



Conference Agenda

SUNDAY, MARCH 14, 2021

9:30 AM – 5 PM

MDA Insights in Research Investor Summit (Invitation Only)

MONDAY, MARCH 15, 2021	
6 AM – 6 PM	Poster Session Open
9:30 – 10 AM	Welcome & Keynote Presentation
10 AM – 6 PM	Exhibit Hall Open
10 – 11 AM	New Biomarkers in Neuromuscular Disease Research Elizabeth McNally, MD, PhD (Chair) Annemieke Aartsma-Rus Glenn Walter, PhD Thurman Wheeler, MD
11 AM – 12 PM	MOVR & Real-World Evidence Rayne Rodgers, MPH (Chair) Susan Apkon, MD DeWayne Townsend, DVM, PhD
12 – 1:30 PM	Industry Forum Lunch Presented by Alexion Pharmaceuticals A Treatment for Adult Patients With Anti-Acetylcholine Receptor (AChR) Antibody-Positive Generalized Myasthenia Gravis (gMG)
	Industry Forum Lunch Presented by Genentech Evrysdi® (risdiplam) Experiences: Explore the Science and Hear From People Living With Spinal Muscular Atrophy
	Industry Forum Lunch Presented by Novartis Gene Therapies Adena-Associated Virus (AAV) Platform: Considerations for Gene Therapy
1:30 – 3 PM	Axonal Degeneration and Transport Defects Michael Shy, MD (Chair) Robert Baloh, MD Michael Coleman, PhD Erika Holzabaur, PhD Charlotte Summer, MD
	Therapeutic Considerations for Dominant Neuromuscular Diseases Beth Barton, PhD (Chair) Veronique Bolduc, PhD Andrew Findlay, MD Eugene Yeo, PhD, MBA Toshifumi Yokota, PhD

MONDAY, MARCH 15, 2021 Cont.

3 – 4:30 PM	Gene Editing in Neuromuscular Disease Eric Olson, PhD (Chair) Charles Gersbach, PhD Erik Sontheimer, PhD
	Innovative Modeling in Neuromuscular Disease Jamshid Arjomand, PhD (Chair) Robert Bloch, PhD Clive Svendsen, PhD Mina Gouti, PhD Ronald Cohn, MD, FACMG
4:30 – 6 PM	Non-viral Delivery in Neuromuscular Disease Stanley C. Froehner, PhD (Chair) Oxana Beskrovnaya, PhD James Dahlman, PhD Melissa Spencer, PhD Nicholas Whitehead, PhD
6 – 8 PM	MDA Scholars Virtual Meeting (Invitation Only)

TUESDAY, MARCH 16, 2021	
6 AM – 9 PM	Exhibit Hall & Poster Session Open
8 – 9AM	Welcome & Keynote Presentation
9 – 10 AM	Lab-to-Life: Inflammatory Myopathies Thomas Lloyd, MD, PhD (Chair) Steven Greenberg, MD Andrew Mammen, MD, PhD Lauren Pachman, MD
10 – 11 AM	Lab-to-Life: Mitochondrial Diseases including Friedreich's Ataxia Carlos Moraes, PhD (Chair) Gino Cortopassi, PhD Anu Soumalainen, MD, PhD
11 AM – 12 PM	Lab-to-Life: Spinal and Bulbar Muscular Atrophy Kenneth Fischbeck, MD (Chair) Andrew Lieberman, MD, PhD Diane Merry, PhD Carlo Rinaldi, MD, PhD
12 – 1:30 PM	Industry Forum Lunch Presented by Amylyx Pharmaceuticals Pathways and Paradigms in ALS: Life and Death in the Neuron
	Industry Forum Lunch Presented by Biogen The Evolution of Treatment with SPINRAZA® (nusinersen) Over the Last 4 Years
	Industry Forum Lunch Presented by Sarepta Therapeutics Understanding Diagnosis and Phenotypes in Muscular Dystrophies
1:30 – 2:30 PM	Lab-to-Life: Congenital MD Kevin Campbell, PhD (Chair) Carsten Bonnemann, MD Madeline Durbeej-Hjalt, PhD Qi Lu, MD, PhD

TUESDAY, MARCH 16, 2021 Cont.

2:30 – 4 PM	<p>Barriers to Clinical Trial Participation John Day, MD, PhD (Chair) Kate Eichinger, PT, PhD, NCS Colleen Labbadia, Parent Catherine Seiner, PT, MHS</p>
	<p>Optimizing ALS Clinical Care and Clinical Research in an MDA ALS Care Center in 2021 Lora Clawson (Chair) Kristen Riley, PhD Rachel Slusher, PT, DPT Alpa Uchil, MSN, MPH, CRNP</p>
	<p>The Changing Regulatory Environment: Impact on Neuromuscular Disease Drug Development James Valentine (Chair) Mindy Leffler Paul Melmeyer Lei Xu, MD, PhD</p>
4:30 – 6 PM	<p>When Gut Feelings Aren't Helping, Revisited: Navigating Complicated Ethics in Neuromuscular Care in 2021 John Brandsema, MD (Chair) Alyssa Burgart, MD Tom Crawford, MD Barkri Elsheikh, MD Julie Parsons, MD</p>
	<p>Rare Disease Initiatives Barry Byrne, MD, PhD (Chair) Alan Beggs, PhD Philip John (PJ) Brooks, PhD Jane Larkindale, Dphil Laura Brod Hameed</p>
	<p>Reproductive Health, Family Planning & Parenting Elicia Estrella, MS, CGC, LGC (Chair) Deodonne Bhattarai, Parent Judy Jackson, MS, LCGC Sharyn Lincoln, MS, LCGC Alexandria Lisi, BA Biology</p>
6 – 9 PM	<p>Dedicated Exhibit Hall & Poster Session <i>This time is dedicated to viewing Exhibit Booths & Posters exclusively</i> <i>Exhibit Booths & Posters to be staffed in real time (if possible)</i></p>

WEDNESDAY, MARCH 17, 2021	
6:30 AM – 9 AM	Dedicated Poster Session <i>This time is dedicated to viewing Posters exclusively Posters to staffed in real time (if possible)</i>
9 AM – 6 PM	Exhibit Hall & Poster Session Open
9 – 10:30 AM	Impact of COVID-19 on Neuromuscular Disease Care Nicholas Johnson, MD, MS-CI (Chair) Kathy Mathews, MD Diana Mnatsakanova, MD
	Measuring Progression of Disease & Selecting Endpoints Erik Henricson, PhD, MPH (Chair) Tina Duong, PT, PhD Chad Heatwole, MD, MS-CI Stan Nelson, MD
10:30 AM – 12 PM	Clinical Trials in the Age of COVID-19 Jeffrey Statland, MD (Chair) Nicholas Johnson, MD, MS-CI Petra Duda, MD, PhD Michelle Mellion, MD Lauren Morgenroth, MS
	Navigating SMA Treatment Decisions Perry Shieh, MD, PhD (Chair) John Brandsema, MD Diana Castro, MD
12 – 1:30 PM	Industry Forum Lunch Presented by Biogen Current and Unmet Needs of Patients With SMA Across the Lifespan
	Industry Forum Lunch Presented by PTC Therapeutics Duchenne Muscular Dystrophy: Advancing the Body of Evidence for Corticosteroid Treatment
	Industry Forum Lunch Presented by Sarepta Therapeutics Development of AAVrh74 Micro-dystrophin Gene Transfer Therapy for Duchenne
1:30 – 3 PM	Telehealth in Neuromuscular Disease Care Mario Saporta, MD, PhD, MBA, FAAN (Chair) Gyula Ascadi, MD, PhD Matthew Caraher, PR, DPT Volkan Granit, MD Michael Shy, MD
	Therapies Targeting Pulmonary Function in Neuromuscular Disease Jonathan Finder, MD (Chair) John Coleman, III, MD Hank Mayer, MD

WEDNESDAY, MARCH 17, 2021 Cont.

3 – 4:30 PM	Case Studies in Access to New Therapies Claudia Chiriboga, MD, MPH, FAAN (Chair) Anne Connolly, MD, FAAN Basil Darras, MD Matt Harms, MD
	A Multidisciplinary Approach to Clinical Trials; The Role of Physical and Occupational Therapists in Neuromuscular Research Sally Dunaway Young, PT, DPT (Chair) Jacqueline Montes, PT, Ed (Chair) Kate Eichinger, PhD, DPT Tim Estilow, OTR/L Sara Feldman, PT, DPT, ATP
4:30 – 6 PM	Cardiomyopathy Across the Neuromuscular Disease Spectrum Linda Cripe, MD (Chair) Andreas Barth, MD, PhD, FAHA Kan Hor, MD Chet Villa, MD
	Ethical Considerations in Gene Therapy Trials Kevin Flanigan, MD (Chair) Diana Bharucha-Goebel, MD Barry Byrne, MD, PhD
6 – 9 PM	Dedicated Exhibit Hall Session <i>This time is dedicated to viewing Exhibit Booths exclusively</i> <i>Exhibit Booths to be staffed in real time (if possible)</i>

THURSDAY, MARCH 18, 2021	
6:30 AM – 9 AM	Dedicated Exhibit Hall & Poster Session <i>This time is dedicated to viewing Exhibit Booths & Posters exclusively</i> <i>Exhibit Booths & Posters to be staffed in real time (if possible)</i>
9 AM – 6 PM	Exhibit Hall & Poster Session Open
9 AM - 12 PM	Oral Presentations from Abstract Submissions Part I
	WVE-004, an Investigational Stereopure Antisense Oligonucleotide for the Treatment of Amyotrophic Lateral Sclerosis (ALS) and Frontotemporal Dementia Yuanjing Liu, PhD
	Slc20a1/Pit1 and Slc20a2/Pit2 are Essential for Normal Skeletal Myofiber Function and Survival Clemens Bergwitz, MD
	Resolution of Causality in Myotonic Dystrophy, Type 1 (DM1) via a PATrOL™-Enabled Therapy Dietrich Stephan, PhD
	AAV Gene Therapy for TNNT1-Associated Nemaline Myopathy Eleonora D'Ambrosio, MD
	Dendrimer-Conjugated GCPII Inhibitor Selectively Targets Activated Macrophages in SOD1G93A Muscle Which Delays Functional Loss and Denervation Carolyn Tallon, PhD
	Galectin-1 Improves Sarcolemma Repair and Decreases Inflammatory Response in LGMD2B Models Pam Van Ry
	Micro-dystrophin Gene Therapy Delivery and Therapeutic Plasma Exchange in Non-Human Primates Ellyn Peterson
	A Hotspot in CAPN3 as the Likely Cause of Autosomal-Dominant Calpainopathy (LGMD4) Michelle Allen-Sharpley, MD, PhD
	Molecular Markers of Clinical Severity in Becker Muscular Dystrophy from a Remote Study Utkarsh Dang, PhD
	A Pilot Study Using a Novel Wearable Sensor to Examine Physical Activity in Individuals with Charcot Marie Tooth Disease 1A Katy Eichinger, PT, PhD
Quantitative Muscle Analysis in FSHD Using Whole-Body MRI: Composite Muscle Measurements for Cross-Sectional Analysis Michelle Mellion, MD	
12 – 1:30 PM	Industry Forum Lunch Presented by NS Pharma A Treatment Option for Patients with Duchenne Muscular Dystrophy Amenable to EXON 53 Skipping
	Industry Forum Lunch Presented by Sanofi The Challenges of Newborn Screening for Neuromuscular Disorders The Pompe Disease Experience
	Industry Forum Lunch Presented by Solid Biosciences Real World Outcome Measures in Duchenne Muscular Dystrophy: Current and Novel Assessments of Meaningful Patient Benefit

THURSDAY, MARCH 18, 2021 Cont.

THURSDAY, MARCH 18, 2021 Cont.	
	Oral Presentations from Abstract Submissions Part II
	Galectin-1 Improves Sarcolemma Repair and Decreases Inflammatory Response in LGMD2B Models Anna Jesus, MD Mbe
	Predictors of Functional Outcomes in Patients with Facioscapulohumeral Muscular Dystrophy Natalie Katz, MD PhD
	Apitegromab, a Novel High-Affinity Anti-proMyostatin Monoclonal Antibody for Treating Spinal Muscular Atrophy: Results of a Phase 2 Interim Analysis Amy Place, PhD, MBA, MS, RD, CLT
	Predictive Factors of Nusinersen Treatment Response in Infantile-onset SMA: Results from the ENDEAR/SHINE Studies Laurent Servais, MD, PhD
	Kinetics of Early Antibody Response and Immunotoxicity Following Zolgensma for the Treatment of SMA Type 1 Stephanie Salbarria
	SUNFISH Part 2: 24-Month Efficacy and Safety of Risdiplam in Patients with Type 2 or Non-Ambulant Type 3 Spinal Muscular Atrophy (SMA) Maryam Oskoui, MD, MSc, FRCPC, FAAN
	Nusinersen in Children with SMA Who Received Onasemnogene Abeparvovec: Design of the Phase 4, Open-Label RESPOND Study Julie Parsons, MD
1:30 - 6 PM	Long-Term Follow-Up (LTFU) of Onasemnogene Abeparvovec Gene Therapy in Spinal Muscular Atrophy (SMA) Jerry R. Mendell, MD
	A Randomized, Double-Blind, Placebo-Controlled, Gene-Delivery Clinical Trial of rAAVrh74.MHCK7. Micro-Dystrophin for Duchenne Muscular Dystrophy Jerry R. Mendell, MD
	Lower Long-Term Risk of Death or Permanent Ventilation and First Hospitalization Among Participants with ALS Receiving AMX0035 in the CENTAUR Trial Sabrina Paganoni, MD, PhD
	IGNITE-DMD: Phase I/II Ascending Dose Study of Single IV Infusion of SGT-001 Microdystrophin Gene Therapy for DMD-One Year Efficacy and Safety Results Barry Byrne, MD, PhD
	Onasemnogene Abeparvovec Gene Therapy in Presymptomatic Spinal Muscular Atrophy (SMA): SPR1NT Study Update in Children with 3 Copies of SMN2 Kevin Strauss, MD
	Safety, β-sarcoglycan Expression, and Functional Outcomes from Systemic Gene Transfer of rAAVrh74.MHCK7.SGCB in Limb Girdle Muscular Dystrophy Type 2E Louise Rodino-Klapac, PhD
	Safety and Efficacy of PF-06939926 Gene Therapy in boys with Duchenne Muscular Dystrophy: Update on data from the Phase 1b Study Beth Belluscio, MD, PhD
	Early Intervention with Edaravone in Study 19 was Associated with Decreased Hospitalization, Tracheostomy, Ventilation, and Death in Patients with ALS Benjamin Brooks, MD
	2.5-years of Vamorolone Treatment in Duchenne Muscular Dystrophy: Results of an Open Label Long-Term Extension Utkarsh Dang, PhD
7 PM	Meeting Adjourned