



Conference Agenda

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME
ROOM LOCATION SUBJECT TO CHANGE

Saturday, March 7, 2026

| | | |
|-------------------|--|---|
| 8:00 AM – 6:00 PM | Registration Open for Large Exhibit Booth Sponsors | Registration Desk in Main Foyer before escalators (lobby level) |
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Sunday, March 8, 2026

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| 7:00 AM – 7:00 PM | Registration Open | Registration Desk in Main Foyer before escalators (lobby level) |
| 7:30 AM – 3:30 PM | MDA Care Center Directors Meeting (Invite Only) | |
| 7:30 AM – 3:30 PM | MDA Allied Health Workshop (Invite Only) <i>Navigating Care Needs with Evolving Treatments: What's Changed?</i> Chairs: Gina Killian & Heather Hantak | |
| 12:00 – 2:00 PM | MDA Industry Council (Invite Only) | |
| 1:00 – 4:00 PM | MDA Trainee Networking Session | |
| 2:00 – 4:00 PM | Neuromuscular Advocacy Collaborative (Invite Only) | |
| 4:00 – 5:00 PM | Neuromuscular Stakeholder Networking Session (Invite Only) | |
| 6:00 – 8:00 PM | Welcome Reception & Posters in Exhibit Hall | Orlando Ballroom |

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME
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Monday, March 9, 2026

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|-------------------|--|---|
| 6:30 AM – 6:00 PM | Registration Open | Registration Desk in Main Foyer before escalators (lobby level) |
| 7:00 – 8:00 AM | Industry Forum Breakfast presented by argenx <i>First and only IgG Fc-antibody fragment for the treatment of generalized myasthenia gravis (gMG) in anti-AChR antibody positive adults</i> | Key Largo ABCD |
| | Industry Forum Breakfast presented by Biogen <i>Evaluating SPINRAZA (Nusinersen): Pivotal Data And RESPOND Interim Results From The Phase 4 Open-Label Study</i> | Florida 4 |
| | Industry Forum Breakfast presented by Dyne <i>Functional Improvement: Moving Beyond Dystrophin In DMD</i> | Key West ABCD |
| | Industry Forum Breakfast presented by Novartis <i>BTK in Neuroinflammatory Disease: Pathophysiology and Implications for Generalized Myasthenia Gravis</i> | Orlando V-VI |
| | Industry Forum Breakfast presented by Sarepta <i>Bridging the Gap: Incorporating Patient and Caregiver Perspectives into DMD Clinical Outcomes</i> | Florida 5-7 |
| 8:30 – 9:30 AM | <p>Opening Remarks Sharon Hesterlee, PhD President and CEO of the Muscular Dystrophy Association</p> <p>2026 MDA Donavon Decker Community Impact in Research Award Allison Moore, Hereditary Neuropathy Foundation (HNF)</p> <p>2026 MDA Research Momentum Award Łukasz Sznajder, PhD, University of Nevada</p> <p>Special Presentation <i>In Their Shoes: The Patient Experience</i> Lily Sander, MDA's National Ambassador</p> | Orange Ballroom |
| 9:30 – 10:15 AM | Keynote Address John F. Crowley, President & CEO of the Biotechnology Innovation Organization (BIO) | Orange Ballroom |
| 10:15 – 10:45 AM | Exhibit Hall & Poster Sessions Open | Orlando Ballroom |
| 10:45 – 11:45 AM | FDA's Rare Disease Innovation Hub and Rare Disease Initiatives Amy Comstock Rick, JD, Center for Drug Evaluation and Research (CDER) | Orange Ballroom |
| | 2026 MDA Legacy Award for Achievement in Clinical Research Michio Hirano, PhD, Columbia University | |
| 12:00 – 1:30 PM | Industry Forum Lunch presented by Edgewise Therapeutics <i>Elevation Becker Outcomes: Unlocking New Insights, Mobilizing the Community, and Redefining Care</i> | Florida 4 |
| | Industry Forum Lunch presented by Genentech <i>Considerations in SMA Management Across the Lifespan: Newborns to adults</i> | Florida 5-7 |
| | Industry Forum Lunch presented by Johnson & Johnson <i>Discover An FDA-Approved FcRn Option for Your gMG Patients 12 Years Of Age and Older</i> | Key West ABCD |
| | Industry Forum Lunch presented by Regenxbio <i>Advancing Duchenne Gene Therapy Trials in a New Era: Optimizing Design and Interpretation</i> | Key Largo ABCD |
| | Industry Forum Lunch presented by Scholar Rock <i>Going Beyond the Motor Neuron to Muscle: Expanding the Focus of SMA Care</i> | Orlando V-VI |

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME
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Monday, March 9, 2026 (cont.)

12:00 – 1:30 PM

Exhibit Hall & Poster Sessions Open

Orlando Ballroom

ALS

2:00 – 3:30 PM

From Genes to Pathways: New Frontiers in ALS Drug Discovery

ALS: Translating Genetic Discoveries Into Therapies

Chair – Bryan Traynor, MD, PhD, MMSc, FANA, FRCPI, FRCP

Lessons from the C9orf72 ASO Trial

Jonathan Glass, MD

The Treatable ALS Genome: Current Reality And Future Possibilities

Matthew Harms, MD

The Threads of Disease: Connecting Genes, Proteins, and Pathways in ALS

Ruth Chia, PhD

Orlando IV

Disease Mechanisms & Therapeutic Strategies

2:00 – 3:30 PM

Breaking the Cycle: Therapeutic Strategies for Repeat Expansion Diseases

Therapeutics Development In Spinal And Bulbar Muscular Atrophy (SBMA)

Chair – Christopher Grunseich, MD

New Treatments for Friedreich Ataxia

David Lynch, MD, PhD

Comparing RNA-Silencing Modalities for Nuclear-Retained DMPK Transcripts

Paymaan Jafar-nejad, MD

Dysregulated RNA Splicing In Myotonic Dystrophy And Development Of Potential Combination Therapeutics

Andy Berglund, PhD

Orange ABC

Neurology

CE Accredited

2:00 – 3:30 PM

Multi-System Management of Neuromuscular disorders

Integrating Research Into Multidisciplinary Care in ALS

Chair – Suma Babu, MBBS, MPH

Best Practices For Management Of Pulmonary Issues In ALS And Other Neuromuscular disorders

Katherine Dudley, MD, MPH

Best Practices And Innovation In AAC For ALS And Other Neuromuscular Diseases Withspeech Dysfunction

John Costello, MD

Orange D

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME
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Monday, March 9, 2026 (cont.)

Allied Health CE Accredited

2:00 - 3:30 PM

Coordinating and Administering Care: How to Pull Off the Balancing Act?

Chair – Christina Trout, RN, MSN

Where Does Palliative Care Fit In?

Ambereen Mehta, MD, MPH, FAAHPM

Multidisciplinary Support: Social Work

Julianne Meiser, MSS, LCSW

Beyond the Clinic Walls: Partnering with MDA to Reduce Burden on Patients and Providers

Brooke Smith, LMSW, MEd

Orange EFG

Care Trends CE Accredited

2:00 - 3:30 PM

Healthcare & Policy: Practicing Medicine in Turbulent Times

Chair– Joel Cartner, Esq.

Han Phan, MD

Chair - Leslie Delfiner, MD

Florida 1-3

3:30 – 4:00 PM

Exhibit Hall & Poster Sessions Open

Orlando Ballroom

3:30 – 3:55 PM

Networking Break

ALS

4:00 – 6:00 PM

Advancing ALS Therapeutics: Targets, Tools, and Trial Readiness

TDP43: From Disease Mechanisms To Biomarkers And Therapeutic Targets

Sami Barmada, MD, PhD

Impact Of Intrathecal Delivery Of INS1202 AAV9-SOD1-Shrna On Hallmarks Of Neurodegeneration In A Murine Disease Model Of ALS And A Patient-Derived In Vitro Model

Laura Ferraiuolo, PhD

Emerging ALS Therapeutics Targets Associated with TDP-43 Dysfunction

Evangelos Kiskinis, PhD

Modeling the Human Corticospinal Tract on a Chip

Chair – Nicholas Maragakis, MD

Healey ALS MyMatch: A Multicenter Early Phase Clinical Trial Initiative

Suma Babu, MBBS, MPH

Jinsy Andrews, MD

Orlando IV

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Monday, March 9, 2026 (cont.)

Disease Mechanism & Therapeutic Strategy

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|----------------|---|------------|
| 4:00 – 6:00 PM | <p>Muscle Regeneration and Repair</p> <p><i>Sarcolemma Resealing In Striated Muscle</i> Dan Michele, PhD</p> <p><i>Synthetic Polymers For Muscle Membrane Stabilization In DMD</i> Joseph Metzger, PhD</p> <p><i>Using novel protein therapeutics that target sarcolemmal membrane repair to treat Duchenne muscular dystrophy</i> Chair – Noah Weisleder, PhD</p> <p><i>Skeletal Muscle Regeneration From Pluripotent Stem Cell-Derived Myogenic Progenitors</i> Rita Perlingeiro, PhD</p> <p><i>Signaling Mechanisms Of Autophagy And Muscle Weakness In Duchenne Muscular Dystrophy</i> Jie Chen, PhD</p> <p><i>Beyond 2D: Utilizing Curi Bio's 3D Engineered Muscle Tissues for Predictive Disease Modeling</i> Nicholas Geisse, PhD</p> | Orange ABC |
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Neurology

CE Accredited

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| 4:00 – 6:00 PM | <p>Faculty and Fellows Interactive Neuromuscular Case Discussions Chairs – Bakri Elsheikh, MBBS, FRCP, FAAN & Katherine Mathews, MD</p> <p>Panelists – Eleni Drakou, MD Manny Mostofi, MD Lynn Daboul, MD</p> | Orange D |
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Allied Health

CE Accredited

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| 4:00 – 6:00 PM | <p>Gene Therapy: Impact on the Care Team – Goals & Roles Chair– Aravindhan Veerapandiyam, MD</p> <p><i>Gene Transfer Therapy: From the Nurse Coordinator's Perspective</i> Chair - Tancia Edmond, RN</p> <p><i>Translating Gene Therapy into Care: The Advanced Practice Provider's Role</i> Meganne Leach</p> <p><i>Social Work Role in the Duchenne Clinic</i> Daria Floyd, LMSW</p> <p><i>Role of Psychology in supporting Patients & Families Receiving Gene Therapy</i> Natalie Truba, PhD</p> | Orange EFG |
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Monday, March 9, 2026 (cont.)

Care Trends

CE Accredited

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| <p>4:00 – 6:00 PM</p> | <p>Technology & Innovation: Role in NMD Care</p> <p><i>Disruptive PROs: Quantifying Meaningful Patient Changes In Response to Therapeutic Intervention</i> Chair – Chad Heatwole, MD, MS-CI</p> <p><i>An Adjudication System for Expert Diagnosis</i> Jonathan Katz, MD</p> <p><i>Technology & Innovation: Role in Neuromuscular care</i> Tina Duong, MPT, PhD</p> <p><i>Digital Measures in Clinical Trials</i> Jamie Adams, MD</p> | <p>Florida 1-3</p> |
| <p>6:00 – 8:00 PM</p> | <p>Poster & Networking Reception</p> | <p>Orlando Ballroom</p> |

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Tuesday, March 10, 2026

| | | |
|--|---|---|
| 6:30 AM – 4:00 PM | Registration Open | Registration Desk in Main Foyer before escalators (lobby level) |
| 7:00 – 8:00 AM | Industry Forum Breakfast presented by BioMarin <i>Critical Evaluation Of Clinical Outcomes</i> | Key West ABCD |
| | Industry Forum Breakfast presented by Benitec Biopharma <i>Breakfast with Benitec: BB-301, Transforming the Treatment Landscape of Oculopharyngeal Muscular Dystrophy</i> | Key Largo ABCD |
| | Industry Forum Breakfast presented by BridgeBio <i>LGMD2I/R9 - Diagnostic Challenges and Novel Therapeutic Approaches</i> | Florida 5-7 |
| | Industry Forum Breakfast presented by Thermo Fisher Scientific <i>AI and the Future of Clinical Trial Design and Execution</i> | Orlando V-VI |
| | Industry Forum Breakfast presented by UCB <i>Advancing Care in Early-Onset TK2d: Translating Science into Treatment</i> | Florida 4 |
| Drug Development Considerations | | |
| 8:30 – 10:30 AM | Immune Response Considerations in Gene Therapy <i>Toward Safer AAV Gene Therapy: Mechanistic Immune Insights from Mouse Models and Patients</i> Melissa Spencer, PhD <i>Characterizing and Modulating Immune Responses to CRISPR Gene Therapies</i> Allison Keeler, PhD <i>Acute Toxicity Of High-Dose AAV Gene Therapy For DMD</i> Dongsheng Duan, PhD <i>Informing AAV Gene Therapy Point of Care Risk/Benefit Analysis Through Precision Diagnostics</i> Bradley Hamilton, PhD <i>Modulating Immunity to Enable Safe and More Effective Gene Therapy</i> Manuela Corti, PhD <i>Treg-Focused Strategies For Promoting Dystrophin Tolerance</i> Chair – Armando Villalta, PhD | Orlando IV |

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Tuesday, March 10, 2026 (cont.)

Disease Mechanism & Therapeutic Strategy

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|-----------------|--|------------|
| 8:30 – 10:30 AM | <p>Inherited Peripheral Neuropathies: Unique Challenges in Drug Developments <i>Unique Challenges In Drug Development And Clinical Trial Design</i> Chair – Brett Morrison, MD, PhD</p> <p><i>Clinical Outcome Measures And Biomarkers For CMT: Are We Ready For Clinical Trials?</i> Vera Fridman, MD</p> <p><i>Quantitative Muscle MRI Biomarkers In CMT Clinical Trials</i> Jasper Morrow, MBChB, PhD, FRACP</p> <p><i>Wearable Sensors To Measure Gait, Balance And Daily Activity In Adults With Charcot-Marie-Tooth Disease</i> Kayla Cornett, PhD</p> <p><i>CMT Clinical Trials: Current Landscape and Future Outlook</i> Joshua Burns, PhD</p> <p><i>Insights From Intrathecal IGHMBP2 Gene Delivery For IGHMBP2-Related Conditions</i> Megan Waldrop, MD</p> | Orange ABC |
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Allied Health CE Accredited

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| 8:30 – 10:30 AM | <p>Managing Diet & Exercise: In Clinic and Beyond Chair - Laura Watne, MS, RD, CSP</p> <p><i>Case Example: Weight Loss and Exercise in Duchenne Muscular Dystrophy</i> Chairs – Meghan Moore Burk, PhD, DPT, PT, NCS</p> <p><i>Managing Underweight in the Neuromuscular Population</i> Rose Jenkins</p> <p><i>Exercise</i> Tanja Taivassalo, PhD</p> <p><i>Measuring Exercise... Why it Matters</i> Tina Duong, MPT, PhD</p> <p><i>Considerations For Obesity Medication Use In Neuromuscular Disorders</i> Jaime Moore</p> | Orange EFG |
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Tuesday, March 10, 2026 (cont.)

| Neurology CE Accredited | | |
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| 8:30 – 9:30 AM | Emerging Care Strategies: Optimizing Therapies Chair – Aravindhyan Veerapandiyan, MD <i>Emerging Care Strategies: Optimizing Therapies in SMA</i> Arpita Lakhotia, MD Ezgi Saylam, MD | Orange D |
| 9:30 – 10:30 AM | Emerging Care Strategies: Early Intervention Chair – Brianna Brun, MD <i>DMD NBS Experience at Cincinnati Children's Hospital</i> Cuixia Tian, MD <i>Ethics of early diagnosis and early intervention</i> John Brandsema, MD | Orange D |
| 10:30 AM – 1:30 PM | Exhibit Hall & Poster Sessions Open | Orlando Ballroom |
| 10:30 – 10:55 AM | Networking Break | |
| Lab to Life | | |
| 11:00 AM – 12:00 PM | Titinopathies <i>Perspectives From A Titin Patient Advocate</i> Chair – Sarah Foye, OT <i>Decoding Titin: Practical Strategies for Tackling a Giant Variant Challenge</i> Marco Savarese, PhD <i>Optimizing Trial Readiness For Titinopathies: Understanding Full Clinical Spectrum, Natural History And Multidisciplinary Approach To Diagnosis</i> Anna Sarkozy, MD, PhD | Orlando IV |
| 11:00 AM – 12:00 PM | Therapeutic Landscape for Autoimmune NMDs <i>Abbreviated Short Introduction to the Immunology of Cell Therapy</i> Armando Villalta, PhD <i>Cell Therapy in Autoimmune Neuromuscular Disorders: Experience in Myasthenia Gravis and Myositis</i> Chair – Tahseen Mozaffar, MD <i>Clinical Trial Logistics and Safety Characterization of CAR-T Therapy in Autoimmune Neuromuscular Disorders</i> Ali Habib, MD | Orange ABC |
| 11:00 AM – 12:00 PM | Infantile/Pediatric Forms of Adult-Onset NMDs <i>Charcot-Marie-Tooth Disease And Related Neuropathies Of Childhood</i> Joshua Burns, PhD <i>Dysregulation Of Sphingolipid Metabolism In A Monogenic Form Of Juvenile ALS</i> Chair – Payam Mohassel, MD <i>Facioscapulohumeral Muscular Dystrophy Presenting In Childhood</i> Katherine Mathews, MD | Orange D |

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Tuesday, March 10, 2026 (cont.)

Lab to Life

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| 11:00 AM – 12:00 PM | <p>Limb-Girdle Muscular Dystrophies <i>Preparing For Clinical Trials In LGMD</i> Chair - Nicolas Johnson, MD, MSCI</p> <p><i>Systematic Variant Resolution in LGMDs</i> Conrad Weihl, MD, PhD</p> <p><i>Preclinical Development Of AAV Therapies For LGMD</i> Ellie Carrell, PhD</p> | Orange EFG |
| 11:00 AM – 12:00 PM | <p>Mitochondrial Myopathies <i>Mitochondrial Proteotoxic Stress: from Disease Mechanisms to Therapeutic Strategies</i> Chair – Giovanni Manfredi, MD, PhD</p> <p><i>Gene Editing in Mitochondrial Diseases</i> Carlos Moraes, PhD</p> <p><i>Turning the Oxygen and Vitamin Dials</i> Isha Jain, PhD</p> | Florida 1-3 |
| 12:00 – 1:30 PM | <p>Industry Forum Lunch presented by argenx <i>Emerging Therapeutic Targets In Idiopathic Inflammatory Myopathy (Myositis): A Role For Autoantibodies?</i></p> | Orlando V-VI |
| | <p>Industry Forum Lunch presented by Avidity Biosciences <i>Biomarker Advancements in Rare NMD: Insights from DMD and FSHD</i></p> | Key West ABCD |
| | <p>Industry Forum Lunch presented by Catalyst Pharmaceuticals <i>Vamorolone in DMD: Real-World Experience, Emerging Questions and the Path to Long-Term Evidence</i></p> | Key Largo ABCD |
| | <p>Industry Forum Lunch presented by ITF Therapeutics <i>Clinical Perspectives on HDAC Inhibition in Duchenne Muscular Dystrophy"</i></p> | Florida 5-7 |
| | <p>Industry Forum Lunch presented by Novartis <i>New Possibilities in SMA Treatment for Adult and Pediatric Patients</i></p> | Florida 4 |
| Drug Development Considerations | | |
| 2:00 – 4:00 PM | <p>Patient Registries Reimagined: Experience, Evidence, and Evolution Laura Rufibach, PhD Fernando Vieira, MD Smith Heavner Bhaskar Roy, MHS Chair – Andre Paredes, PhD Kathryn Bryant Knudson</p> | Orlando IV |

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Tuesday, March 10, 2026 (cont.)

Disease Mechanisms & Therapeutic Strategies

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|----------------|--|------------|
| 2:00 – 4:00 PM | <p>Inflammation & Immune Dysregulation in NMD</p> <p><i>Overview Of Innate And Adaptive Immune Response Pathways In Neuromuscular Diseases</i> Chair – Kanneboyina Nagaraju, PhD, DVM</p> <p><i>Inflammation in Dysferlinopathy: From Mismanagement to Mechanistic Insight</i> Jyoti Jaiswal, PhD</p> <p><i>Spatial Transcriptomics Reveals Mechanism Of Autoimmunity Driven By Internalized Autoantibodies</i> Iago Pinal Fernandez, MD, PhD</p> <p><i>Investigating Type 1 Interferon Effects in Myositis</i> Melissa Morales, PhD</p> <p><i>Implications Of Immune Dysfunction In Disease Progression And Therapeutic Development For Duchenne Muscular Dystrophy</i> James Novak, PhD</p> | Orange ABC |
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Neurology CE Accredited

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| 2:00 – 4:00 PM | <p>Gene Therapy: Safety, Monitoring, & Reporting Chair – Barry Byrne, MD, PhD</p> <p><i>Intrathecal AAV for Giant Axonal Neuropathy: Immunological Observations</i> Carsten Bönnemann, MD</p> <p><i>Immune responses to AAV and associated toxicities</i> Melissa Spencer, PhD</p> <p><i>AAV Gene Therapy: Hepatotoxicity Monitoring, And Risk Mitigation</i> Perry Shieh, MD, PhD</p> <p><i>Beyond the Infusion: Safety, Surveillance, and Stewardship in AAV Gene Therapy for NMD</i> Kathryn Wagner, MD, PhD</p> | Orange D |
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Allied Health CE Accredited

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| 2:00 – 4:00 PM | <p>Adaptive Equipment in Pediatric NMD: Offering Support through Transition</p> <p><i>Palliative Care: Supporting Patients Through Transition, Discussing Goals Of Care</i> Katie Maddox</p> <p>Tom Simon, ATP Chair – Kaitlin Batley, MD Melissa McIntyre, DPT</p> | Orange EFG |
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Tuesday, March 10, 2026 (cont.)

Care Trends CE Accredited

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| 2:00 – 4:00 PM | <p>Checking In: Asking the Right Questions to Assess Health & Well-Being Chair – Colette Gramszlo, PhD</p> <p><i>Pediatric Neuromuscular Disease: Emotional and Behavioral Health</i> Natalie Truba, PhD</p> <p><i>Checking In During Puberty: Sexual and Reproductive Health Conversations in Youth with Neuromuscular Conditions</i> Nora Renthal, MD, PhD</p> <p><i>How to have Gutsy Conversations: Communication Strategies for GI Health in Pediatric Muscular Dystrophy</i> Jennie David, PhD</p> | Florida 1-3 |
| 4:00 – 4:25 PM | Exhibit Hall & Poster Sessions Open | Orlando Ballroom |
| 4:00 – 4:25 PM | Networking Break | Orlando Ballroom |

Drug Development Considerations

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|----------------|---|------------|
| 4:30 – 6:00 PM | <p>Beyond the Science: Navigating Today's Drug Development Climate Chair - Paul Melmeyer</p> <p>Michael Rice Sarah Wicks Karin Hoelzer Jennifer Farmer</p> | Orlando IV |
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Disease Mechanisms & Therapeutic Strategies

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|----------------|---|------------|
| 4:30 – 6:00 PM | <p>Reimagining Delivery: Non-Viral Approaches in Gene Therapy</p> <p><i>SORT LNPs Encapsulating Cas9 Mrna Achieve Efficient Editing In Skeletal Muscle In A Dystrophic Mouse Model</i> Chair – Scot Wolfe, PhD</p> <p><i>Discovery and Development of Muscle-Directed Lipid Nanoparticles</i> Mark Herbert, MSc</p> <p><i>Breaking the Size Barrier: Redosable Delivery Of Full-Length Dystrophin Using The Fusogenix PLV Platform</i> John Lewis, PhD</p> <p><i>Non-Viral Gene Replacement Therapy for DMD Utilizing RIPPLE Ultrasound Mediated Delivery of a Transgene Expressing Full-Length Human Dystrophin</i> Ivan Krivega, PhD</p> | Orange ABC |
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Tuesday, March 10, 2026 (cont.)

Neurology

CE Accredited

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| 4:30 – 6:00 PM | <p>Office Hours: Navigating the Business of Care</p> <p><i>Institutional and Procurement Considerations</i> Chair – Crystal Proud, MD</p> <p><i>Strategic Guide to Insurance Denials</i> Omer Abdul-Hamid, MD</p> <p><i>The Business of Genetics in NMD</i> Jordan Bontrager, MS, CGC</p> | Orange D |
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Allied Health

CE Accredited

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| 4:30 – 6:00 PM | <p>Recreation: Making Exercise Fun!</p> <p>Chair – Diane Murrell, MSW, LCSW</p> <p><i>The Power of Possible: Unlocking Outdoor Adventures Introduction of Southern Outdoor Dreams</i> Duke Damron</p> <p><i>Exercise, Adaptive Sports & Recreation in our MDA Population: Limitations and Opportunities</i> Cosmo Kwok, MD</p> | Orange EFG |
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Care Trends

CE Accredited

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| 4:30 – 6:00 PM | <p>ALS: Optimizing Care and Support</p> <p><i>Delivering the News No One Wants to Hear</i> Elyse Everett, MD, MOT</p> <p><i>Holistic Approaches To Mental Health Care In ALS</i> Chair - Kelly McCoy Gross, RN</p> <p><i>Dying with Dignity</i> Cara Siegel, MD</p> | Florida 1-3 |
|----------------|--|-------------|

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

Orlando Ballroom

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME
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Wednesday, March 11, 2026

| | | |
|--------------------|--|---|
| 8:00 AM – 11:00 AM | Registration Open | Registration Desk in Main Foyer before escalators (lobby level) |
| 8:30 AM – 3:00 PM | <p align="center">New, Novel, and Noteworthy: NMD Highlights</p> <p align="center">We can't wait to reconvene in 2026 and highlight the most impactful abstract submissions during our final day oral presentations. You won't want to miss it!</p> | |
| 8:00 AM – 3:00 PM | Clinical Trial Updates | Florida 4 |
| 8:00 – 8:15 AM | <p>Phase 1 Interim Results Evaluating the Safety, Tolerability, Pharmacokinetics, and Exploratory Efficacy of Salanersen for Spinal Muscular Atrophy Diana Castro, MD</p> | |
| 8:15 – 8:30 AM | <p>DEVOTE Part C and ONWARD Integrated Results: Higher Doses of Nusinersen in Nusinersen-Experienced Participants with Spinal Muscular Atrophy (SMA) Nancy Kuntz, MD</p> | |
| 8:30 – 8:45 AM | <p>Efficacy and Safety of Efgartigimod Ph20 Sc in Adult Participants with Active Idiopathic Inflammatory Myopathy: Phase 2 Results from the ALKIVIA Study Nicholas Streicher, MD</p> | |
| 8:45 - 9:00 AM | <p>Efficacy and Safety of Descartes-08 in AChR+ generalized myasthenia gravis: subgroup analysis of a phase 2b randomized placebo-controlled trial Tahseen Mozaffar, MD</p> | |
| 9:00 – 9:15 AM | <p>Ignaseclant (NMD670) for Generalized Myasthenia Gravis - the SYNAPSE-MG study status update Thomas Holm Pedersen, PhD</p> | |
| 9:15 - 9:30 AM | <p>FREEDOM-DM1: Final results from a Phase 1, placebo-controlled SAD study to evaluate PGN-EDODM1 in people with myotonic dystrophy type 1 (DM1) Johanna Hamel, MD</p> | |
| 9:30 – 9:45 AM | <p>A Global Phase 3 Trial Assessing the Efficacy and Safety of Z-basivarsen in Myotonic Dystrophy Type 1 Douglas Kerr, MD, PhD, MBA</p> | |
| 9:45 – 10:00 AM | <p>Interim observations from a long-term open label study evaluating daily nomlabofusp administration in patients with Friedreich's ataxia Russell Clayton, DO</p> | |
| 10:00 – 10:15 AM | <p>Update on FORTIS: A phase 1/2 open-label clinical trial on AT845 gene replacement therapy for late-onset Pompe disease Tahseen Mozaffar, MD</p> | |
| 10:15 – 10:30 AM | BREAK | |
| 10:30 – 10:45 AM | <p>Initial Findings From The Phase I LUMINA Trial Of AMX0114 In Amyotrophic Lateral Sclerosis Lauren Kett</p> | |
| 10:45 – 11:00 AM | <p>Prevails: A Phase 3, Randomized, Double-Blind, Placebo-Controlled Study To Evaluate The Efficacy And Safety Of Pridopidine In Participants With ALS Angela Genge, MD, FRCP</p> | |
| 11:00 – 11:15 AM | <p>A First-In-Human Dose-Escalation Study Of Intrathecal INS1202 Gene Therapy In Adults With Amyotrophic Lateral Sclerosis (ALS) Mark Stahl, MD, PhD</p> | |
| 11:15 – 11:30 AM | <p>An Early Feasibility Study Of Multi-Site Direct Current Stimulation Treatment Of People With ALS Using The Myoregulator® Device Nader Yaghoubi, MD, PhD</p> | |
| 11:30 – 11:45 AM | <p>A Phase 1a/B Open Label Study Of SAT-3247 In Healthy Volunteers And Adult Patients With Duchenne Muscular Dystrophy Wildon Farwell, MD</p> | |

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ROOM LOCATION SUBJECT TO CHANGE

Wednesday, March 11, 2026 (cont.)

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| 11:45 AM – 12:00 PM | RGX-202, An Investigational Gene Therapy For The Treatment Of Duchenne Muscular Dystrophy: Interim Phase I/II Clinical Data Caolina Tesi-Rocha, MD | |
| 12:00 – 12:15 PM | Initial Results From An Open-Label Phase 1/2 Study Of BMN 351, An Antisense Oligonucleotide For Exon 51-Skip Amenable Duchenne Muscular Dystrophy Erik Niks, MD, PhD | |
| 12:15 – 12:30 PM | Development And First-In-Human Experience With LE051: An RNA Editing Drug For Duchenne Muscular Dystrophy Pengfei Yuan, PhD | |
| 12:30 – 12:45 PM | Del-Zota Treatment Is Associated With Near Normalization Of CK Levels And Improvements In Key Functional Outcomes At 1 Year In Participants With DMD44 Craig McDonald, MD | |
| 12:45 – 1:00 PM | Zeleciment Rostudirsen Significantly Increased Dystrophin Protein Levels And Led To Functional Improvement In Clinical Measures In The DELIVER Trial Kevin Flanigan, MD | |
| 1:00 – 1:15 PM | Update On The INSPIRE DUCHENNE Phase 1/2 Study Of The Next-Generation Microdystrophin Gene Therapy Candidate SGT-003 For Duchenne Muscular Dystrophy Aravindhan Veerapandiyam, MD | |
| 1:15 – 1:30 PM | 48-Week Data From The Phase 2 Open-Label FORWARD-53 Study Of WVE-N531 In Boys With Duchenne Muscular Dystrophy Amenable To Exon 53 Skipping Li-Jung Tai, MD, PhD | |
| 1:30 – 1:45 PM | Givinostat Weight-Based Flexible Dosing: Rationale And Efficacy At The Different Doses Aravindhan Veerapandiyam, MD | |
| 1:45 – 2:00 PM | GNT0004 is an AAV-8 based gene therapy for treating DMD, containing gene coding for a shortened functional dystrophin (hMD1) with a Spc5.12 promoter, targeting skeletal and cardiac muscles. Francesco Muntoni, MD | |
| 2:00 – 3:00 PM | Late-Breakers | Florida 4 |
| 2:00 – 2:15 PM | Interim Analysis from Ongoing Phase 3 FORTIFY Study of BBP-418 for Patients with Limb Girdle Muscular Dystrophy 2I/R9 Meets Efficacy Endpoints Katherine Mathews, MD | |
| 2:15 – 2:30 PM | Efficacy and Safety of ignaseclant in Adults with Charcot-Marie-Tooth Disease: Top Line Results of a Phase 2a Study W. David Arnold, MD | |
| 2:30 – 2:45 PM | Delandistrogene Moxeparvovec in Duchenne Muscular Dystrophy: Functional and Safety Outcomes up to 3 Years Post-Infusion in the EMBARK Study Craig McDonald, MD | |
| 2:45 – 3:00 PM | Confirmation of musculoskeletal and cardiac benefit in DMD from deramiocel, an allogeneic cell therapy, in the Phase 3 HOPE-3 study Craig McDonald, MD | |
| 8:00 am - 12:45 PM | Novel Therapeutic Approaches | Florida 1-3 |
| 8:00 – 8:15 AM | <i>Novel Gene Therapies For DMD Cardiomyopathy</i> David Hammers, PhD | |
| 8:15 – 8:30 AM | <i>Trna Therapeutics Restore Full-Length Proteins In Muscle Stop Codon Disease</i> Stephen Eichhorn, PhD | |
| 8:30 – 8:45 AM | <i>Delivery Of Antisense Oligonucleotides To Satellite Cells In Preclinical Models Of Duchenne Muscular Dystrophy</i> Mahasweta Girgenrath, PhD | |

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME
ROOM LOCATION SUBJECT TO CHANGE

Wednesday, March 11, 2026 (cont.)

| | | |
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| 8:45 - 9:00 AM | Evaluation Of Early Administration Of AAV9-Midlength-Dystrophin In The Mdx Mouse Model Of Duchenne Muscular Dystrophy Gretchen Thomsen, PhD | |
| 9:00 – 9:15 AM | (Z)-Endoxifen Restores Muscle Performance And Lowers Damage Biomarkers In Mdx5cv Dystrophic Mice Sandra Hammer, PhD | |
| 9:15 - 9:30 AM | Targeting DUX4 By Myocyte-Selective Sirna Conjugate To Treat FSHD David Strohlic, PhD | |
| 9:30 – 9:45 AM | Assessing The Impact Of SAT-3247 On Muscle Force In A Mouse Model Of FSHD Philip Lambert, PhD | |
| 9:45 – 10:00 AM | Tgfb Inhibitors And L-Type Calcium Channel Blockers Are Potential Drug Repurposing Candidates For LMNA-MD Nathaniel Mohar, BS | |
| 10:00 – 10:15 AM | CRISPR/Cas-9 Mediated Disruption Of The Mutant Allele Ameliorates Susceptibility To Weakness In Murine Models Of Periodic Paralysis Stephen Cannon, MD, PhD | |
| 10:15 – 10:30 AM | Break | |
| 10:30 – 10:45 AM | Efficacy, Biodistribution, And Safety Of An AAV Gene Therapy In XLMTM Disease Model Mice And Juvenile Non-Human Primates Takeshi Sato, DVM, PhD | |
| 10:45 – 11:00 AM | PX578: A First-In-Class POLG Activator To Increase Mtdna For Treatment Of Mitochondrial DNA Depletion Syndromes Juli Jones, PhD | |
| 11:00 – 11:15 AM | Acute Vision Loss Linked To Novel Biallelic FDX2 Loss-Of-Function Variants: Insights Into Biochemical Mechanisms And Therapeutic Implications Rotem Orbach, MD | |
| 11:15 – 11:30 AM | DYNE-401 Demonstrates Potential To Address Pompe Disease With Low And Infrequent Dosing Tyler Picariello, PhD | |
| 11:30 – 11:45 AM | A High-Throughput Assay For Measuring Ribitol Response Across FKRPs Variants Yujiao Yang, PhD | |
| 11:45 AM – 12:00 PM | A Novel Agent For Preventing Fatty Degeneration Of Muscles In LGMD2B Monique Floer, PhD | |
| 12:00 – 12:15 PM | Development Of An Orally Bioavailable GCPII Inhibitor To Promote Remyelination After Peripheral Nerve Injury Yu Su, MD, PhD | |
| 12:15 – 12:30 PM | A Dual Cargo Aav9 Vector Reduces Mutant Nefl With Wild Type Human Nf-L Protein And Improves Cmt2e Disease Pathology Dennis Perez-Lopez, PhD | |
| 12:30 – 12:45 PM | N-Of-1 Personalized Therapy For CMT2S: From Patient-Specific Preclinical Models To First-In-Human Dosing Sandra Smieszek, PhD | |
| 8:00 AM -12: 45 PM | Molecular Insights | Florida 5-7 |
| 8:00 – 8:15 AM | Plasma Proteome Alterations Define Disease Associated Pathways In VCP-Associated Multisystem Proteinopathy Devesh Pant, PhD | |
| 8:15 – 8:30 AM | Alsin Loss Of Function Impairs Rac1 Signaling And Endosomal Trafficking In ALS2 Ayse Yesbek Kaymaz, PhD | |

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Wednesday, March 11, 2026 (cont.)

| | |
|---------------------|---|
| 8:30 – 8:45 AM | A Novel Mechanism Of Kinase Regulation At The Neuromuscular Junction Jakob Proemer, BSc, MSc |
| 8:45 - 9:00 AM | SMN Deficiency Disrupts Lineage Specification In Neuromesodermal Progenitors Revealed By A Neuromuscular Organoid Model Of Spinal Muscular Atrophy Zeynep Dokuzluoglu |
| 9:00 – 9:15 AM | Profibrotic Factors And Extracellular Matrix Remodeling Associated With Muscle Fibrosis In Myotonic Dystrophy Type 1 Preeti Kumari, PhD |
| 9:15 - 9:30 AM | Unveiling The Inflammatory Landscape Of Myotonic Dystrophy Type 1 (DM1): From Systemic Immune Signals To Tissue Architecture Marwa Zafarullah, PhD |
| 9:30 – 9:45 AM | Mitochondrial Fusion Dynamics Adaptations To Metabolic Stress Are Under Opposing Effects Of The Mtcu Gatekeepers, MICU1 And MICU2 Benjamin Cartes-Saavedra, PhD, MSc |
| 9:45 – 10:00 AM | Activation Of Circadian Clock Rescues The Sarcolemma Defect And Promotes Regenerative Capacity To Ameliorate Muscular Dystrophy Ke Ma, MD, PhD |
| 10:00 – 10:30 AM | Break |
| 10:30 – 10:45 AM | Targeting Resolution Of Inflammation To Enhance Skeletal Muscle Regenerative Capacity In Duchenne Muscular Dystrophy Sarah Tiufekchiev-Grieco |
| 10:45 – 11:00 AM | A Novel DMD Conditional Mouse Reveals Essential Roles For Dystrophin In Skeletal Muscle And Brain Matthew Alexander, PhD |
| 11:00 – 11:15 AM | Rat Duchenne Muscular Dystrophy Models For Preclinical Studies And Deciphering Tissue Repair Mechanisms Frederic Relaix |
| 11:15 – 11:30 AM | Sphingolipid And Microrna Signaling Contribute To Innate Immune Activation In Dystrophic Muscle Alyson Fiorillo, PhD |
| 11:30 – 11:45 AM | Loss Of LAMA2 Impairs Peripheral Nerve Myelination Through Perturbation Of Calcium Signaling In LAMA2-Related Muscular Dystrophy John Wang, MD, PhD |
| 11:45 AM – 12:00 PM | Fbxo30/MUSA1 Is A Novel Critical Regulatory Element For Z-Line Homeostasis And Skeletal Muscle Function Martina Esposito |
| 12:00 – 12:15 PM | High-Throughput Functional Mapping Of Sarcoglycan Variants: A Pre-Clinical Framework For Improving Limb-Girdle Muscular Dystrophy Diagnosis Shushu Huang, MD, PhD |
| 12:15 – 12:30 PM | Loss Of Calpain-3 And Exercise Disrupt Junctional Sarcoplasmic Reticulum Proteins In Skeletal Muscle Revealed By Proteomics Elizabeth Barton, PhD |
| 12:30 – 12:45 PM | Lysosomal Signaling Meets Glucose Homeostasis: A Glycogen Accumulation Independent Perspective In The Fight Of Pompe Disease Andrea Armani, PhD |