



MYOTONIC
DYSTROPHY
FOUNDATION

Care and a Cure

2018 MDF ANNUAL CONFERENCE

September 14-15, 2018
Nashville, TN



Welcome to the 2018 MDF Annual Conference!



We are delighted that you have joined us in the exciting city of Nashville, TN for what is the leading global summit of DM families, researchers, physicians and industry representatives. **We are welcoming more than 125 First-Timers to the conference this year** – a record! Please be sure to greet them and help make them part of this great community.

Learn, Connect, Share, Celebrate

You will find a robust array of education and networking opportunities this year, including new sessions such as *Patient Report Out on Cannabis Use for Symptom Management*, *Occupational Therapy: Strategies for Managing Disease Progression*, and a fun, interactive lesson on adaptive dance. You'll also find ongoing favorites such as *DM 101: Getting a Handle on the Basics*, family panel discussions for caregivers, DM2 family members and more, all designed to make life easier.

We are also presenting two exciting new general sessions, one featuring a panel of community members and audience members reporting out on changes in disease symptoms over time, and the release of the first-ever clinical care recommendations for this disease. **We need your help** to make sure clinicians treating our community are using them, and we'll have some very entertaining skits with DM doctors and family members to help you understand your role in our dissemination efforts.

More Research Sessions

We've expanded our very popular Research track this year to include a Poster Session, presentations on drug development efforts from industry, an update on drug development acceleration efforts led by MDF, and more.

Professional Meetings Track

MDF is presenting a full-day Friday closed meeting track for academic, industry and federal agency professionals, designed to drive coordination, communication and transparency across the DM drug development pipeline and accelerate therapy development. We will let you know how this track goes in our post-conference report.

In short, while we say it every year, this year's conference is our most robust and comprehensive yet, and your participation is what makes it a success. Thank you for coming, and for all you do to drive Care and a Cure for myotonic dystrophy. **Enjoy!**

A handwritten signature in black ink, appearing to read 'Molly White', with a long horizontal line extending to the right.

Molly White
Chief Executive Officer

Agenda- Friday, September 14, 2018

Time	DM Overview & Management	Community Engagement & Support	JOA Track	Research Showcase		
8:30 AM – 5:30 PM	Registration <i>Governor's Lobby</i>					
9:00 AM – 9:50 AM	Support Group Facilitators Breakfast (Closed) <i>Invited Participants</i>					
10:00 AM – 10:50 AM	First Timers' Tea <i>Governor's Ballroom</i>					
12:30 PM – 1:00 PM	Conference Welcome <i>Governor's Ballroom</i>					
1:00 PM – 1:50 PM	Gut Instincts: GI Symptom Management Leila Neshatian, MD, MSc <i>Governor's Ballroom</i>	The DM2 Patient Experience: Community Panel Facilitator: Thomas McPeck <i>Chamber B</i>	Congenital Community Panel Facilitator: Woodie Kessel, MD, MPH <i>Chamber C</i>	JOA Program Barry Cohen, PhD; Diane Bade, RN, CAVS <i>Chamber A</i>	Research & Vendor Showcase <i>Governor's Lobby</i>	
2:00 PM – 2:50 PM	Breathe Easy: Respiratory Symptom Management Valeria Sansone, MD <i>Governor's Ballroom</i>	DM101: Getting a Handle on the Basics Nicholas Johnson, MD <i>Chamber D</i>	Male Caregivers Community Panel Facilitator: John Fitzpatrick <i>Chamber B</i>			Female Caregivers Community Panel Facilitator: Erica Kelly <i>Chamber C</i>
3:00 PM – 3:50 PM	Poster Session <i>Governor's Ballroom</i>					
4:00 PM – 4:50 PM	Staying Strong: DM and Exercise Katy Eichinger, PhD <i>Chamber D</i>	Patient Report Out on Cannabis for Symptom Management Facilitator: Woodie Kessel, MD, MPH <i>Governor's Ballroom</i>	Becoming a DM Advocate: Community Update & Training Jenn Dale; Faegre Baker Daniels <i>Chamber C</i>			
5:00 PM – 5:50 PM	Chair Tap Adaptive Dance Class Mindy Kim <i>Governor's Ballroom</i>					
5:00 PM – 6:00 PM	Break					
6:00 PM – 8:00 PM	Opening Reception <i>Water's Edge</i>					

Agenda - Saturday, September 15, 2018

Time	Keynote Sessions	JOA Track	Research Showcase	
7:00 AM – 9:00 AM	Breakfast <i>Governor's Lobby</i>			
8:00 AM – 5:00 PM	Registration <i>Governor's Lobby</i>			
9:00 AM – 10:50 AM	Living with DM: Patients Report on Changes Over Time Moderator: Charles Thornton, MD <i>Governor's Ballroom</i>	JOA Program Barry Cohen, PhD; Diane Bade, RN, CAVS <i>Chamber A</i>	Research & Vendor Showcase <i>Governor's Lobby</i>	
11:00 AM – 11:50 AM	Strategies for Managing Progression Cynthia Gagnon, PhD, OT; Missy Dixon, PhD <i>Governor's Ballroom</i>			
12:00 PM – 12:50 PM	Lunch <i>Governor's Lobby & Ballroom</i>			DM2 Research Update Lunch Chad Heatwole, MD <i>Chamber E</i>
1:00 PM – 2:20 PM	Consensus-based Care Recommendations: What Do They Mean for You? Facilitator: Jacinda Sampson, MD, PhD <i>Governor's Ballroom</i>			
2:20 PM – 2:40 PM	Break			
2:40 PM – 3:15 PM	Accelerating the Search for Therapies: What's Happening, What's Next Elizabeth Ackermann, PhD <i>Governor's Ballroom</i>			
3:15 PM – 3:30 PM	Conference Challenge			
3:40 PM – 4:50 PM	Industry Updates on Drug Development Ranjan Batra, PhD, Locana; Matthew Disney, PhD, Expansion Therapeutics; Joseph Horrigan, MD, AMO Pharma Ltd.; Laurence Mignon, PhD, Ionis Pharmaceuticals <i>Governor's Ballroom</i>			
5:00 PM – 5:15 PM	Conference Q&A <i>Governor's Ballroom</i>			
5:15 PM – 6:00 PM	No-host Cocktail Reception <i>Governor's Lobby</i>			
6:00 PM – 9:00 PM	Closing Dinner, Line Dancing Class & Dance Party <i>Governor's Ballroom</i>			

Speakers



Elizabeth Ackermann, PhD
Myotonic Dystrophy Foundation

Lisa Ackermann is CSO at MDF. She has over 20 years of pharmaceutical drug discovery and development experience, leading both preclinical and clinical teams in the areas of rare genetic disorders and Alzheimer's Disease. Prior to joining MDF she worked for over 8 years at Ionis Pharmaceuticals serving as VP of Clinical Development.



Diane Bade, RN, CAVS

Diane Bade is a registered nurse by training and a caregiver by avocation. Diane is the widow of Christopher Bade, having journeyed with him for over 30 years with DM1. Diane lives in Washington State. Her adult children live with DM1: Scott, Christine and Nicholas, who passed away in 2017.



Ranjan Batra, PhD
Locana

Ron Batra is the Vice President of Research and Development at Locana Bio in San Diego. He is involved in pre-clinical development and translational therapeutics for myotonic dystrophy and other RNA mediated diseases. Locana utilizes cutting-edge RNA targeting CRISPR approaches to target disease causing RNA.



Barry Cohen, PhD

Barry Cohen is a retired consulting psychologist and DM caregiver who lives in Florida. Dr. Cohen and his son, Terry, who lives with DM1, have published several books directed to the myotonic dystrophy community, including his most recent, "Living Wisely: For Millennials & Beyond".



Jenn Dale
Faegre Baker Daniels

Jenn Dale is an advisor on the Health and Biosciences team at Faegre Baker Daniels Consulting. A veteran congressional staffer who has served as an operative for three congressional representatives, Jenn is an experienced navigator of the federal legislative process.



Matt Disney, PhD
The Scripps Research Institute & Expansion Therapeutics

Matt Disney is a professor in the Department of Chemistry at the Scripps Research Institute in Florida. His lab recently developed lead therapeutics that improve defects associated with the most common adult-onset forms of muscular dystrophy (myotonic dystrophy Types 1 and 2) in both animal and cellular models of the disease.



Missy Dixon, PhD
University of Utah

Missy Dixon is the Program Director of the Utah Program for Inherited Neuromuscular Disorders at the University of Utah. Her research interests include understanding cognitive functions and adaptive behaviors in inherited muscle disorders, including congenital myotonic dystrophy (CDM) in order to develop interventions tailored to affected individuals.



Katy Eichinger, PT, PhD
University of Rochester

Katy Eichinger is a physical therapist and Assistant Professor at the University of Rochester where she is involved in the clinical care of individuals with adult and pediatric neuromuscular conditions. She is part of the neuromuscular research team and is involved in natural history studies and clinical trials involving patients with myotonic dystrophy and other neuromuscular diseases.

Speakers



John Fitzpatrick
Myotonic Dystrophy Foundation

John Fitzpatrick is a director on the MDF Board and serves as Executive Director of Educate Texas. He has been involved in the educational and political arenas, including a four-year stint on Capitol Hill where he worked on education and workforce development policies. He lives in Austin, Texas with his family.



Nicholas Johnson, MD, FAAN
Virginia Commonwealth University

Nicholas E. Johnson is an associate professor and Vice Chair of Research in Neurology at Virginia Commonwealth University with a focus in inherited neuromuscular disorders. His laboratory is working to identify the pathogenesis of myotonic dystrophy and appropriate clinical endpoints for these conditions.



Cynthia Gagnon, PhD, OT
Université de Sherbrooke

Cynthia Gagnon is an associate professor on the faculty of Medicine and Health Sciences at Sherbrooke University and director of the Groupe de recherche interdisciplinaire sur les maladies neuromusculaires (GRIMN). She is the principal investigator of the largest interdisciplinary DM1 natural history study, leading to 21 publications in the field of participation, mobility, muscle and central nervous impairments.



Erica Kelly

Erica Kelly attended Phillips Academy Andover and Dartmouth University, and then worked in banking and recruitment in New York and London. She is married to Jeremy Kelly, MDF Lifetime Trustee, and mother to Jack and Ben, who were diagnosed with DM in 2005. Erica is a longstanding volunteer at MDF who grew up in Bend, Oregon and now lives in Mill Valley, California.



Chad Heatwole, MD
University of Rochester

Chad Heatwole is an associate professor, clinician, and researcher at the University of Rochester, who focuses on the care and management of patients with muscular dystrophy. Dr. Heatwole's primary research interest is in the development, evaluation, and testing of novel experimental therapeutics for neuromuscular diseases.



Woodie Kessel, MD, MPH
Myotonic Dystrophy Foundation

Woodie Kessel is MDF Board Chair and a community pediatrician and child advocate. Dr. Kessel is currently the CEK Senior Child Health Scholar in Residence at the C E Koop Institute, Dartmouth College and Medical School; Professor of Pediatrics, Geisel School of Medicine, Dartmouth College; and Professor of the Practice at the University of Maryland's School of Public Health. Previously, Dr. Kessel served as U.S. Assistant Surgeon General.



Joseph Horrigan, MD
AMO Pharma Ltd.

Joe Horrigan is the Chief Medical Officer at AMO Pharma. He is a pediatric neuropsychiatrist with 20 years of experience in the pharmaceutical and biotech industry, both as a clinical investigator and as a sponsor. He is currently helping to develop new therapies for DM and expand AMO's drug development pipeline.



Mindy Kim

Mindy Kim is the founder of Footworkz, a dance company that teaches young students to dance without the added pressure of competition. Mindy was diagnosed with DM in 2010 and facilitates an MDF support group in Greensboro, North Carolina. Mindy's disease progression has led her to create seated dance classes.

Speakers



Tom McPeek

Tom McPeek, from Ohio, spent 30 years helping individuals who were in some way involved in the criminal justice system. Tom was diagnosed with DM2 in 2008 and is currently retired due to his DM diagnosis, but continues to be active in the MDF community as a volunteer.



Jacinda Sampson, MD, PhD Stanford University

Jacinda Sampson is a clinical associate professor at Stanford University Hospitals and Clinics. She is a neuromuscular and neurogenetics specialist, who has been caring for patients and families with myotonic dystrophies and other familial neurological disorders for over 12 years. She is actively involved in research in myotonic dystrophy.



Laurence Mignon, PhD Ionis Pharmaceuticals

Laury Mignon is a director of Clinical Development at Ionis Pharmaceuticals. She was responsible for the Phase 1/2A clinical trial for IONIS-DMPKRx, a potential therapy for DM1 that was completed in January 2017, and a part of the Ionis team that developed Spinraza, a drug approved for the treatment of spinal muscular atrophy. Prior to joining Ionis Pharmaceuticals, Dr. Mignon was a clinical scientist at Orexigen Therapeutics and an Associate Scientist in the Department of Neurology at UCLA.



Valeria Sansone, MD NEuroMuscular Omnicentre (NEMO)

Valeria Sansone has extensive clinical and bench science experience in neuromuscular disorders both from a clinical and basic science perspectives. For the past 22 years she has been in charge of in-patients with muscle disorders at the University Department of Neurology in Milan and the national out-patient neuromuscular clinic at the NEMO clinic. Her main field of research has included the myotonic dystrophies.



Leila Neshatian, MD, MSc Stanford University

Leila Neshatian is a neurogastroenterologist and an assistant professor of Medicine at Stanford University, Division of Gastroenterology and Hepatology. Her clinical areas of expertise and research include functional gastrointestinal and motility disorders.



Charles Thornton, MD University of Rochester

Charles Thornton is a clinician and researcher whose work spans the interval from lab bench to clinic. He has contributed to the knowledge of genetics, molecular mechanisms, clinical features, natural history, and biomarkers of myotonic dystrophy, collaborating broadly to develop new treatments and bring them to clinical trials.

Acknowledgements

The Myotonic Dystrophy Foundation extends its sincere appreciation to

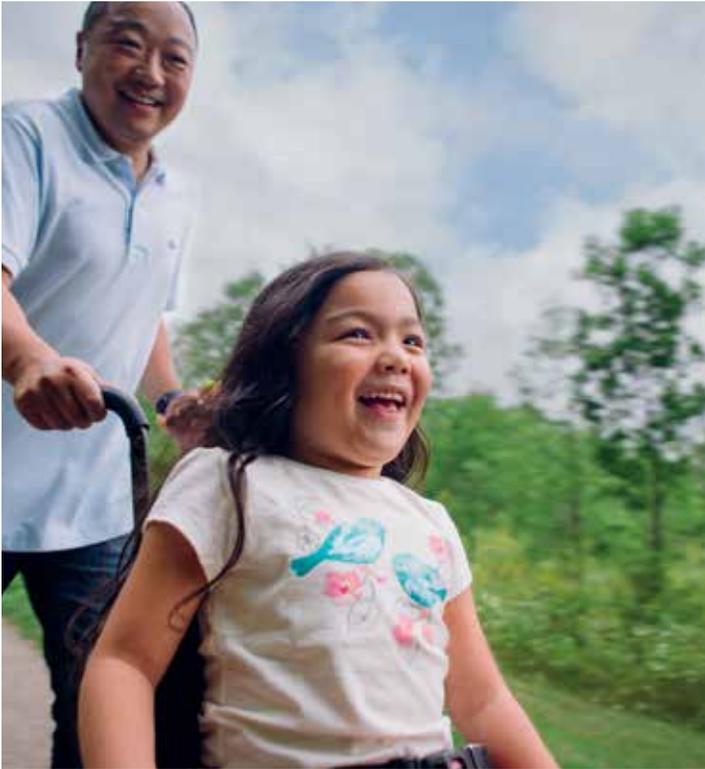
THE PROMISE TO KATE FOUNDATION
for providing 2018 conference scholarship
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THE COHEN FAMILY TRUST
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juvenile-onset adult attendance at the 2018
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Driven to discover the cure

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Venue Map



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