MDA CLINICAL & SCIENTIFIC CONFERENCE 2025

Conference Agenda

ALL CONFERENCE SESSION TIMES LISTED ARE IN CENTRAL TIME
*SPEAKERS LISTED IN ORDER OF PRESENTATION

Saturday, March 15, 2025		
12:00 – 6:00 PM	Registration Open for Care Center Directors	Geppetto Foyer, Tower Side
12:00 – 6:00 PM	Registration Open for Large Exhibit Booth Sponsors	Trinity Exhibit Hall
5:00 – 7:00 PM	MDA Care Center Network Happy Hour	Gossip Bar

Sunday, March 16, 2025		
7:00 AM – 7:00 PM	Registration Open	Geppetto Foyer, Tower Side
7:30 AM – 3:30 PM	MDA Care Center Directors Meeting (Invitation Only)	Chantilly East
7:30 AM – 3:30 PM	Allied Health Workshop *Shifting Care for Shifting Needs: Coordinating Care and Increasing Quality of Life via Assistive Devices*	Chantilly Werst
1:00 – 5:00 PM	Trainee Networking Session	Wedgewood Ballroom
2:00 – 5:00 PM	Neuromuscular Advocacy Collaborative Meeting (Invitation Only)	Monet
6:00 – 8:00 PM	Welcome Reception & Posters in Exhibit Hall	Trinity Exhibit Hall

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Monday, March 17, 2025		
6:30 AM – 6:00 PM	Registration Open	Geppetto Foyer, Tower Side
7:00 – 8:00 AM	**Industry Forum Breakfast presented by Amicus** *Moving Forward Together: Discovering a Treatment Approach for Late-Onset Pompe Disease*	Grand ABC
	Industry Forum Breakfast presented by argenx *Developments in CIDP: Pathophysiology, Disease Burden, and Clinical Updates from the ADHERE/ADHERE+ Trials Investigating FcRn Inhibition*	Grand DE
	Industry Forum Breakfast presented by Avidity Biosciences *Advancing RNA Therapeutics: Exploring Antibody Oligonucleotide Conjugates (AOCs) for Rare Neuromuscular Diseases*	Chantilly East
	Industry Forum Breakfast presented by ITF Therapeutics *Learn about the Role of an HDAC inhibitor in the Treatment of Duchenne Muscular Dystrophy*	Chantilly West
8:30 – 9:50 AM	**Opening Remarks** Donald S. Wood, PhD President and CEO of the Muscular Dystrophy Association **MDA Legacy Award** Katherine Mathews, MD **MDA Community Impact in Research Award** Donavon Decker	Trinity Ballroom
9:50 – 10:20 AM	Networking Break in Exhibit Hall	Trinity Exhibit Hall
10:20 AM – 12:00 PM	**MDA Research: Exploring the Past, Innovating the Present, Shaping the Future** **Keynote Address** **Panel Discussion on NMD**	Trinity Ballroom
12:00 – 1:30 PM	**Industry Forum Lunch presented by Biogen** *Advancing Care for Friedreich Ataxia: Clinical Insights and Patient Cases with the First Approved Treatment*	Chantilly West
	Industry Forum Lunch presented by Novartis *Long-Term Impact of Gene Therapy: Lessons from the SMA Community*	Grand ABC
	Industry Forum Lunch presented by PPD, part of Thermo Fisher Scientific *The Evolution of Neuromuscular Trials: Leveraging the Challenges and Successes of the Past to Forge a Promising Future*	Grand DE
	Industry Forum Lunch presented by Sarepta *From Assessment to Action: A Holistic Approach to Optimizing Neuromuscular Disease Care*	Chantilly East
	Industry Forum Lunch presented by UCB *The Diagnostic Dilemma: Genetic Testing vs Muscle Biopsy in Mitochondrial Myopathies*	Wedgewood Ballroom

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Monday, March 17, 2025 cont.		
Allied Health Track		
2:00 – 3:30 PM	**The Latest in Pulmonary and Cardiac Management in Neuromuscular Disease** ***(CE Accredited)*** *Applying Cardiac Guidelines to the Neuromuscular Patient* Co-Chair – Elizabeth McNally, MD, PhD *Managing Cardiac Care Before, During and After Gene Therapy* Beth Kaufman, MD *Natural History of Pulmonary Care: How Do Lifetime Events Impact Breathing Function?* Co-Chair - Bethany Lussier, MD *Pivotal Decision Points: Case Studies to Guide Decisions in Pulmonary Care* Jeanette Brown, MD, PhD	Cortez AB
Disease Mechanism Track		
2:00 – 3:30 PM	**Neural Regeneration – The Next Frontier** Chair – Kuldip Dave, PhD *Is ALS a Recoverable Disease? Role of Neural Repair in the CNS and PNS* Brett Morrison, MD, PhD *Neural Regeneration from the SCI Perspective* Binhai Zheng, PhD *Glial Cells and Neural Plasticity: Partners in Neuroprotection and Repair* Isobel Scarisbrick, PhD *A Novel Approach to Slowing ALS Disease Progression Using Non-Invasive Multi-site DCS* Nader Yaghoubi, MD, PhD	Coronado BCD
Care Management Track		
2:00 – 3:30 PM	**Preparing for Tomorrow's Leaders & Fostering diversity Among Providers** ***(CE Accredited)*** *What Can We Learn or Model from Other Fields* Chair – Diana Castro, MD *NMD Care: What Can We Learn from Other Fields. Successful Transition from Pediatric to Adult Congenital Heart Disease: It Takes More Than a Village* Kan Hor, MD *Optimizing Comprehensive Care in Neuromuscular Disease - Learning from Other Models* Oscar 'Hank' Mayer, MD	Cortez CD

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Monday, March 17, 2025, cont.		
Diagnostic Rolodex Track		
2:00 - 3:30 PM	**Myasthenia Gravis: Presentation and Diagnosis; Current Therapies; and Current & Future Research** ***(CE Accredited)*** *Myasthenia Gravis: Presentation and Diagnosis; Current Therapies; and Current and Future Research* Chair – Sarah Heintzman, MS, APRN-CNP, FNP-C, CCRC *Myasthenia Gravis: Presentation and Diagnosis; Current Therapies; and Current and Future Research* Mary Petrulis, MD *Myasthenia Gravis Current Therapies* Neelam Goyal, MD *Myasthenia Gravis: Current and Future Research* Ali Habib, MD	Coronado A
Catalyzing Innovation: Strategies for Streamlining Drug Development Track		
2:00 - 3:30 PM	**Innovative Research Programs: Filling Gaps in Therapeutic Development** Chair – Laury Mignon, PhD *Project PaLaDIn – Enhancing the Patient Voice in NMD* David Allison *Treat NMD Advisory Committee for Therapeutics* Lindsay Alfano, PT, DPT, PCS *IAMRARE Research Program: Advancing Patient-Centered Research* Janine Lewis Angela Lek *AFM-Telethon, A Non-Profit Association Developing Innovation for Therapeutic Applications* Jean Francois Braind *Next Generation Trial Design Today: Collaborative Data Science to Unlock Better Drugs to Patients Sooner* Susan Ward	De Soto AB
3:30 – 3:55 PM	Networking Break in Exhibit Hall	Trinity Exhibit Hall

Monday, March 17, 2025, cont.		
Allied Health Track		
4:00 – 6:00 PM	**Optimizing Nutrition and Physical Therapy in Neuromuscular Disease: Practical Applications for Patient Care** *(CE Accredited)* Chair – Umme Salma Vahanvaty, MS, RD, CSP *Nutrition Focused Physical Exam in Patients with Neuromuscular Disease* Alicia Gilmore, MS, RD, CSO, LD *Physical Therapy in Neuromuscular Disorders: Evidence-Based Exercise & Care Recommendations* Constance de Monts, PT, DPT *Current Trends and Challenges in Pediatric Obesity* Ahlee Kim, MD *Constipation in Patients with Neuromuscular Disease* Shruti Nabar, MD	Cortez AB
Disease Mechanism Track		
4:00 – 6:00 PM	**Muscle Regeneration in Diseased Muscles: How Close Are We?** *First Clinical Trial of Myopaxon, An Allogeneic Off-The-Shelf Ipsc-Derived Myogenic Progenitor Cell Product for DMD* Chair – Rita Perlingeiro, PhD *Ex Vivo Gene Therapy for DMD: Safety, Efficacy and Affordability* Giulio Cossu, MD, FMedSci, FEAS *Primary Human Muscle Stem Cells as ATMP In Treatment of Muscular Dystrophies* Simone Spuler, MD *Cell-Based Approaches for Treatment of Muscle Diseases: Lessons from Gene Therapy* Carl Morris, PhD *Improving Regeneration in Aged and Dystrophic Muscle: The Role of Senescent Cells* Pura Muñoz-Cánoves, PhD *Imaging Cell and Gene Therapies* Glenn Walter, PhD	Coronado BCD

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Monday, March 17, 2025, cont.		
Care Management Track		
4:00 – 6:00 PM	**Developing a Sustainable Multidisciplinary Team Model** ***(CE Accredited)*** *Developing a Sustainable Business Model and Leveraging/Securing Institutional Resources to Make a Robust Multidisciplinary Care Team Available* Chair – Erika Finanger, MD, MS *Developing a Sustainable Multidisciplinary Team Mode* Kaitlin Batley, MD Carolyn Kelley Aravindhan Veerapandiyan, MD	Cortez CD
Diagnostic Rolodex Track		
4:00 – 6:00 PM	**Myositis: Best Practices & Updates** *Classification of Idiopathic Inflammatory Myopathies (IIM)* Chair – Tahseen Mozaffar, MD *Role of Pathology in IIM* Suur Biliciler, MD *Role of Imaging in IIM* Namita Goyal, MD *Inclusion Body Myositis: What is New?* Bhaskar Roy, MBBS, MHS	Coronado A
Catalyzing Innovation: Strategies for Streamlining Drug Development Track		
4:00 – 5:00 PM	**Lessons Learned from MOVR Data** Aravindhan Veerapandiyan, MD *A Reflection on Motor Neuron Disease Based on the MOVR Database* Bhaskar Roy, MD *Current State of Cardiac Therapies in Duchenne Muscular Dystrophy* DeWayne Townsend, DVM/Phd Chair – Andre Paredes, PhD	De Soto AB
5:00 – 6:00 PM	MOVR Workshop (**Invitation Only**)	De Soto AB
6:00 – 8:00 PM	Poster & Networking Reception	Trinity Exhibit Hall
8:00 – 10:30 PM	MDA 75th Celebration	Trinity Ballroom

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Tuesday, March 18, 2025		
6:30 AM – 6:00 PM	Registration Open	Geppetto Foyer, Tower Side
7:00 – 8:00 AM	**Industry Forum Breakfast presented by Astellas** *Where Are We with Gene Therapies for Rare Neuromuscular Disorders?*	Chantilly West
	Industry Forum Breakfast presented by Biogen *Pioneering Pathways: Evolving Use of Neurofilament in Neurodegenerative Disease*	Grand DE
	Industry Forum Breakfast presented by Catalyst *Navigating DMD Treatment: Integrating Data with Patient Stories*	Grand ABC
	Industry Forum Breakfast presented by The France Foundation *New Frontiers in the Treatment of DMD: Across the Age Spectrum*	Chantilly East
ALS/FTD Track		
8:30 – 9:30 AM	**Leveraging Genetics for ALS Therapeutics** *Clarifying the ALS Mutome* Chair – Matt Harms, MD *Harnessing Genetic Data to Guide Drug Repurposing in Motor Neuron Diseases* Sara Saez Atienzar, MSc, PhD *Progress Update for ALS Compute: A Central Repository for Harmonized WGS Data* John Landers, PhD *Role of Mutant SPTLC1 in Juvenile ALS* Devesh Pant, PhD	Coronado A
9:30 – 10:30 AM	**Role of Glial Cells in Disease Pathogenesis** Chair – Rita Sattler, PhD *Identification of a Neuroprotective Microglial State in C9ORF72 ALS/FTD* Justin Ichida, PhD *How Astrocytes Harm Human Motor Units In ALS: a Dual Mechanism of Action* Ludo Van den Bosch, PhD *Activation of Inflammatory Pathways in ALS* Allison Ebert, PhD *Contribution of Astrocytic Sparcl1 to Cortical Synaptic Dysfunction in C9ORF72-FTD/ALS* Robert Culibrk, PhD	Coronado A

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Tuesday, March 18, 2025 cont.		
	Disease Mechanism Track	
8:30 – 9:30 AM	**Approaches to Delivering/Restoration of Large-Sized Genes** Chair – Jeffrey Chamberlain, PhD *SIMPLI-GT: a Novel Gene Therapy Method to Deliver and Express Large Proteins* Hichem Tasfaout, PhD, PharmD *Recent Advances in Base Editing and Nonviral Delivery for DMD Correction* Yu-Chung (Andy) Pien *Engineered Lentiviral Vectors for Skeletal Muscle Targeting* Doug Millay, PhD *The Potential of tRNA Therapeutics to Restore Full-Length Proteins for Muscle Stop Codon Disease* Stephen Eichhorn, PhD	Coronado BCD
9:30 – 10:30 AM	**Targeting Signaling Pathways to Treat Muscular Dystrophies** *Preclinical Studies on the Modulation of Signaling Pathways as a Therapeutic Approach for Muscular Dystrophies* Chair – Matthew Alexander, PhD Steve Welc, PhD *Development of Anti-LTBP4 Stabilizing Antibodies for the Treatment of Muscular Dystrophy* Alexis Demonbreun, PhD *CRISPR-Engineered Mutations and MERFISH Single Cell Spatial Transcriptomics Reveal Key Processes of FSHD Pathogenesis* Kyoko Yokomori, Phd, DVM *FGF21-Mediated Muscle/Bone Interactions in Duchenne Muscular Dystrophy* Hongshuai Li, MD, PhD	Coronado BCD

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Tuesday, March 18, 2025 cont.		
Care Management Track		
8:30 – 9:30 AM	**Navigating Transition: Strategies for Care Across Settings and Life Stages** ***(CE Accredited)*** Chair – Yaacov Anziska, MD *Going from Pediatric to Adult Health Care When Living with Muscular Dystrophy* Tyus Hill *Supporting Patients in the Transition to Adulthood* Jodi Wolff, PhD, MSSW *Treating New Adults with Chronic Diseases* Christina Trout, RN, MSN The Role of Adult Multidisciplinary Clinics in Transitioning Neuromuscular Care Bakri Elsheikh, MBBS, FRCP, FAAN *Transition to Adult Care: More Important Now Than Ever* Lauren Elman, MD	Cortez CD
9:30 – 10:30 AM	**Building Bridges: Community Partnerships for Comprehensive Multidisciplinary Care** ***(CE Accredited)*** *Community Collaboration: Collaborating with Providers in the Community to Provide Multidisciplinary Care and Bridging Unmet Needs* Chair – Susan Apkon, MD Mark Terrelonge, MD, MPH Elizabeth 'Lynne' Wood, MD	Cortez CD

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Tuesday, March 18, 2025 cont.		
Genetic Medicine Track		
8:30 – 9:30 AM	**Gene Therapy Updates - Where Are We Today?** ***(CE Accredited)*** *Genetic Medicine: Gene Therapy Updates* Chair – John Brandsema, MD *Gene Therapy in the Clinic in 2024: Common Themes Across Neuromuscular Disorders and Other Therapeutic Areas* Susan Matesanz, MD *Treating an Adult with Gene Therapy: They Are Not "Big Children"!* Emma Ciafaloni, MD, FAAN *Emerging Therapies: The Research Pipeline and the Potential for Two Gene Therapies for the Same Disease* Diana Bharucha-Goebel, MD	Cortez AB
9:30 – 10:30 AM	**Experiences in DMD – Panel Discussion** ***(CE Accredited)*** Chair – Barry J. Byrne, MD, PhD Julie Parsons, MD Kevin Flanigan, MD Carmen Leon Astudillo, MD Mindy Cameron Aravindhan Veerapandiyan, MD	Cortez AB
8:30 – 10:30 AM	**Pompe Disease: Mechanisms, Therapeutic Advances, Advocacy, and Integrated Care Approaches (*Session in Partnership with TREAT-NMD)*** ***(CE Accredited)*** *Historical Learnings from Pre-ERT to Current ERT Landscape in Pompe Disease* Chair - Priya Kishnani, MD *Understanding Molecular Challenges and Opportunities in Pompe Disease* Catherine Rehder, PhD *Pompe Disease: Next Generation Therapies, and New Treatments on the Horizon* Mark Roberts *Role of Biomarkers in Disease Monitoring* Ferdinand Knieling *Multidisciplinary Approach and Rehab Services for Pompe Disease* Tracy Boggs, PT, MPT, NCS *Diagnostic Odessey and Living with Pompe: A Perspective from Pompe Patients* Tiffany House, JD *Diagnostic Odyssey and Living with Pompe: A Perspective from Pompe Patients* Amanda Joost	De Soto AB

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Tuesday, March 18, 2025 cont.		
10:30 – 10:55 AM	Networking Break in Exhibit Hall	Trinity Exhibit Hall
	Genetic Medicine Track	
11:00 AM – 12:00 PM	**New Genetic Technologies in Diagnosis and Treatment of Neuromuscular Disease** ***(CE Accredited)*** *New Genetic Technologies in Diagnosis and Treatment of Neuromuscular Disease* Chair – Kelly Minks, MS CGC Jordan Bontrager, MS CGC *It's Obviously FSHD! Or is it?* Natalie Katz, MD, PhD *Long-Read and RNA Sequencing: Case Examples* Alayne Meyer, MS, CGC	Cortez AB
	Lab to Life Track	
11:00 AM – 12:00 PM	**Non-Muscle Organ Systems in Myotonic Dystrophy** *A Model of DM GI Dysfunction in Mice* Chair – Thomas Cooper, MD *Altered Drug Metabolism and Increased Susceptibility to Fatty Liver Disease in Myotonic Dystrophy* Auinash Kalsotra, PhD Pradeep Mammen, MD *Mechanisms of CNS Dysfunction in Myotonic Dystrophy* Eric Wang, PhD	Coronado BCD
	Scientific Progress and Community Insights into Rare Myopathies *Biology and approaches to treatment of ADSS1 myopathy* Chair – Alan Beggs, PhD *Patient voice: ADSS1 Myopathy* Priyanka Kakkar & Naveen Baweja *Biology and approaches to treatment of GNE myopathy* Noah Weisleder, PhD *Patient voice: GNE Myopathy* Al Stork	Cortez CD

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Tuesday, March 18, 2025 cont.		
11:00 AM – 12:00 PM	**Rational Disease-Modifying Therapies for Charcot-Marie-Tooth Disease and Related Neuropathies – Are We There Yet?** Chair – Joshua Burns, PhD *Genetic Discoveries and Clinical Trials in CMT* Michael Shy, MD, FAAN, FANA *Treatment Pipeline for CMT* Mario Saporta, MD, PhD, MBA *Challenges and Opportunities in Pediatric Clinical Trials* Richard Finkel, MD *Clinical Trial Readiness for TRPV4 Channelopathies* Charlotte Sumner, MD	Coronado A
	Pre-Clinical and Clinical Efforts in Oculopharyngeal Muscular Dystrophy (OPMD) Chair – Mathew Wicklund, MD *History of the Study of OPMD as it Sets the Stage for New Therapeutic Trials* Co-chair – Bernard Brais, MDCM, PhD *Drug Development Approaches to OPMD* Guy Rouleau, PhD *Advances in Non-Instrumental Dysphagia Assessment for Oculopharyngeal Muscular Dystrophy* Claudia Côté, PhD *Interim Clinical Data Summary: A Phase 1b/2a Open-Label, Dose Escalation Study to Evaluate the Safety Andand Clinical Activity of Intramuscular Doses of an AAV9-Based Gene Therapy (BB-301) Administered to Subjects with Oculopharyngeal Muscular Dystrophy (OPMD) with Dysphagia* Jerel Banks, PhD	De Soto AB
12:00 – 1:30 PM	**Industry Forum Lunch presented by argenx** *Reviewing the Evolving Treatment Approaches in Generalized Myasthenia Gravis*	Chantilly West
	Industry Forum Lunch presented by Dyne Therapeutics *Harnessing the Force™ Platform to Advance Targeted Therapies for Neuromuscular Diseases*	Grand DE
	Industry Forum Lunch presented by Edgewise Therapeutics *Spotlight on Becker Muscular Dystrophy: Understanding the Lived Experience of Becker and Clinical Advancements with a Novel Agent*	Wedgewood Ballroom
	Industry Forum Lunch presented by Genentech *Exploring Oral SMA Treatment: Discover the Latest Developments for Evrysdi*	Grand ABC
	Industry Forum Lunch presented by Scholar Rock *Muscle, Myostatin, and More: Evolving Needs and Approaches in SMA*	Chantilly East

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Tuesday, March 18, 2025 cont.		
ALS / FTD Track		
2:00 – 3:00 PM	**Exploring ALS Innovations: Motor Speech, BCIs, and AI Solutions** *Where AI Can (and Can't) Help with ALS* Chair – Ernest Fraenkel, PhD *Motor Speech Analysis and Listener Effort as ALS Outcome Measurers* James Berry, MD, PhD *BCIs for Restoring Speech and Communication* Daniel Rubin, MD, PhD	Coronado A
3:00 – 4:00 PM	**Advances in Biomarker Exploration** Chair – Tania Gendron, PhD *HDGFL2, a Biomarker for TDP-43 Proteinopathies* Leonard Petrucelli, MD, PhD *Multi-Domain Smartphone Assessments for Neurological and Neuromuscular Disorders: Progress in the FTD-ALS Spectrum* Adam Staffaroni, PhD *Plasma Extracellular Vesicle Tau Isoform Ratios and TDP-43 Inform about Molecular Pathology in Frontotemporal Dementia and ALS* Hanna Huber, PhD *Large-Scale Cerebrospinal Fluid Proteomics Identifies Molecular Signatures of C9orf72 Frontotemporal Dementia* Rowan Saloner, PhD	Coronado A
Disease Mechanism Track		
2:00 – 3:00 PM	**Fibroadipogenic Progenitors - Mechanisms and Therapeutic Opportunities** *Role of FAPs in Muscle Diseases And Its Modulation As A Therapeutic Strategy* Fabio Rossi, MD *Uncovering the Origins of Genuine Senescent Cells to Mitigate Skeletal Muscle Damage: Are Fibroadipogenic Progenitors the Primary Source?* Michael Wosczyna, PhD *Fibroadipogenic Progenitors in Muscular Dystrophy: Contribution to Pathology and Therapeutic Potential* Chair – Marshall Hogarth, PhD Carsten Bönnemann, MD	Coronado BCD

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Tuesday, March 18, 2025 cont.		
3:00 – 4:00 PM	**Genetic Therapies in the Pipeline** *AAV.U7snRNA as a Platform to Treat Neuromuscular Disorders* Chair – Nicolas Wein, PhD *Genetic Precision Medicine for the Collagen VI-Related Muscular Dystrophies: Targeting a Recurrent Pseudoexon-Inducing Variant* Veronique Bolduc, PhD *Advancing Duchenne Muscular Dystrophy Research: Precision Models and Therapeutic Strategies* Zhenya Ivakine, PhD *Gene Editing Correction of DMD Mutations in Human Cells and Humanized Mice* Mateusz Durbacz, MSc	Coronado BCD
	Care Management Track	
2:00 – 4:00 PM	**Preparing for Tomorrow's Leaders & Fostering Diversity Among Providers** ***(CE Accredited)*** Chair - Terry Heiman-Patterson MD Co-Chair - Urvi Desai, MBBS, MS, MD, FAAN Ericka Greene, MD, MACM Vovanti Jones, MD	Cortez CD
	Genetic Medicine Track	
2:00 – 4:00 PM	**Gene Therapy for Intermediate/Experienced Sites** ***(CE Accredited)*** Chair – Natalie Goedeker, DNP, CPNP Craig McDonald, MD, PhD Katherine Mathews, MD	Cortez AB
4:00 – 4:25 PM	Networking Break in Exhibit Hall	Trinity Exhibit Hall

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Tuesday, March 18, 2025 cont.

ALS / FTD Track

4:30 – 6:00 PM	**Newest Therapeutic Target Discoveries** Chair – Nicholas Maragakis, MD *SARM1 Inhibition Confers Neuroprotection in Preclinical Models of Neurological Injury and Disease* Shilpa Sambashivan, PhD *Validation of an AAV Gene Therapy for Amyotrophic Lateral Sclerosis* Philip Wong, PhD *Applying Advanced AAV Strategies for Sporadic ALS* Defne Amado, MD, PhD *Repeat Associated Non-AUG (RAN) Proteins as Therapeutic Targets in C9orf72 ALS and Other Neurodegenerative Diseases* Laura Ranum, MD, PhD *Therapeutic Development in Neurodegenerative Diseases with TDP-43 Proteinopathy* Clotilde Lagier-Tourenne, MD, PhD	Coronado A

Disease Mechanism Track

4:30 – 6:00 PM	**Genetic Modifiers – New Targets for Muscular Dystrophies** *Alpha7 Integrin Enhancing Small Molecule for the Treatment of Duchenne Muscular Dystrophy* Chair – Dean Burkin, PhD *Genetic Modifiers in Congenital Myopathy* Vandana Gupta, PhD *Modifiers of LMNA-Associated Muscular Dystrophy* Lori Wallrath, PhD *Galectin-3 Inhibition Reduces Inflammation and Shifts Macrophage Profiles in LAMA2-CMD* Yonne Tenorio de Menezes, PhD *Targeting Cell-Matrix Interactions for the Muscular Dystrophies* Rachelle Crosbie, PhD *Genetic Modifiers in LGMD* Beth McNally, MD	Coronado BCD

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Tuesday, March 18, 2025 cont.		
Care Management Track		
4:30 – 6:00 PM	**Changing Policies and Pushing Boundaries** ***(CE Accredited)*** *Changing Policies and Pushing Boundaries* Chair – Leigh Maria Ramos-Platt, MD *Leading for Your Patients at the National Level* Nicholas Johnson, MD, MSCI, FAAN *Getting the Genie Out of the Bottle* Lamar Davis II, MD *Enterprise Partnership: Collaborating in High-Cost Drug Program Development* Malika Maddison, MHA, MBA, RT(T) *Collaborating Across 50 States & Within Your Own* Chamindra Laverty, MD	Cortez CD
Genetic Medicine Track		
4:30 – 6:00 PM	**Gene Therapy Funding Strategies: Workshop for MDA Care Centers** ***(CE Accredited)*** Chair – Hoda Abdel-Hamid, MD, MSc, FAAN Co-Chair – Barry Byrne, MD, PhD Ashutosh Kumar, MD Matthew Ginsberg, MD Crystal Proud, MD	Cortez AB
6:00 – 8:00 PM	Poster & Networking Reception	Trinity Exhibit Hall

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Wednesday, March 19, 2025		
8:00 – 11:00 AM	Registration Open	Geppetto Foyer, Tower Side
8:00 AM – 1:45 PM	**New, Novel, Noteworthy & Late-Breaking: NMD Highlights** We are thrilled to have had an overwhelming number of incredible abstract submissions as we reconvene in 2025. 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. This is a final day not to be missed!	
8:00 AM – 1:45 PM	**Clinical Trial Updates**	Coronado ABCD
8:00 – 8:15 AM	*First-In-Human Phase 1 Study of Orally Administered SAT-3247 In Healthy Volunteers and Adult Participants with Duchenne Muscular Dystrophy (DMD)* Phil Lambert, PhD	
8:15 – 8:30 AM	*RGX-202, An Investigational Gene Therapy for The Treatment of Duchenne Muscular Dystrophy: Interim Clinical Data* Carolina Tesi Rocha, MD	
8:30 – 8:45 AM	*Safety and Efficacy from The Ongoing Phase 1/2 DELIVER Trial of DYNE-251 In Males with DMD Mutations Amenable to Exon 51 Skipping* Kevin Flanigan, MD	
8:45 – 9:00 AM	*CONNECT1-EDO51: A 12-Week Open-Label Phase 2 Study to Evaluate PGN-EDO51 Safety and Efficacy in People with Duchenne Amenable to Exon 51 Skipping* Steve Han, MD, PhD	
9:00 – 9:15 AM	*Del-Zota Produced Statistically Significant Increases in Exon Skipping and Dystrophin Levels in EXPLORE44, A Phase 1/2 Study in Patients with DMD44* Aravindhan Veerapandiyan, MD	
9:15 – 9:30 AM	*Long-term functional outcomes and safety of delandistrogene moxeparvovec in DMD: EMBARK 2-year and Pooled 3-year analyses* Crystal Proud, MD	
9:30 – 9:45 AM	*Muscle MRI Outcomes in Patients With Duchenne Muscular Dystrophy Treated with Delandistrogene Moxeparvovec: Findings from EMBARK Part 1* Krista Vandenborne, PhD	
9:45 – 10:00 AM	BREAK	
10:00 – 10:15 AM	*Safety and Efficacy of DT-DEC01 Therapy in Non-Ambulatory Duchenne Muscular Dystrophy Patients Up to 24 Months After Systemic Administration* Maria Siemionow, MD, PhD, DSc	
10:15 – 10:30 AM	*Interim Data Following 24 Weeks of Treatment With WVE-N531 In the Phase 2 Open-Label FORWARD-53 Study* Li-Jung Tai, MD, PhD	
10:30 – 10:45 AM	*CANYON Trial Results: Sevasemten, An Investigational Fast Skeletal Myosin Inhibitor, Reduced Muscle Damage Biomarkers and Stabilized Function in BMD* Craig McDonald, MD	
10:45 – 11:00 AM	*Efficacy and Safety of Apitegromab in Individuals with Type 2 and Type 3 Spinal Muscular Atrophy Evaluated in The Phase 3 SAPPHIRE Trial* Thomas Crawford, MD	
11:00 – 11:15 AM	*Long-Term Vatiquinone Treatment Slows FA Disease Progression Relative to FACOMS Natural History)* Jonathan Cherry, PhD	

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Wednesday, March 19, 2025 cont.		
11:15 – 11:30 AM	*Topline Efficacy and Safety Results from REACH: Phase 3 Placebo-Controlled Trial of Losmapimod for Facioscapulohumeral Muscular Dystrophy (FSHD)* Jeffrey Statland, MD	
11:30 – 11:45 AM	*Miglustat: A First-in-Class Enzyme Stabilizer for Late-Onset Pompe Disease* Jon Brudvig, PhD	
11:45 AM – 12:00 PM	BREAK	
12:00 – 12:15 PM	*RAINBOWFISH: 2-year Efficacy and Safety Data in Risdiplam-Treated Infants with Presymptomatic Spinal Muscular Atrophy (SMA)* Richard Finkel, MD	
12:15 – 12:30 PM	*Exploring Higher Doses of Nusinersen in Spinal Muscular Atrophy (SMA): Final Results from Parts B and C of the 3-part DEVOTE Study* Thomas Crawford, MD	
12:30 – 12:45 PM	*Safety and Efficacy of DYNE-101 in Adults with DM1: Phase 1/2 ACHIEVE Trial Data* James Lilleker, MD, MBChB, MRCP, PhD	
12:45 – 1:00 PM	*FREEDOM-DM1: A Phase 1, Placebo-Controlled Single Ascending Dose Study to Evaluate PGN-EDODM1 in People with Myotonic Dystrophy Type 1 (DM1)* Jane Larkindale, PhD	
1:00 – 1:15 PM	*Long-Term Safety, Tolerability and Efficacy of AMO-02 in Children, Adolescents and Adults with Congenital and Childhood Myotonic Dystrophy* Emily Fantelli	
1:15 – 1:30 PM	*Interim Study Update for the BB-301 Gene Therapy Phase 1b/2a First in Human Trial in Subjects with Oculopharyngeal Muscular Dystrophy with Dysphagia* Jerel Banks, MD, PhD	
1:30 – 1:45 PM	*A Clinical DMD Cytosine Base Editing Drug* Chunyan He, PhD	
1:45 – 2:00 PM	*Initial Experience From the INSPIRE DUCHENNE Phase I/II Study of SGT-003 Microdystrophin Gene Therapy for Duchenne Muscular Dystrophy* Kevin Flanigan, MD	
2:00 – 2:15 PM	*Oral Thromboxane Receptor Antagonist Demonstrates Significant Cardiac Benefit in 12-Month Phase 2 Trial in Duchenne Muscular Dystrophy Patients* John Jerry Parent	
2:15 – 2:30 PM	*Intrathecal Onasemnogene Abeparvovec For Patients with Spinal Muscular Atrophy: Phase 3, Randomized, Sham-Controlled, Double-Blind STEER Study* Crystal Proud, MD	
8:00 – 9:45 AM	Preclinical Gene Correction Strategies	Cortez CD
8:00 - 8:15 AM	*ARCUS-Mediated Excision of Exons 45-55 Leads to Functional Del45-55 Dystrophin and Restoration of Skeletal Muscle-Function for the Treatment of DMD* Adam Mischler, PhD	
8:15 – 8:30 AM	*Splicing is Improved Using a Novel AAV-Microrna Delivery Platform As A Treatment For Myotonic Dystrophy Type 1* Martin Goulet, PhD	
8:30 – 8:45 AM	*AAV Gene Therapy For LMNA-Associated Laminopathies* Monique Otero	
8:45 – 9:00 AM	*A Survival-Enhanced R155H Homozygote VCP Mouse Model: A Platform for Testing AAV Gene Therapy in Multisystem Proteinopathy* Lan Weiss, PhD	

MDA CLINICAL & SCIENTIFIC CONFERENCE 2025

Conference Agenda

ALL CONFERENCE SESSION TIMES LISTED ARE IN CENTRAL TIME
*SPEAKERS LISTED IN ORDER OF PRESENTATION

Wednesday, March 19, 2025 cont.		
9:00 – 9:15 AM	*Novel CMT2E Mouse Models with NEFL E397K Mutation Reveal Early Chronic Axonal Neuropathy and Phenotype Rescue via AAV Knockdown-and-Replace Therapy* Christian Lorson, PhD	
9:15 – 9:30 AM	*Systemic AAV Gene Therapy with Next Generation Engineered Capsids for Treatment of CNS and Cardiac Symptoms in Friedreich's Ataxia* Ryan Kast, PhD	
9:30 – 9:45 AM	*CSF Delivery of INS1202 AAV9-SOD1-shRNA Rescues Muscle Function and Hallmarks of Neurodegeneration in a Disease Model Of ALS* Laura Ferraiuolo, PhD	
9:45 – 10:00 AM	BREAK	
10:00 AM – 1:00 PM	Preclinical Studies in Dystrophies and Myopathies	Cortez CD
10:00 – 10:15 AM	*Identification of Enzymatically Modified Isoquercitrin (EMIQ) as a Therapeutic Candidate for Myotonic Dystrophy tTpe 1* Subodh Mishra, PhD	
10:15 – 10:30 AM	*Discovery of Small Molecules that Bind CUG Repeats, Displace Muscleblind Protein, and Improve Pathogenesis of Myotonic Dystrophy Type 1* Domi Stickens	
10:30 – 10:45 AM	*Evaluation of an Adiponectin Receptor Agonist, ALY688, In The Mdx Model Of DMD* Henry Hsu, MD	
10:45 – 11:00 AM	*Novel Inhibitor PK007 Reduces Muscle Inflammation and Myonecrosis in Mdx Mouse Models* Sai Yarlagadda, PhD	
11:00 – 11:15 AM	*Irodanoprost, A Tissue-Targeted EP4 Receptor Agonist, Improves Muscle Histology And Function in a Rat Model Of Severe Duchenne Muscular Dystrophy* Paul Kostenuik, PhD	
11:15 – 11:30 AM	*Novel Small Molecule Mitochondriotropics Reverse Skeletal Muscle Pathology and Improve Resilience in The D2.Mdx Model Of Duchenne Muscular Dystrophy* Matt Whiteman, MD, PhD	
11:30 – 11:45 AM	*Non-Viral Gene Therapy for DMD Allowing Full-Length Dystrophin Delivery to Skeletal, Cardiac, and Diaphragm Muscles* Ivan Krivega, PhD	
11:45 AM – 12:00 PM	BREAK	
12:00 – 12:15 PM	*Small Non-Coding tRNA Derivative Stabilizes Heart Function in Mice with Duchenne Muscular Dystrophy* Russell Rogers, PhD	
12:15 – 12:30 PM	*MLAB-001: A Novel Muscle-Targeted Notch Agonist as a Potential New Treatment for Duchenne Muscular Dystrophy* Yunziang Zhu, PhD	
12:30 – 12:45 PM	*Extracellular Vesicles Ameliorate Innate Immune Responses to AAV Gene Therapy* Bradley Hamilton, PhD	
8:00 - 9:45 AM	Advancing NMD Research with Digital Tools and Patient Data	Cortez AB
8:00 - 8:15 AM	*De-Risking Duchenne Muscular Dystrophy Drug Development Through Collaboration, Data Sharing and Understanding of Disease Stages and Progression* Paige Martin, PhD	

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Wednesday, March 19, 2025 cont.		
8:15 – 8:30 AM	*BetterLife FSHD and FSHD Navigator: Harnessing Patient Data and Lived Experience to Improve Health and Drive Research* Amanda Hill, MBA & Anna Gilmore	
8:30 – 8:45 AM	*Feasibility of Using a Wearable Digital Health Technology Sensor to Assess Ambulation n DM1 Aand CMT* Laurent Servais, MD, PhD	
8:45 – 9:00 AM	*Remote Monitoring of Physical Activity and Upper Limb Function in Adults with Charcot-Marie-Tooth Disease* Kayla Cornett, PhD	
9:00 – 9:15 AM	*Digital Health Technology for Remote Symptoms Monitoring in Myasthenia Gravis* Ashkan Vaziri, PhD	
9:15 – 9:30 AM	*Reliability and Sensitivity of a Home-Based Video Assessment for Patients with Multisystem Proteinopathy* Lindsay Alfano, PT, DPT, PCS	
9:30 – 9:45 AM	*Reliability of Remote Assessments in Myotonic Dystrophy Type 1* Laura Tufano	
9:45 – 10:00 AM	BREAK	
10:00 AM – 12:00 PM	Molecular Insights into NMDs	Cortez AB
10:00 – 10:15 AM	*A Large, Real-World Study of Serum Nfl from People with ALS Enrolled in the ARC Study* Fernando Vieira, MD	
10:15 – 10:30 AM	*Low-Density Lipoprotein Receptor is Critical for Sporadic ALS CSF-Induced Neurotoxicity* Jamie Kay Wong, PhD	
10:30 – 10:45 AM	*Skeletal Muscle Targeted ClC-1 Ion Channel Inhibitor Improves Skeletal Muscle Function nd Respiratory Function In A Rat Model of MuSK-MG* Martin Brandhøj Skov, PhD	
10:45 – 11:00 AM	*Restoration Oof Motor Neuron Function Via Pharmacological n Vivo Regulation Oof a Potassium Channel in SMA Mice* Nandhini Sivakumar, PhD	
11:00 – 11:15 AM	*Spinal and Bulbar Muscular Atrophy Cardiomyocytes Exhibit Arrhythmia* Asuka Eguchi, PhD	
11:15 – 11:30 AM	*Long-Read Whole Genomic Sequencing Reveals Novel Pathogenic Alleles in Friedreich Ataxia* Sanjay Bidichandani, MBBS, PhD	
11:30 – 11:45 AM	*Spatial Transcriptomics of Motor Neuron Subcellular Compartments Reveals an Axon-Specific RNA Signature and Local Translation Defects in FUS-ALS* Diana Piol, PhD	