

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME

Saturday, March 2, 2024

12:00 – 6:00 PM	Registration Open for Care Center Directors & Large Exhibit Booth Sponsors	Orlando Ballroom
5:30 – 7:00 PM	MDA Care Center Network Happy Hour	

Sunday, March 3, 2024

7:00 AM – 8:00 PM	Registration Open	
8:00 AM – 3:30 PM	MDA Care Center Directors Meeting (Invite Only)	Lake Eola
8:00 AM – 3:30 PM	MDA Care Center Network Ancillary Care Symposia (Invite Only)	Lake Mizell
1:00 – 5:00 PM	MDA Trainee Networking Session (Invite Only)	Champions Gate
2:00 – 5:00 PM	Neuromuscular Advocacy Collaborative Meeting (Invite Only)	Lake Nona
6:00 – 8:00 PM	Welcome Reception in Exhibit Hall	Orlando Ballroom

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME

Monday, March 4, 2024

6:00 AM – 6:00 PM	Registration Open	
7:00 – 8:00 AM	Industry Forum Breakfasts: argenx, Sarepta	
8:30 – 9:30 AM	Opening Remarks MDA Legacy Award	Orange Ballroom
9:30 – 9:55 AM	Networking Break in Exhibit Hall	Orlando Ballroom
9:30 – 10:30 AM	Exhibit Hall and Poster Sessions Open	Orlando Ballroom
10:00 AM – 12:00 PM	Keynote Address Panel Discussion on NMD in the Age of Therapy	Orange Ballroom
12:00 – 1:30 PM	Industry Forum Lunches: Biogen, Edgewise, Genentech, Pfizer, Sarepta	
12:00 – 1:30 PM	Exhibit Hall and Poster Sessions Open	Orlando Ballroom
Clinical Considerations		
2:00 – 3:30 PM	Clinical Case Reviews: Part I John Day, MD, PhD (Co-Chair) Bakri Elsheikh, MBBS, FRCP, FAAN (Co-Chair) Nanette Joyce, MD Chamindra Laverty, MD Jennifer Roggenbuck, MS, CGC Jacinda Sampson, MD Megan Waldrop, MD Matthew Wicklund, MD, FAAN	
Lab to Life		
2:00 – 3:30 PM	Translational research in ALS: Novel Biomarkers and Model Systems Rita Sattler, MSc, PhD (Chair) Jimena Andersen, PhD Fredrick (Eric) Arnold, PhD Askar Safipour Afshar MS Michael Ward, MD, PhD	
Clinical Approaches in Streamlining Care		
2:00 – 3:30 PM	Newborn Screening Marcia Felker, MD (Chair) Thomas Crawford, MD Katerina Kucera, PhD Nishitha Pillai, MD Norma Tavakoli, PhD	

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME

Monday, March 4, 2024 cont.

Practical Consideration in Gene Therapy

2:00 - 3:30 PM	Where Are We in Gene Therapy? Holly Peay, PhD (Chair) Diana Castro, MD Richard Finkel, MD Perry Shieh, MD, PhD	
3:30 - 4:30 PM	Exhibit Hall and Poster Sessions Open	
3:30 - 3:55 PM	Networking Break sponsored by Entrada Therapeutics	

Clinical Considerations

4:00 - 6:00 PM	Clinical Case Reviews: Part II John Day, MD, PhD (Co-Chairs) Bakri Elsheikh, MBBS, FRCP, FAAN (Co-Chairs) Dominic Fullenkamp, MD, PhD Natalie Katz, MD, PhD Jennifer Roggenbuck, MS, CGC Matthew Wicklund, MD, FAAN	
----------------	--	--

Lab to Life

4:00 - 5:00 PM	ALS Genetic Discovery, Mechanisms, and Therapeutic Development Matthew Harms, MD (Chair) Sam Alworth Devesh Pant, PhD Tess Pottinger, PhD Bryan Traynor, MD, PhD	
5:00 - 6:00 PM	Panel Session: Natural History Studies: Most Recent Collaborative Consortia Efforts Matthew Harms, MD (Chair) Robert Bowser, PhD James Berry, MD Stephanie Fradette, PharmD Amelie Gubit, PhD	

Clinical Approaches in Streamlining Care

4:00 - 6:00 PM	Pediatric Neuromuscular Disease Elena Caron, MD (Chair) Faria Abdullah, MD Brian Lindsey, DO Amanda Witt, MD	
----------------	---	--

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME

Monday, March 4, 2024 cont.

Practical Consideration in Gene Therapy

4:00 – 6:00 PM	Is My Institution Equipped for Gene Replacement Therapies? Crystal Proud, MD (Chair) Omer Abdul-Hamid, MD Nancy Kuntz, MD Aravindhan Veerapandiyan, MD	
6:00 – 8:00 PM	Poster & Networking Reception	

Tuesday, March 5, 2024

6:00 AM – 6:00 PM	Registration Open	
7:00 – 8:00 AM	Industry Forum Breakfasts: Biogen, Catalyst, ML Bio Solutions, UCB	
Clinical Considerations		
8:30 – 10:30 AM	Decisions in the Age of Treatment Richard Finkel, MD (Chair) Anne Connolly, MD Katherine Mathews, MD Julie Parsons, MD Perry Shieh, MD, PhD	
Lab to Life		
8:30 – 10:30 AM	Development of Gene Therapy for Rare and Ultra-Rare Neuromuscular Disease Alan Beggs, PhD (Chair) Anna Buj-Bello, PhD Casey Childers, DO, PhD Suyash Prasad, MBBS	
Clinical Approaches to Streamlining Care		
8:30 – 10:30 AM	Adult Neuromuscular Disease: Engaging Primary Care and Other Non-NMD Experts Lora Clawson, MSN, CRNP (Chair) Becky Hopfinger, MSN, MBA, FNP-BC Bruce Rabin, MD, PhD	
Practical Considerations in Gene Therapy		
8:30 – 10:30 AM	My Institution is Not Equipped to Administer Gene Replacement Therapy: Now What? John Brandsema, MD (Chair) Taylor Kaschak, BSN, RN	
Disease Mechanism & Therapeutic Strategies		
8:30 – 10:30 AM	Role of Immune System and the Microenvironment in Neuromuscular Disorders Aaron Beedle, PhD (Co-Chair) Jyoti Jaiswal, MSc, PhD (Co-Chair) Alexis Demonbreun, PhD Daniel Kopinke, PhD Melissa Morales, PhD Andreas Patsalos, MS, PhD Armando Villalta, PhD Yonne Tenario de Menzes, PhD	

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME

Tuesday, March 5, 2024 cont.

10:30 AM – 1:30 PM	Exhibit Hall and Poster Sessions Open	
10:30 – 10:55 AM	Networking Break	
Clinical Considerations		
11:00 AM – 12:00 PM	Setting Patients Up for Success: Life Transitions Jamie Eskuri, MD (Chair) Candice Johnson, OTD, ORT/L Kaitlin Lewis, CTRS Emelia Rogers, LICSW	
Lab to Life		
11:00 AM – 12:00 PM	Neuromuscular Junction Disease Linda Kusner, PhD (Chair) Henry Kaminski, MD Linda Kusner, PhD Ricardo Maselli, MD Stephen Meriney, PhD	
Clinical Approaches to Streamline Care		
11:00 AM – 12:00 PM	Novel Strategies for Clinical Trial Recruitment & Participation Gil Wolfe, MD, FAAN (Chair) Amy Bartlett, BA, CCRC Helen Hemley, MPA	
Practical Considerations in Gene Therapy		
11:00 AM – 12:00 PM	Gene Therapy in DMD: Is It Now or Never? Natalie Goedeker, DNP, CPNP (Chair) Emma Ciafaloni, MD, FAAN	
Disease Mechanism & Therapeutic Strategies		
11:00 AM – 12:00 PM	Single-Cell and Spatial Transcriptomics to Understand Disease Doug Millay, PhD (Chair) Veronique Belzil, MS, PhD Jordi Diaz Manera, PhD Carrie Miceli, PhD Mathula Thangarajh, MD, PhD	
12:00 – 1:30 PM	Industry Forum Lunches: argenx, Biogen, Fulcrum, Sanofi, Scholar Rock	

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME

Tuesday, March 5, 2024 cont.

Lab to Life

2:00 PM – 4:00 PM

Dystrophies
Katherine Mathews, MD (Chair)
Russell Butterfield, MD, PhD
Renatta Knox, MD, PhD
Elizabeth McNally, MD, PhD
Charles Thornton, MD
Kevin Wright, PhD

Clinical Approaches to Streamline Care

2:00 PM – 4:00 PM

Underserved Communities and Developing Countries & Working with What We've Got: Overcoming Challenges in the Healthcare System
Monika Krzesniak-Swinarska, MD (Co-Chair)
Meeta Cardon, MD (Co-Chair)
Anna Kostera-Pruszczyk, MD, PhD
Leslie Morrison, MD

Practical Considerations in Gene Therapy

2:00 PM – 4:00 PM

Day-of: Orchestrating the Team & Planning for the Unexpected & We Did It - Now What? Navigating post GTT Monitoring in "Real Life"
Julie Parsons, MD (Chair)
Diana Bharucha-Goebel, MD
Nora Capocci, MPH
Erika Finanger, MD
Leigh Ramos-Platt, MD

Disease Mechanism & Therapeutic Strategies

2:00 – 4:00 PM

Multi-Disease and Next Generation Genetic Therapies
Dwi Kemaladewi, PhD (Co-Chair)
Noah Weisleder, PhD (Co-Chair)
Dounia Abbadi, PhD
Thomas Gaj, PhD
Sajedah Hindi, PhD
Anders Naar, PhD
Sharif Tabebordbar, PhD
Hichem Tasfaout, PharmD, PhD

3:30 – 4:30 PM

Exhibit Hall and Poster Sessions Open

4:00 – 4:25 PM

Networking Break

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME

Tuesday, March 5, 2024 cont.

Clinical Considerations

4:30 – 6:00 PM
Overview of Therapeutics for FSHD
Jeffrey Statland, MD (Chair)
Enrico Bugiardini, PhD
Natalie Katz, MD, PhD
Rabi Tawil, MD

Lab to Life

4:30 – 6:00 PM
CMT (Peripheral Nerve Disease)
Michael Shy, MD (Chair)
Daniel Calame, MD, PhD
David Herrmann, MBBCh
Alysia Vrailas-Mortimer, PhD
Stephan Züchner, MD, PhD

Clinical Approaches to Streamline Care

4:30 – 6:00 PM
Multidisciplinary Care Panels: Meeting the Needs of the Patient TODAY
Amanda Peltier, MD (Chair)
Urvi Desai, MBBS, MD, MD, FAAN

Practical Considerations in Gene Therapy

4:30 – 6:00 PM
Navigating Difficult Conversations
Diedre Devier, PhD (Chair)
Kaitlin Batley, MD
Aravindhan Veerapandiyan, MD

Disease Mechanism & Therapeutic Strategies

4:30 – 6:00 PM
Dysfunction in Cellular Organelles and Trafficking in Neuromuscular Disorders
Vandana Gupta, PhD, MSc (Co-Chair)
Carlos Moraes, PhD (Co-Chair)
Daniel Christopher Bittel, PhD
Giovanni Manfredi, MD, PhD
Raghavendra Singh, PhD
Eric Wang, PhD

6:00 – 8:00 PM
Poster & Networking Reception



Conference Agenda

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME

Wednesday, March 6, 2024

7:00 AM – 1:00 PM	Registration Open	
8:30 AM – 12:30 PM	<p style="text-align: center;">New, Novel, and Noteworthy: NMD Highlights</p> <p style="text-align: center;">We are thrilled to have had an overwhelming number of incredible abstract submissions as we reconvene in 2024. To that end, we have redesigned our Clinical Trial Presentations to include a wider range of topics which also allows for even more live presentations selected directly from the abstract submissions.</p> <p style="text-align: center;">This is a final day not to be missed!</p>	