
See you in the conference sessions!



Tanya Stevenson, EdD, MPH
Chief Executive Officer

**If you're not already signed up, register for the
MDF 2020 Virtual Conference here now: www.myotonic.org/conference.**

**Our mission, "Care and a Cure," is to enhance the quality of life
of people living with myotonic dystrophy (DM) and accelerate
research focused on finding treatments and a cure.**



Conference Checklist



Use the checklist below for the best 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/360049482414-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 **Schedule > Agenda**
 - Select the + next to sessions you want to attend to add them to **My Agenda**
 - Review your schedule under **Schedule > My Agenda**
- ☐ Get to know attendees, speakers, sponsors and exhibitors
 - View everyone attending the conference under the **People** 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 **Schedule > 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 and Showcase by visiting **Exhibitors > Sponsors or Exhibitors**
 - Read about exhibitors and sponsors just as you would a session
 - Talk to the hosts live during the Showcase hours: **10 AM – 12 PM** and **4 – 6 PM Pacific Time** by clicking **Enter Live Showcase Booth**

Enjoy the Conference!

Conference Tracks

Track 1: New to the DM Community

This Track is designed for recently diagnosed individuals and families seeking to understand more about DM, families who have not attended the MDF conference in the past and would like to develop a more solid foundation about the disease, as well as anyone in need of a refresher on DM. Sessions in this Track will include:

- **Myotonic Dystrophy 101: Understanding DM**
Dr. Ericka Greene (Simpson) of Houston Methodist.
- **DM Symptom Management & Treatment Options**
Dr. Laurie Gutmann of Indiana University.
- **Most Commonly Asked Questions about DM**
Dr. Jacinda Sampson of Stanford University will be interviewed by Dr. Lois Oppenheim of Montclair State University.
- **Self-Care & Stress Management for Caregivers During the COVID-19 Pandemic**
Christina Irving, LCSW of the Family Caregiver Alliance's National Center on Caregiving and MDF Support Group Facilitator Ted Salwin.

Track 2: Understanding DM Research and Progress

This Track is designed to help broaden community understanding of DM research. Following some of the research breakthroughs since Hans Steinert first identified DM in 1909, this Track will explain the importance of animal models, small molecules, medication repurposing, gene editing/therapy, and more. This Track will explore where we are now on the path toward treatment and a cure, potential roadblocks to treatment, review the current DM drug development pipeline, as well as what the future of DM research may hold. Sessions in this Track include:

- **Understanding the Foundations of DM Research**
Dr. Andy Berglund of the RNA Institute.
- **Treatment & Cure: Where are We Now**
Dr. Eric Wang of the University of Florida.
- **Current Approaches to DM2 Research**
Dr. Johanna Hamel of University of Rochester, Dr. Paloma Gonzalez-Perez of the Massachusetts General Hospital, and MDF Research Fellows Dr. Raphael Benhamou of Scripps Research Institute and Dr. Jana Jenquin of University of Florida.
- **What Comes Next: Preparing for DM Clinical Trials**
Dr. Nicholas Johnson of Virginia Commonwealth University.

Track 3: Community's Choice

This Track was designed to meet community request for popular topics in very high demand, including Nutrition, Advocacy, Mental Health, and more. Sessions in this track include:

- **Nutrition Considerations for Living with DM**
Clinical Dietitian Sara Kevern of Stanford University.
- **Advocacy for DM Research and Progress**
Moderated by Kevin Brennan of Bluebird Strategies. Panelists to include Dana Richter, a Senior Policy Advisor for Senator Shelley Moore Capito (WV) and DM Community Advocates and PRMRP reviewers, Suzette Ison and Suzanne Perkins.
- **Managing Gastrointestinal (GI) Impacts of DM**
Dr. Leila Neshatian of Stanford University.
- **Staying Mentally Healthy in the Context of COVID-19 and Rare Disease**
Dr. Kathleen Bogart of Oregon State University and Dr. Katy Eichinger of University of Rochester.

Conference Agenda for Friday, September 25, 2020

Time*	Track 1: New to the DM Community	Track 2: Understanding DM Research and Progress	Track 3: Community's Choice
10:00 AM - 12:00 PM	Exhibitor & Sponsor Showcase Open <i>Representatives will be live in their booths to answer questions!</i>		
10:00 AM - 11:00 PM	First Timers' Tea		
12:00 PM - 12:50 PM	Opening Session: Welcome & State of the Foundation <i>MDF CEO and Board of Directors</i>		
1:00 PM - 1:50 PM	Myotonic Dystrophy 101: Overview of DM <i>Ericka Greene, MD, FAAN, Houston Methodist</i>	Understanding the Foundations of DM Research <i>Andrew Berglund, PhD, The RNA Institute</i>	Nutrition Considerations for Living with DM <i>Sara Keavern, RD, CDN, CNSC, Stanford Health Care</i>
2:00 PM - 2:50 PM	Chair Yoga <i>Ellen Shapiro</i>		Special JOA Session: Stump the Doctor! <i>John Day, MD, PhD, Stanford University</i>
3:00 PM - 3:50 PM	DM Symptom Management & Treatment Options <i>Laurie Gutmann, MD, Indiana University</i>	Treatment & Cure: Where Are We Now? <i>Eric Wang, PhD, University of Florida</i>	Managing the GI Impacts of DM <i>Leila Neshatian, MD, MSc, Stanford University</i>
4:00 PM - 4:45 PM	Industry Updates <i>Avidity Biosciences Ionis Pharmaceuticals Dyne Therapeutics NeuBase Therapeutics</i>		
4:45 PM - 6:00 PM	Happy Hour & Networking Session		
4:00 PM - 6:00 PM	Exhibitor & Sponsor Showcase Open <i>Representatives will be live in their booths to answer questions!</i>		

***All times Pacific Daylight Time (Pacific).**

The conference website should adjust the online program agenda to read in local time zones.

Conference Agenda for Saturday, September 26, 2020

Time*	Track 1: New to the DM Community	Track 2: Understanding DM Research and Progress	Track 3: Community's Choice
10:00 AM - 12:00 PM	Exhibitor & Sponsor Showcase Open <i>Representatives will be live in their booths to answer questions!</i>		
10:00 AM - 11:00 AM	Strength Training for DM <i>Rachel Kahn, CSCS, No Limits Collaborative</i>		
12:00 PM - 12:50 PM	Welcome to Day Two: Stories of Inspiration <i>Lisa Harvey-Duren, MDF Founding ED, Presenting the Kayla Vittek Memorial Award</i> <i>Rob Besecker, MBA</i> <i>Tanya Stevenson, EdD, MPH, Myotonic Dystrophy Foundation</i>		
1:00 PM - 1:50 PM	Most Commonly Asked Questions about DM <i>Jacinda Sampson, MD, PhD, Stanford University</i>	Current Approaches to DM2 Research <i>Johanna Hamel, MD, University of Rochester</i> <i>Jana Jenquin, PhD, University of Florida</i> <i>Raphael Benhamou, PhD, Scripps Research Institute</i> <i>Paloma Gonzalez-Perez, MD, PhD, Massachusetts General Hospital</i>	Advocacy for DM Research and Funding <i>Kevin Brennan, Bluebird Strategies</i> <i>Suzette Ison, RN, CMCN</i> <i>Dana Richter, Senator Shelley Moore Capito (WV)</i> <i>Suzanne Perkins, PhD</i>
2:00 PM - 2:50 PM	Moderated Networking Sessions DM1 - DM2 - CDM - JOA - Caregiving - Professionals		
3:00 PM - 3:50 PM	Self-Care & Stress Management for Caregivers During the COVID-19 Pandemic <i>Christina Irving, LCSW, Family Caregiver Alliance</i> <i>Ted Salwin, MDF Support Group Facilitator</i>	What Comes Next: Preparing for DM Clinical Trials <i>Nicholas Johnson, MD, Virginia Commonwealth University</i>	Staying Mentally Healthy in the Context of COVID-19 & Rare Disease <i>Kathleen Bogart, PhD, Oregon State University</i> <i>Katy Eichinger, PT, PhD, DPT, NCS, University of Rochester</i>
4:00 PM - 4:30 PM	Industry Updates <i>Syros Pharmaceuticals</i> <i>AMO Pharma</i> <i>Harmony Biosciences</i>		
4:45 PM - 6:00 PM	Dance Party!		
4:00 PM - 6:00 PM	Exhibitor & Sponsor Showcase Open <i>Representatives will be live in their booths to answer questions!</i>		

***All times Pacific Daylight Time.**

The conference website should adjust the online program agenda to read in local time zones.

Conference Session Descriptions

Friday, September 25, 2020

● **Exhibitor & Sponsor Showcase**

10:00 AM - 12:00 PM Pacific

Visit the Exhibitor & Sponsor Showcase! Learn more about our amazing sponsors and community members. Visit their virtual booths to talk with sponsors, researchers, and local organizations about their work in real time!

● **First Timers' Tea**

10:00 AM - 11:00 AM Pacific

A welcome session for first-time attendees. Learn tips for having a successful conference experience and receive a little mentorship to help you navigate the two-day event.

● **Welcome & State of the Foundation**

12:00 PM - 12:50 PM Pacific

Welcome to our first Virtual MDF Conference! Learn more about the Foundation's recent impact and current projects. Presented by MDF's CEO and Board Members.

● **Myotonic Dystrophy 101: Overview of DM**

1:00 PM - 1:50 PM Pacific

An overview of the basics of DM for the newly diagnosed and those who might appreciate a refresher on DM. This session will review the genetics of the different types of DM as well as anticipation, the many body systems affected by this disease, what is common/not, anesthesia precautions, cognitive and neuropsychiatric effects, expectations, preparedness, and more. Presented by Dr. Ericka Greene of Houston Methodist.

● **Understanding the Foundations of DM Research**

1:00 PM - 1:50 PM Pacific

In this presentation you will learn about many of the key scientific discoveries that have allowed researchers, clinicians and the biotechnology and pharmaceutical industries to develop therapeutic strategies for DM. You will learn about the identification of the mutations in the genes that cause DM1 and DM2 and how understanding the mechanism through which these mutations cause DM. You will learn how the mutations can

change over time in a person and why individuals with DM have such a wide range of symptoms. This foundation of DM knowledge has provided researchers with diverse ways to approach DM therapies and treatments. Presented by Dr. Andy Berglund of the RNA Institute. This presentation sponsored in part by Neubase Therapeutics.

● **Nutrition Considerations for Living with DM**

1:00 PM - 1:50 PM Pacific

This session will explore the basics of nutrition and optimal nutrition for myotonic dystrophy. We will discuss one of the most requested topics, dietary supplements, as well as energy optimization, weight gain/loss, and more. Presented by Clinical Dietitian Sara Kevern of Stanford University.

● **Chair Yoga**

2:00 PM - 2:50 PM Pacific

Join community favorite Ellen Shapiro for a gentle chair yoga session. We will do some breath awareness, stretching, and mindful movements. Yoga helps to calm us and build flexibility and strength. If you are experiencing anxiety during these unusual times, this might be a nice relaxing session for you! No yoga experience or equipment necessary!

● **Special JOA Session: Stump the Doctor!**

2:00 PM - 2:50 PM Pacific

The Juvenile-onset Adult Community will have their annual Stump the Doctor session with the fabulous Dr. John Day of Stanford University. All JOA community members are welcome to attend. Please bring your toughest questions about DM to stump Dr. John Day!

● **DM Symptom Management & Treatment Options**

3:00 PM - 3:50 PM Pacific

This session will explore the many symptoms of DM as well symptom management strategies/techniques. We will discuss alleviation of symptoms including fatigue and daytime sleepiness, treatments for systemic issues, quality of life, prevention, medications, and more. Presented by Dr. Laurie Gutmann of Indiana University. This presentation sponsored in part by Dyne Therapeutics.

Conference Session Descriptions (continued)

● **Treatment & Cure: Where are We Now**

3:00 PM – 3:50 PM Pacific

There are more efforts now than ever in the biotechnology and pharmaceutical industries to bring medicines to our community. This presentation will outline various approaches that are under development to treat DM. You will learn about strategies being taken and progress that has been made in the recent past. You will also learn about potential hurdles that stand in the way of delivering effective therapeutics to DM patients, and how scientists and drug developers are working to overcome these hurdles. It is an exciting time for our community, and this presentation seeks to keep everyone informed about the exciting progress being made in this area. Presented by Dr. Eric Wang of the University of Florida. This presentation sponsored in part by Ionis Pharmaceuticals.

● **Managing Gastrointestinal (GI) Impacts of DM**

3:00 PM – 3:50 PM Pacific

This session will provide an overview of gastrointestinal health as it relates to myotonic dystrophy, including diagnostic testing, treatment options, and therapies. Presented by Dr. Leila Neshatian, Gastrointestinal Director at Stanford Health Care.

● **Industry Updates (FRIDAY)**

4:00 PM – 4:45 PM Pacific

This session will include presentations by Avidity Biosciences, Ionis Pharmaceuticals, Dyne Therapeutics, and NeuBase Therapeutics. Representatives from biotech and pharma companies will provide updates on their drug development efforts in the DM field, including companies new to the MDF Annual Conference.

● **Happy Hour & Networking Session**

4:45 PM – 6:00 PM Pacific

Grab your beverage of choice to join your friends and make new ones at Happy Hour! Choose one of our themed rooms and join in fun, casual conversation with people who get it. Relax and unwind with other members of our amazing community. Happy Hour is designed to keep topics light and connections strong! Everyone is welcome. This session designed by North Carolina Support Group Facilitator, Mindy Kim.

● **Exhibitor & Sponsor Showcase**

4:00 PM – 6:00 PM Pacific

Visit the Exhibitor & Sponsor Showcase! Learn more about our amazing sponsors and community members. Visit their virtual booths to talk with sponsors, researchers, and local organizations about their work, participation opportunities and more - in real time!

Saturday, September 26, 2020

● **Exhibitor & Sponsor Showcase**

10:00 AM – 12:00 PM Pacific

Visit the Exhibitor & Sponsor Showcase! Learn more about our amazing sponsors and community members. Visit their virtual booths to talk with sponsors, researchers, and local organizations about their work, participation opportunities and more - in real time!

● **Strength Training for DM**

10:00 AM – 12:00 PM Pacific

This class will begin with a warm-up including a body scan, breath work, active-stretching, and joint/spinal mobility work. The intensity will build as we layer on full-body strength training with functional, task-oriented movements, and some balance and coordination exercises. We will wrap up with some grounding movements to calm your nervous system and send you on your way! This class can be performed seated or standing. Equipment is not required but the following equipment is optional - dumbbells (or soup cans), a resistance band (or household towel). Presented by Certified Strength and Conditioning Specialist (CSCS), Rachel Kahn.

● **Day Two Opening Session: Stories of Inspiration**

12:00 PM – 12:50 PM Pacific

Start Day 2 off with MDF and inspirational stories, including the Kayla Vittek Memorial Award and special presentation from Rob Besecker, MBA. This presentation sponsored in part by Avidity Biosciences.

● **Most Commonly Asked Questions about DM**

1:00 PM - 1:50 PM Pacific

Get answers to some of the most commonly asked questions about myotonic dystrophy. Dr. Jacinda Sampson of Stanford University will be interviewed by Dr. Lois Oppenheim of Montclair State University.

Conference Sessions (continued)

● **Current Approaches to DM2 Research**

1:00 PM - 1:50 PM Pacific

Learn about the current developments in DM2 research. Moderated by Dr. Johanna Hamel of University of Rochester. Panelists will include Dr. Paloma Gonzalez-Perez of the Massachusetts General Hospital, and MDF Research Fellows Dr. Raphael Benhamou of Scripps Research Institute and Dr. Jana Jenquin of University of Florida.

● **Advocacy for DM Research and Funding**

1:00 PM - 1:50 PM Pacific

Advocating for myotonic dystrophy research, resources, and inclusion. Through this panel discussion you will:

1. Learn about the past, present and future of MDF's research advocacy plans and how you can help increase federal funding for DM research.
2. Get an insider perspective from a congressional staff person on what works and doesn't work when you lobby Congress for increased research funding and other health care priorities.
3. Understand from a DM consumer reviewer how federal agencies like the Department of Defense PRMRP allocate research funds to award grants to fund DM research.

Moderated by Kevin Brennan of Bluebird Strategies. Panelists to include Dana Richter, a Senior Policy Advisor for Senator Shelley Moore Capito (WV) and DM Community advocates and PRMRP reviewers, Suzette Ison and Suzanne Perkins. This presentation sponsored in part by Harmony Biosciences.

● **Moderated Networking Sessions**

2:00 PM - 2:50 PM Pacific

Meet fellow community members in topic specific moderated networking sessions! Networking topics include DM1, DM2, CDM, JOA, Caregiving & Professionals.

● **Self-Care & Stress Management for Caregivers During the COVID-19 Pandemic**

3:00 PM - 3:50 PM Pacific

This session will explore the challenges caregivers face, particularly during COVID-19 pandemic, as well as best practices and realistic ways to help manage stress to support your own well-being. Caregiving resources, including those offered by MDF, will be discussed by the Family Caregiver Alliance and MDF's own Caregiving Support Group Facilitator. Presented by Christina Irving, LCSW, Client Services Director at Family Caregiver Alliance's National

Center on Caregiving and MDF Support Group Facilitator Ted Salwin.

● **What Comes Next: Preparing for DM Clinical Trials**

3:00 PM - 3:50 PM Pacific

With many potential drugs and treatments in development, this session will explore the different stages of the clinical trials process, how COVID-19 has impacted the research process as well as the FDA review/approval process, how the different therapeutic approaches might be administered, as well as how the DM community can prepare now to be ready to participate in trials in the future. Presented by Dr. Nicholas Johnson of Virginia Commonwealth University.

● **Staying Mentally Healthy in the Context of COVID-19 & Rare Disease**

3:00 PM - 3:50 PM Pacific

This session will discuss the differences in anxiety and depression among those living with rare diseases, look closely at the recent study exploring the impact of COVID-19 on the quality of life of the DM community, as well as identify successful coping and resilience strategies found among people with rare disease, especially during times of isolation and social distance. Presented by Dr. Kathleen Bogart of Oregon State University and Dr. Katy Eichinger of University of Rochester.

● **Industry Updates (SATURDAY)**

4:00 PM - 4:30 PM Pacific

This session will include presentations by Syros Pharmaceuticals, AMO Pharma, and Harmony Biosciences. Representatives from biotech and pharma companies will provide updates on their drug development efforts in the DM field, including companies new to the MDF Annual Conference.

● **Dance Party!**

4:45 PM - 6:00 PM Pacific

Join us for closing remarks and a virtual dance party! Get your dancing shoes ready to groove to your favorite songs or join the Disco Room, the Rumba Room, and more! Dance Party designed by Northern California Support Group Facilitator, Dr. Leslie Krongold.

● **Exhibitor & Sponsor Showcase**

4:00 PM - 6:00 PM Pacific

Visit the Exhibitor & Sponsor Showcase! Learn more about our amazing sponsors and community members. Visit their virtual booths to talk with sponsors, researchers, and local organizations about their work, participation opportunities and more - in real time!

Conference Speakers



Raphael Benhamou, PhD
Scripps Research Institute
Current Approaches to DM2 Research



Andrew Berglund, PhD
Director & Professor of Biological Sciences
The RNA Institute
Understanding the Foundations of DM Research



David Berman, MBA
Board Member
Myotonic Dystrophy Foundation
Welcome & State of the Foundation



Rob Besecker, MBA
Illinois Support Group Facilitator
Welcome to Day 2: Stories of Inspiration



Kathleen Bogart, PhD
Director of the Disability and Social Interaction Lab and Associate Professor
Oregon State University
Staying Mentally Healthy in the Context of COVID-19 & Rare Disease



Kevin Brennan
Bluebird Strategies
Advocacy for DM Research and Funding



John Day, MD, PhD
Professor of Neurology, Pediatrics and Pathology
Stanford University
Special JOA Session: Stump the Doctor!



Katy Eichinger, PT, PhD, DPT NCS
Physical Therapist & Assistant Professor
University of Rochester
Staying Mentally Healthy in the Context of COVID-19 & Rare Disease



Paloma Gonzalez-Perez, MD, PhD
Director of Muscle Disorder Clinic
Massachusetts General Hospital
Current Approaches to DM2 Research



Ericka Greene, MD, FAAN
Division Head, Neuromuscular Medicine
Houston Methodist
Myotonic Dystrophy 101: Overview of DM



Laurie Gutmann, MD
Professor and Chair of Neurology
Indiana University
DM Symptom Management & Treatment Options



Johanna Hamel, MD
Assistant Professor of Neurology, Pathology and Laboratory Medicine
University of Rochester
Current Approaches to DM2 Research



Lisa Harvey-Duren
Founding Executive Director
Myotonic Dystrophy Foundation
Welcome to Day 2



Christina Irving, LCSW
Client Services Director
Family Caregiver Alliance
Self-Care & Stress Management for Caregivers During the COVID-19 Pandemic



Suzette Ison, RN, CMCN
Indianapolis Support Group Facilitator
Advocacy for DM Research and Funding

Conference Speakers (continued)



Jana Jenquin, PhD
University of Florida
***Current Approaches to
DM2 Research***



Suzanne Perkins, PhD
Michigan Support Group Facilitator
***Advocacy for DM Research
and Funding***



Dr. Nicholas Johnson, MD
Virginia Commonwealth University
***What Comes Next: Preparing
for DM Clinical Trials***



Dana Richter
Senior Policy Advisor to Senator
Shelley Moore Capito (WV)
***Advocacy for DM Research
and Funding***



Rachel Kahn
Nationally Certified Pilates Teacher
No Limits Collaborative
Strength Training and DM



Ted Salwin
Caregiving Support Group Facilitator
***Self-Care & Stress Management
for Caregivers During the
COVID-19 Pandemic***



Jeremy Kelly
Board Chair & Lifetime Trustee
Myotonic Dystrophy Foundation
Welcome & State of the Foundation



Jacinda Sampson, MD, PhD
Clinical Associate Professor
Stanford University
***Most Commonly Asked
Questions about DM***



Sara Kevern, RD, CDN, CNSC
Nutrition Support Specialist
Stanford Health Care
***Nutrition Considerations
for Living with DM***



Ellen Shapiro
Certified Yoga Teacher
Chair Yoga



Martha Montag-Brown
Board Member
Myotonic Dystrophy Foundation
Welcome & State of the Foundation



Tanya Stevenson, EdD, MPH
Chief Executive Officer
Myotonic Dystrophy Foundation
***Welcome & State of the Foundation
Welcome to Day 2***



Leila Neshatian, MD, MSc
Gastrointestinal Director
Stanford University
Managing the GI Impacts of DM



Eric Wang, PhD
Assistant Professor in the Dept. of
Molecular Genetics & Microbiology
University of Florida
Treatment & Cure: Where Are We Now?


Lois Oppenheim, PhD
Professor and Chair of Modern
Languages and Literatures
Montclair State University
***Most Commonly Asked
Questions about DM***


Learn more about each of the speakers on the conference
website when you log in on or after September 21st.


Register here now if you haven't already: www.myotonic.org/conference

Sponsor Showcase


Check out our Conference Sponsor's Virtual Showcase booths any time by clicking on the menu **Exhibitors>Sponsors** and then click on their logos. Interact live with representatives of these companies during the Conference from **10AM to 12PM and 4PM to 6PM Pacific both days**.


 **AMO Pharma** will be on hand to discuss results seen in the clinical studies to date and future clinical studies for DM. **Live in the booth: Ibraheem Mahmood, Chief Executive Officer; Joseph Horrigan, Chief Medical Officer; Mike Snape, Chief Scientific Officer and other clinical team members.**

 **AskBio BioPharmaceutical** is developing life-saving AAV gene therapy in partnership with patients, families, medical societies, and patient advocacy groups that is focused on neuromuscular and central nervous system conditions including myotonic dystrophy. **Hosts live in the booth: Matthew Alsante, Director of Patient Advocacy and Dr. Jonathan Doering, Project Manager.**


 **Audentes** develops genetic medicines with the potential to deliver transformative value for patients and is currently exploring three gene therapy modalities: gene replacement, exon skipping gene therapy, and vectorized RNA knock-down. **Come talk with the Audentes team live in our booth.**


 **Avidity Biosciences** is proud to be a Conference Partner of the MDF 2020 Virtual Conference. Our goal is to improve the lives of people affected by serious diseases by advancing a new class of targeted RNA therapies, called AOCs (Antibody Oligonucleotide Conjugates) initially focused on developing a pipeline of AOCs in muscle diseases, including DM1. **We invite you to talk live with our team: Art Levin, Chief Scientific Officer; Lisa Ackermann, Vice President of Clinical Development; Alissa Peters, Director of Patient Advocacy, Rob Burke, Director of Research; Barbora Malecova, Associate Director of Research.**


 **Biogen** was founded in 1978 as one of the world's first global biotechnology companies and has led innovative scientific research with the goal over the last decade to defeat devastating neurological diseases. Our booth will focus on patient centered drug development and clinical endpoints for DM1. **The research team will be live in the booth on Friday.**

 **Dyne Therapeutics** is focused on innovative life-transforming therapeutics for patients with genetically driven diseases utilizing their proprietary FORCE™ platform to overcome the current limitations of muscle tissue delivery to advance modern oligonucleotide therapeutics. Dyne is targeting muscle tissue with the goal of stopping or reversing progression


in a broad range of diseases including DM1. **Live in the booth will be Romesh Subramanian, PhD, Chief Scientific Officer and Molly White, Vice President, Medical Communications & Advocacy.**


 **Expansion Therapeutics** Visit the Expansion Therapeutics booth to learn about their DM program and progress.


 **Harmony Biosciences** Come explore the Harmony Biosciences booth to learn about their DM program and progress.

 **Ionis** is the leading innovator in RNA-targeted therapeutics and was purposely designed to create a better, more efficient drug discovery platform, establish a new innovation-centered business model, and maximize the value of every medicine we create and get it to the people who need it most. **Live booth host: Patrick Cauntay, Assistant Director, Patient Centric Drug Development.**

 **Lupin Neurosciences** is a specialty pharma division of Lupin with an initial focus on meeting the unmet needs of patients with myotonic disorders. **Live booth hosts will include: Dr Greg Kaufman, Senior Vice President Global Clinical and Medical Affairs; Dr Alla Zozulya-Weidenfeller, Director Medical Affairs, Neurology EMEA; Brajesh Pandey, Associate Medical Director, US; Zoey Goldwater, Medical Affairs, Neurology EMEA; Beatrice Decane, Medical Affairs US.**

 **Neubase Therapeutics** has a synthetic genetic medicines platform and is developing a new treatment option for patients with DM1. The lead candidate (NT-0200) is designed to engage with the hairpin on just the mutant copy of the DMPK RNA molecule and restore normal cellular function, potentially stopping or slowing disease progression. Other genetic disease therapies, including DM2, are in the pipeline. **Hosted by members of the Neubase team.**

 **Pfizer** Come explore the Pfizer booth to learn about their DM program and progress.

 **Syros** is redefining the power of small molecules to control the expression of genes. Our myotonic dystrophy program goal is to develop an oral medicine that decreases the expression of the mutated copy of the DMPK gene, lowering levels of toxic RNA, to provide a therapeutic benefit for patients with DM1. **Live during the Showcase: John Carulli, PhD, Vice President of Biology.**

Exhibitor Showcase

Check out our Conference Exhibitor's Virtual Showcase booths any time by clicking on the menu **Exhibitors>Conference Exhibitors** and then click on their images. Interact live with representatives during the Conference from **10AM to 12PM and 4PM to 6PM Pacific** both days.



Stanford University Dr. John Day's research team will be available to describe the studies we have ongoing at Stanford University. Visit our booth to learn how to enroll in our Recruitment Database for studies and trials, and learn more about our Myotonic Dystrophy Biobank. **Attendees: Katharine Hagerman, PhD and others from the Stanford team.**



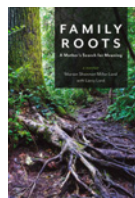
University of Rochester's myotonic dystrophy team conducts research and provides clinical care for people with DM. Our 22 person team is dedicated to finding better treatments for both DM1 and DM2 and we partner with patients and families to understand more about these complicated conditions. We partner with MDF, other medical centers across the globe, as well a pharma and biotech companies. Our National Registry connects patients and families affected by DM with researchers. **Booth hosts: University of Rochester research team: study coordinators, physicians, physical therapists.**



Virginia Commonwealth University is conducting the largest study of myotonic dystrophy so far, END-DM1, which is representing cooperation of families and researchers from six countries in North America and Europe, and is also partnering with biotechnology companies that are working on myotonic dystrophy. **Members of the VCU research team will be live in their booth.**



Neuromuscular Disease Network for Canada (NMD4C) is a new pan-Canadian network that brings together the country's leading clinical, scientific, technical, and patient expertise to improve care, research, and collaboration in neuromuscular disease. **Live in our booth and ready to talk with you: Adrienne Capaldi, Communications Coordinator at NMD4C; Dr. Cynthia Gagnon, Knowledge Translation Lead at NMD4C and Professor of rehabilitation at the University of Sherbrooke, Dr. Erin Beattie, Network Manager at NMD4C; Dr. Homira Osman, Investigator at NMD4C and Director of Knowledge Translation and External Engagement at Muscular Dystrophy Canada; Dr. Valérie Gagné-Ouellet, Knowledge Broker at NMD4C and Research Professional at the Interdisciplinary Research Group on Neuromuscular Disease.**



Larry Lord will present his late wife, Shannon Lord's book entitled *"Family Roots: A Mother's Search for Meaning"* the story of a family confronting its own genetic messaging. Shannon Lord was the Founding Chair of the MDF Board of Directors from 2006-2009. An artist, writer, gardener and volunteer, she was also a myotonic dystrophy advocate for nearly ten years by raising \$300,000 in research funding, speaking at International Myotonic Dystrophy Conferences, the NIH and other places. She convened the American family members who attended the International Myotonic Dystrophy Consortium (IDMC-5) in Quebec in 2005. Out of that meeting MDF evolved with Shannon as a founding member.



Terry Cohen and Barry Cohen, PhD will be presenting two books about living with myotonic dystrophy and leading a full life. *"Living Wisely - For Millennials & Beyond: Essential Skills for Life's Journey"* which explores the essential life skills that millennials beginning their journeys through life-as well as all the rest of us-need to live wisely and well throughout our lives, and *"Travel Near & Travel Far: Step Out of Your Disabled World!"* which is about traveling with a disability and was in The NY Times travel section as well as on national radio shows.



Ann S. Woodbury, an MDF Support Group Facilitator for 10 years and a full-time caregiver for her husband and four children (now adults) who all live with DM. She will be on hand to discuss her soon-to-be-released *"Surviving Myotonic Dystrophy: A Mother's Struggle to Care for her Family with a Rare Disease"*.



Rob Besecker is an award winning inspirational writer who's books include *"Everest Strong: Reaching New Heights with Chronic Illness"* and *"Overcoming Obstacles and Challenges in Life and Pursuing Goals and Dreams"*. He is also an inspirational speaker, healthcare professional and an MDF Support Group Facilitator.

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 more than 78 countries around the world. What helps make this organization so meaningful is your role in it – your advocacy to advance “Care and a Cure” and your support of us and of one another. We're thrilled that so many of you have found each other through the Foundation and are building support groups. We would like to specially thank our Support Group Facilitators for donating their time and energy to create these support opportunities.

Find our virtual support options here: <https://www.myotonic.org/find-support>.

Learn more about our Support Group Facilitators here: <https://www.myotonic.org/support-group-facilitators>.

Find out about upcoming support groups and events on our calendar: <https://www.myotonic.org/calendar/month>. Aside from reaching out directly, you can also share your contact information with our support group facilitators.

If you are interested in volunteering as a Support Group Facilitator, please contact MDF at info@myotonic.org.



Rob Besecker, MBA
Chicago, IL, USA



Teresa Buffone
Ottawa, Canada



Barry Cohen, PhD
Florida, USA



John Cooley
Kansas City Area, USA



Paul Dillon
Worthington, OH, USA



Loraine Dressler
Orange County, CA, USA



Jonathan Freedman
Seattle, WA, USA



Patricia Gibson
Kansas City Region, USA



Bob Gould
Seattle, WA, USA



Kay Hayes
Denver, CO, USA



Tim Haylon
Worcester, MA, USA



Chuck Hunt
Atlanta, Georgia, USA

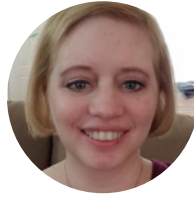
Support Group Facilitators (continued)



Suzette Ison, RN, CMCN
Indianapolis, IN, USA



Emily Jones
Rochester, NY, USA



Mindy Kim
North Carolina, USA &
Adult Facebook Chat



Anke Klein
International German
Speakers



Leslie Krongold, EdD
Northern California, USA



Clare May
Portland, OR, USA



Tom McPeck
DM2 Virtual
Support Group



Sherry Morris
Dallas, TX, USA



Bill Nuttall
Adult Facebook Chat



Suzanne Perkins
Michigan, USA



Ted Salwin
Indianapolis, IN, USA



Patrick Welker
Dallas, TX, USA



Carolyn Valek
Worthington, OH, USA



Ann Woodbury
Utah & Mountain West
Region, USA

Learn more about our Support Group Facilitators here:
<https://www.myotonic.org/support-group-facilitators>.

MDF Research Fellows

Introducing the 2020 MDF Pre- and Postdoctoral Research Fellows! Trainees applied for support for projects focused on basic, translational, or clinical research in myotonic dystrophy (DM). This program not only supports meritorious research, but also helps continue to build a critical mass of researchers engaged in working on DM. To learn more about our MDF Research Fellows go to <https://www.myotonic.org/myotonic-fellows-grant-recipient>. Be sure to visit the Fellows' exhibit booths at the conference during Showcase hours to talk with them live about their research!



Raphael Benhamou, PhD
Scripps Research Institute,
Florida



Shruti Choudhary, Ph.D.
Scripps Research Institute,
Florida



Talita Conte, PhD
University of Montreal



Jana Jenquin, PhD
University of Florida



Sarah Overby
University of Valencia



Carl Shotwell
University of Florida

MDF Staff

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



Tanya Stevenson, EdD, MPH
Chief Executive Officer



Allison Formal, MBA
Senior Science Advisor



Leah Hellerstein,
LCSW, MSW, MPH
Program Director



Kleed Cumming
Communications &
Technology Manager



Nadine Skinner, PhD, MPA
Research Coordinator



Sara Littlefield
Development Associate

MDF Scientific Advisory Committee

The MDF Scientific Advisory Committee is comprised of individuals who collectively have devoted more than ninety 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



Kathie Bishop, PhD
Locana, Inc.



Thomas A. Cooper, MD
Baylor College
of Medicine



John W. Day, MD, PhD
Stanford University



Douglas Kerr, MD, PhD, MBA
Generation Bio



Darren Monckton, PhD
University of Glasgow



Richard Moxley III, MD
University of
Rochester



Laura Ranum, PhD
University of
Florida



Charles Thornton, MD
Board Member

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 with the disorder. 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>.
Visit the Board in their virtual Exhibit Booth during Conference Showcase hours.



Jeremy Kelly
Board Chair & Lifetime Trustee



Martha Montag Brown
Vice-Chair



Elizabeth Florence
Secretary



David Herbert
Treasurer



David Berman, MBA
Board Member



John W. Day, MD, PhD
Board Member



John Fitzpatrick
Board Member



Joel Revill
Board Member



Charles Thornton, MD
Board Member

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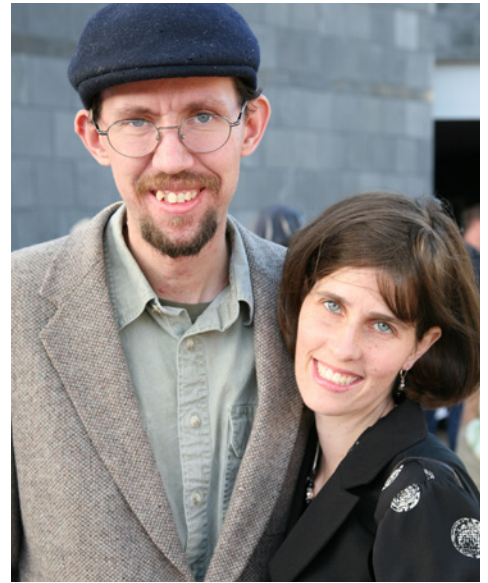


SILVER



BRONZE





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>



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