MDA CLINICAL & SCIENTIFIC CONFERENCE 2027

Conference Agenda

ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN TIME

Saturday, March 20, 2027		
8:00 AM – 6:00 PM	Registration Open for Large Exhibit Booth Sponsors & Conference Attendees	Gatlin 1 Registration

Sunday, March 21, 2027		
7:00 AM – 7:00 PM	Registration Open	Gatlin 1 Registration
	MDA Care Center Directors Meeting (Invite Only)	
	MDA Allied Health Workshop	
	MDA Trainee Networking Session (Invite Only)	
	Industry Council Meeting	
	NeuroMuscular Advocacy Collaborative (Invite Only)	
	Neuromuscular Stakeholder Networking Sesson (Invite Only)	
6:00 – 8:00 PM	Welcome Reception & Posters in Exhibit Hall	Sebastain JK

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Monday, March 22, 2027		
6:30 AM – 7:00 PM	Registration Open	Gatlin 1 Registration
7:00 – 8:00 AM	Industry Forum Breakfast sponsored by Alexion	
	Industry Forum Breakfast sponsored by Bridge Bio	
	Industry Forum Breakfast sponsored by Dyne	
	Industry Forum Breakfast sponsored by EMD Serano	
	Industry Forum Breakfast sponsored by NS Pharma	
	Industry Forum Breakfast sponsored by Sanofi	
8:15 – 10:15 AM	Conference Opening & Welcome Remarks MDA Award Presentations Opening Keynote Address	
10:15 – 10:45 AM	Exhibit Hall & Poster Sessions Open	
10:45 – 11:45 AM	Living with Ultra-Rare Disease: Patient & Caregiver Perspectives	
12:00 – 1:30 PM	Industry Forum Lunch sponsored by argenx	
	Industry Forum Lunch sponsored by ITF Therapeutics	
	Industry Forum Lunch sponsored by Novartis	
	Industry Forum Lunch sponsored by Regenx Bio	
	Industry Forum Lunch sponsored by Servier	
	Industry Forum Lunch sponsored by Scholar Rock	
12:00 – 1:30 PM	Exhibit Hall & Poster Sessions Open	
Track 1 – Preclinical Research		
1:45 – 3:45 PM	Cross-Disease Insights in Muscle Biology Chair – Robert Bloch, PhD	
Track 2 – Disease Spotlight		
1:45 – 3:45 PM	From Risk to Prevention: The Evolving Landscape of ALS Gene Carrier Research Chair – Matthew Harms, MD	

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Monday, March 22, 2027		
Track 3 – Drug Development Considerations		
1:45 – 3:45 PM	**FDA's Evolving Regulation of Neuromuscular Disease Therapies: Where We Are in 2027 and Where We're Going** Chair – Paul Melmeyer	
Track 4 – Disease Intervention Strategies		
1:45 – 3:45 PM	**Gene Therapy: Safety, Sustainability, Open Questions** Chair – Diana Bharucha-Gobel, MD	
Track 5 - Care Team Considerations		
1:45 – 3:45 PM	**Case Studies in Access: Overcoming Obstacles to Optimal Care**	
3:45 – 4:15 PM	Exhibit Hall & Poster Sessions Open	
3:45 – 4:15 PM	Networking Break	
Track 1 – Preclinical Research		
4:00 – 6:00 PM	**The Dystrophin Debate: Exon Skipping, Micro- or Full-Length Dystrophin, and Gene Editing Strategies** Chair – Jeffrey Chamberlain, PhD	
Track 2 – Disease Spotlight		
4:00 – 6:00 PM	**Immune-Mediated Neuromuscular Diseases: Advances in Mechanisms and Therapeutics** Chair – Bhaskar Roy, MBBS, MD, MHS	
Track 3 – Drug Development Considerations		
4:00 – 6:00 PM	**Optimizing the Likelihood of Clinical Trial Success Through Innovative Outcome Measures, Statistical Methodology, Recruitment Precision, and Community Engagement** Chair – Chad Heatwole, MD	
Track 4 – Disease Intervention Strategies		
4:00 – 6:00 PM	**Prenatal Testing & Newborn Screening: How are we doing and what's new?** Chairs – Stephen Chrzanowski, MD & Thomas Crawford, MD	
Track 5 – Care Team Considerations		
4:00 – 6:00 PM	**Great Debates: From Headlines to Healthcare**	
6:00 – 8:00 PM	Poster & Networking Reception	

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Tuesday, March 23, 2027		
6:30 AM – 4:00 PM	Registration Open	Gatlin 1 Registration
7:00 – 8:00 AM	Industry Forum Breakfast sponsored by argenx	
	Industry Forum Breakfast sponsored by Catalyst	
	Industry Forum Breakfast sponsored by Novartis	
	Industry Forum Breakfast sponsored by Regeneron	
	Industry Forum Breakfast sponsored by Satellos	
	Industry Forum Breakfast sponsored by Solid Biosciences	
Track 1 – Preclinical Research		
8:15 – 10:15 AM	**Reaching the Target: Overcoming Delivery Barriers in Neuromuscular Disease** Chair – James Dowling, MD, PhD	
Track 2 – Disease Spotlight		
8:15 – 10:15 AM	**CMT Across The Translational Spectrum: Mechanisms To Therapeutics** Chairs – Christian Lorson, PhD & Kathryn Moss, PhD	
Track 3 – Drug Development Considerations		
8:15 – 10:15 AM	**From Registry to Ecosystem: Unlocking the Power of Patient Data** Chair - Andre Paredes, PhD	
Track 4 – Disease Intervention Strategies		
8:15 – 10:15 AM	**Core Support Systems: Considerations for Orthopedic Care & Pulse Check: Emerging Best Practices for Cardiac Care** Chair – John Brandsema, MD	
Track 5 – Care Team Considerations		
8:15 – 10:15 AM	**Focus on Mental Health: ALS, Gene Therapy and Transition**	
10:15 AM – 1:30 PM	Exhibit Hall & Poster Sessions Open	
10:15 – 10:45 AM	Networking Break	

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Tuesday, March 23, 2027 (cont.)		
Lab to Life		
10:45 – 11:45 AM	**Facioscapulohumeral Muscular Dystrophy (FSHD)** Chair – Lucianne Ronco, PhD	
10:45 – 11:45 AM	**Periodic Paralysis** Chair – Cienna Ditri	
10:45 – 11:45 AM	**Spinal Muscular Atrophy (SMA)** Chair – Kelly Howell, PhD	
10:45 – 11:45 AM	**Friedreich's Ataxia** Chair – Jennifer Farmer, MS	
10:45 – 11:45 AM	**Myotonic Dystrophy** Chair – Andy Rohrwasser, PhD, MBA	
12:00 – 1:30 PM	Industry Forum Lunch sponsored by Benitec	
	Industry Forum Lunch sponsored by Biogen	
	Industry Forum Lunch sponsored by Bridge Bio	
	Industry Forum Lunch sponsored by Dyne	
	Industry Forum Lunch sponsored by Entrada	
	Industry Forum Lunch sponsored by Sarepta	
Track 1 – Preclinical Research		
1:45 – 3:45 PM	**Common Pathways in Neurodegeneration and Neuromuscular Disease** Chair – Brett Morrison, MD	
Track 2 – Disease Spotlight		
1:45 – 3:45 PM	**Congenital Myopathies Unpacked: Diagnosis, Care, and Emerging Therapies** Chair – Alan Beggs, PhD	
Track 3 – Drug Development Considerations		
1:45 – 3:45 PM	**Catalyzing Progress: Innovative Programs Accelerating Neuromuscular Research** Chair – Angela Lek, PhD	
Track 4 – Disease Intervention Strategies		
1:45 – 3:45 PM	**Case Studies 1: Faculty And Fellows Case Discussions & Case Studies 2: Novel Presentations And Complex Treatment Approaches** Chair – Bhakri Elsheikh, MBBS & Katherine Mathews, MD	
Track 5 – Care Team Considerations		
1:45 – 3:45 PM	**Reducing Patient Burden: A Little Help from Remote Technologies**	

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Tuesday, March 23, 2027 (cont.)		
3:45 – 4:10 PM	Exhibit Hall & Poster Sessions Open	
3:45 – 4:15 PM	Networking Break	
Track 1 – Preclinical Research		
4:15 – 6:00 PM	**Building Better Muscle: Advances in Regeneration and Repair** Chair – Elisabeth Barton, PhD	
Track 2 – Disease Spotlight		
4:15 – 6:00 PM	**The Other Half of LGMD: Addressing the Unmet Needs of Dominant LGMDs** Chair – Peter Kang, MD	
Track 3 – Drug Development Considerations		
4:15 – 6:00 PM	**AI in Drug Development: Transforming the Future of Neuromuscular Therapeutics** Chair – Stephan Zuckner, MD, PhD	
Track 4 – Disease Intervention Strategies		
4:15 – 6:00 PM	**All the Tools in our Toolbox: Does Genetic Precision Replace Phenotype?** Chair – Julie Parsons, MD	
Track 5 – Care Team Considerations		
4:15 – 6:00 PM	**Aging Well with NMD: New Challenges in a Longer-Lived Populations**	
6:00 – 8:00 PM	Poster & Networking Reception	

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Wednesday, March 24, 2027		
6:30 AM – 10:30 AM	Registration Open	Gatlin 1 Registration
7:00 – 8:00 AM	Industry Forum Breakfast sponsored by Biogen	
8:30 AM – 1:30PM	**New, Novel, and Noteworthy: NMD Highlights** This session features select presentations drawn from the 2027 abstract submissions, highlighting new and noteworthy research from across the neuromuscular disease field. Presentations may span pre-clinical and translational research, clinical trials, clinical management, and other emerging areas of research and care.	