MDA CLINICAL & SCIENTIFIC CONFERENCE 2022 MARCH 13 - 16

Conference Agenda

ALL CONFERENCE SESSION TIMES LISTED ARE IN CENTRAL TIME

Sunday, March 13, 2022	
9 AM – 5 PM	**Insights in Research Investor Summit (IRIS)**
6 - 8 PM	**Welcome Reception in Exhibit Hall**

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Monday, March 14, 2022	
7 - 8 AM	**Industry Forum Breakfast Presented by Edgewise** A Novel Approach to Protecting Dystrophic Muscle by Targeting Fast Muscle Myosin: EDG-5506
	Industry Forum Breakfast Presented by NS Pharma A Treatment Option for Patients with Duchenne Muscular Dystrophy Amenable to Exon 53 Skipping
8:30 – 9:30 AM	Opening and Keynote Address
10 AM – 8 PM	Exhibit Hall and Poster Sessions Open
9:30 – 10 AM	**Networking Break sponsored by Andelyn Biosciences**
10 AM – 12 PM	**Latest Developments Across the NMD Registry Data Landscape** Rayne Rodgers, MPH (Co-Chair) Elisabeth Kilroy, PhD (Co-Chair) Alexandre Bétourné, PhD, PharmD, PMP Russell Butterfield, MD, PhD Sarah Emmons Nicholas Johnson, MD, MS-CI, FAAN Allison Moore Paul Strumph, MD Session will discuss real-world data in neuromuscular disease and its application to therapy development.
12 – 1:30 PM	**Industry Forum Lunch Presented by Biogen** Supporting Patients with SPINRAZA ® (nusinersen): Learn More About Our Clinical Trial Program and the Latest Real-World Evidence
	Industry Forum Lunch Presented by Pfizer Innovation for Rare Neuromuscular Diseases
	Industry Forum Lunch Presented by Sarepta What Lies Beneath: Uncovering the Clue for Earlier Diagnosis and Care of Young Individuals with Duchenne
1:30 – 2:30 PM	**Lab to Life: LGMD** Nicholas Johnson, MD, MS-CI, FAAN (Chair) Qi Lu, PhD, MD Melissa Spencer, PhD Conrad Chris Weihl, MD, PhD Session will review advances in translational research in LGMD. Speakers will discuss steps to improve genetic diagnosis, development of small molecules, and advances in gene replacement therapy.

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Monday, March 14, 2022 cont.	
1:30 – 3:30 PM	**New Gene Discoveries in NMD** (CME Accredited Session) Alan Beggs, PhD (Co-Chair) Angela Lek, PhD (Co-Chair) Daniel Calame, MD Elizabeth McNally, MD Anne O'Donnell Luria, MD Stephan Zuchner, MD Session will provide an overview of the rich and rapidly evolving body of knowledge in this area, with talks focused on collaborative and crowd-sourcing approaches to information sharing for new disease gene discovery, the latest on genetic heterogeneity among inherited peripheral neuropathies, approaches to undiagnosed neuromuscular diseases, and new genetic modifiers, which can impact the course of a patient's disease.
2:30 – 3:30 PM	**Lab to Life: FSHD** Jeffrey Statland, MD (Chair) Scott Harper, PhD Peter Jones, PhD Michelle Mellion, MD Stephen Tapscott, MD, PhD Session will explore implementation of targeted therapies in clinical trials, molecular understanding of FSHD, and how we measure changes in future clinical trials.
3:30 – 4 PM	**Networking Break sponsored by Hill-Rom**
4 – 5 PM	**Lab to Life: MG** Henry Kaminski, MD (Chair) Petra Duda, MD, PhD Sara M Jones, MD Linda Kusner, PhD Meridith O'Connor, MSW Session will review the journey of treatment to patient from identification of unmet need to prescription of the drug and access for all patients.
	Functional/PROs/Composite Clinical Trial Endpoints in DMD (CME Accredited Session) Erik Henricson, PhD, MPH (Chair) Roxanna Bendixen, PhD, OTR/L, FAOTA Tina Duong, PT, PhD Heather Gordish-Dressman, PhD Session will explore the uses and intersections between Clinically Reported Outcome tools, Person-Reported Outcome tools, and single- and multi-domain composite endpoints in DMD clinical research. Emphasis will be placed on how tools create a continuum of measures that can be extended from observed clinical task performance, to individual perceptions of ability, to community mobility and participation and life satisfaction.

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Monday, March 14, 2022 cont.	
5 – 6 PM	**Lab to Life: Congenital Myopathies/Dystrophies** Chamindra Konersman, MD (Chair) Alan Beggs, PhD Russell Butterfield, MD, PhD Michael W. Lawlor, MD, PhD Dimah Saade, MD Session will provide understanding of the disease spectrum, genetics and phenotypic complexity of congenital myopathies and congenital muscular dystrophies. Understand current and future strategies for disease modifying treatments and preparing for meaningful clinical trial outcome measures.
	Best Practices in Respiratory Care (CME Accredited Session) Brad Troxler, MD (Chair) Jonathan Finder, MD Andrea Fritsch-Eddleman Session will provide a forum to learn about the latest advancements in respiratory care for patients with NMD. Use of advanced airway clearance techniques and positive airway pressure devices will be discussed. The impact of respiratory care on patient with NMD will be shared.
6 – 8 PM	Poster & Networking Reception

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Tuesday, March 15, 2022	
7 - 8 AM	**Industry Forum Breakfast Presented by Amylyx** Amyotrophic Lateral Sclerosis (ALS) in Depth: Key Topics in Pathophysiology and Diagnosis
	Industry Forum Breakfast Presented by Fulcrum Measuring Impact on Disease Progression in FSHD
8:30 – 10 AM	**Mitochondrial Interventions: Small Molecule and Gene Therapy** Carlos Moraes, PhD (Chair) Michio Hirano, MD Qinglan Ling Session will explore a few approaches with great clinical potential that use small molecules or genetic manipulation.
	Promoting Diversity in Research Susan Apkon, MD (Chair) Jay Griffin Gisel Lopez Mark Terrelonge, MD, MPH Session will provide a greater understanding of the barriers to participation in clinical research for patients from diverse backgrounds and opportunities to improve their involvement. Panelists will share their diverse experience and perspectives as clinicians, family members, and industry partners.
	Telehealth Learnings in Clinical Care and Research: Learnings from the Pandemic (CME Accredited Session) Dustin Nowacek, MD (Chair) Alicia Baxter, MS, CPO Gary Gallagher, MD Mary Schmidt, DO Session will discuss telehealth experiences, identify the benefits of telehealth, and recognize the limitations of virtual care.
	When Gut Feelings Aren't Helping, Revisited: Navigating Complicated Ethics in Neuromuscular Care in 2022 (CME Accredited Session) John Brandsema, MD (Chair) Bakri Elsheikh, MD Brian Jackson, MD Julie Parsons, MD Session aims to review cases from neuromuscular practice that have been complicated ethically and generate an open forum for discussion of how to best navigate similar situations when they arise.
10 AM – 8 PM	Exhibit Hall and Poster Sessions Open
10 – 10:30 AM	**Networking Break sponsored by BrainStorm Cell Therapeutics**

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Tuesday, March 15, 2022 cont.	
10:30 AM – 12 PM	**Small Molecule Approaches to Overcoming Muscle Wasting** Helen Blau, PhD (Co-Chair) Michael Rudnicki, OC, PhD, FRS, FRSC (Co-Chair) Alan Russell, PhD Session will discuss small molecule drugs that counter muscle loss and show promise in treating SMA, DMD, and BMD.
	More Data, Lower Burden – Digital Outcome Measures in Neuromuscular Disease Therapy Development James Berry, MD, MPH (Chair) Andrew Geronimo, PhD Jeremy Shefner, MD, PhD Paul Strijbos, PhD Session will explore digital outcomes measures in neuromuscular disease therapy development.
10:30 AM – 12 PM	**Natural History and Trial Readiness for Children with Charcot Marie Tooth Disease** (CME Accredited Session) Michael Shy, MD (Chair) Tim Estilow, OTR/L Richard Finkel, MD Sindhu Ramchandren, MD, MS Session will provide an update on natural history studies, a description of pediatric patient-reported outcomes assessment evaluating the self-reported quality of life for children with CMT, approaches to evaluating and managing children with CMT, and approaches in clinical trial design in children with CMT and other rare forms of NMD.
	Facilitating Multi-Practice Collaboration to Optimize Patient Care (CME Accredited Session) Lora Clawson, MSN, CRNP (Chair) Jena Krueger, MD Amanda Peltier, MD Session will provide an overview of MDA clinical programs & collaborators; describe how these collaborators are engaged; and describe the challenges and successes in maintaining these important collaborations.
12 – 1:30 PM	**Industry Forum Lunch Presented by Genentech** Explore the Science for an Approved Spinal Muscular Atrophy Treatment and Hear from People Living with SMA
	Industry Forum Lunch Presented by PTC Throughout the Disease Continuum: Corticosteroid Treatment in Duchenne Muscular Dystrophy
	Industry Forum Lunch Presented by Sarepta The Intersection of Science and Medicine – An Investigational rAAVrh74-based Gene Transfer Therapy for Individuals with Duchenne

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Tuesday, March 15, 2022 cont.	
1:30 - 2:30 PM	**Building MD Team in NMD** (CME Accredited Session) Yaacov Anziska, MD (Chair) Reesa Antony, MS CCC-SLP, MPH Oscar Mayer, MD Jacqueline Montes, PT, EdD Andre Prochoroff, MD Amy Shaper, MSW, LSW Session will address experiences from a range of specialists about coordinating with the neurologist to provide the best care for our MDA neuromuscular patients.
1:30 – 3:30 PM	**Personalized Medicine/Regulatory Approaches for Rare Disease** Benjamin Rix Brooks, MD Barry Byrne, MD, PhD Rich Horgan
	Progress in Brain Interface Technology Daniel Rubin, MD, PhD (Co-Chair) Leigh Hochberg, MD, PhD (Co-Chair) Eli Kinney-Lang, PhD, BSc Lynn M. McCane, MS Thomas Oxley, MD, PhD Session will review recent progress, with leaders in the field sharing their vision for the clinical applications and future of these important technologies.
	Treatment Decisions for Disorders with Multiple Therapies (CME Accredited Session) Julie Parsons, MD (Chair) Jinsy A. Andrews, MD, MSc, FAAN Katherine Mathews, MD Gil Wolfe, MD Session will discuss FDA approved therapies for SMA, Duchenne Muscular Dystrophy, ALS, and Myasthenia Gravis. Strategies for medical decision making and treatment selection will be reviewed.
2:30 – 3:30 PM	**The Resilient Practitioner** (CME Accredited Session) Deidre Devier, PhD (Chair) Ambereen K Mehta, MD, MPH Danielle Simmons, PhD Session will address topics of compassion fatigue, compassion, and resilience in relation to the physical, emotional, and psychological impact of helping others; address causes of compassion fatigue in the healthcare setting; discuss the role of compassion in healthcare and the neuroscience behind its effects on patients and practitioners; and discuss ways to build resilience into one's life and clinical practice.

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3:30 – 4 PM	**Networking Break**
Tuesday, March 15, 2022 cont.	
4 – 6 PM	**CRISPR – The Next Generation** Eric Olson, PhD (Chair) Peter Jones, PhD Eleonora Maino Melissa Spencer, PhD Session will focus on recent advances and future challenges in translating CRISPR gene editing for genetic myopathies from animal models toward human translation.
	Clinical Trial Design for Rare Neuromuscular Disorders – Where Are We Headed? Diana Bharucha-Goebel MD (Chair) Carsten Bonnemann, MD Barry Byrne, MD, PhD Katherine Mathews, MD Session will explore issues common to trials in rare disease such as phenotypic pleiotropy, genetic heterogeneity, combination therapies and n=1 trials.
4 – 6 PM	**Impact of Gene Transfer Therapy on Long-Term Multidisciplinary Care** (CME Accredited Session) Emma Ciafaloni, MD (Co-Chair) Jonathan Brandsema, MD (Co-Chair) Julie Parsons, MD Edward Smith, MD Session will address the impact of newborn screening and gene therapy on the neuromuscular clinics' infrastructure and on the multidisciplinary teams; new challenges and opportunities for innovation in the multidisciplinary model; new approach to long term management and assessment of new phenotypes in the era of Gene therapies; and how to optimize equitable access to novel gene therapies and multidisciplinary care in the era of telemedicine.
	Business Operations for NMD Clinics (CME Accredited Session) Daragh Heitzman, MD (Chair) Brent Beson, MD Terry Heiman-Patterson, MD Susan Iannaccone, MD Aravindhan Veerapandiyan, MD Session will provide healthcare professionals' perspectives about this topic including costs, reimbursement, obtaining assistance and grant funding, hurdles, research, the role of telemedicine, the role of altruism and future recommendations.
6 – 8 PM	Exhibits & Poster Networking Reception (Exhibit Hall)

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Wednesday, March 16, 2022

New, Novel and Noteworthy: NMD Highlights

We are thrilled to have had an overwhelming number of incredible abstract submissions as we reconvene in 2022. To that end, we have redesigned our Clinical Trail Presentations to include a wider range of topics which also allows for event more live presentations selected directly from the abstract submissions. This is a final day not to be missed!

8 AM – 12:25 PM	Clinical Trial Outcomes
8:00 - 8:15 am	**Nusinersen Effect in Presymptomatic SMA Infants: 4.9 Year Interim of the NURTURE Study** Thomas Crawford, MD
8:20 - 8:35 am	**SUNFISH Parts 1 and 2: 3-year Efficacy and Safety of Risdiplam in Types 2 and 3 SMA** John Day
8:40 - 8:45 am	**The Avalglucosidase Alfa Phase 3 COMET Trial in Participants With Late-Onset Pompe Disease: Efficacy and Safety Results After 97 Weeks** Hani Kushlaf, MD
8:50 - 9:05 am	**Phase 1 Study of Gene Therapy in Late-onset Pompe Disease: Analyses of Safety, Bioactivity, and Secondary Endpoints** Edward Smith, MD
9:10 - 9:25 am	**Efficacy and Safety of Ravulizumab in Anti-Acetylcholine Receptor Antibody-Positive Generalized Myasthenia Gravis: Phase 3 CHAMPION MG Study** Tuan Vu, MD
9:30 - 9:45 am	**Efficacy of Omaveloxolone in Patients with Friedreich's Ataxia: Update of the Delayed-Start Study** S.H. Subramony, MD
9:50 - 10:05 am	**Vamorolone Versus Placebo and Prednisone in Duchenne Muscular Dystrophy: 24-week Double Blind Trial VBP15-004** Paula Clemens, MD
10:10 - 10:25 am	**EDG-5506 Targets Fast Skeletal Myosin to Protect Dystrophic Muscle and Reduce Muscle Damage Biomarkers in a Phase 1 Trial in Becker Muscular Dystrophy** Joanne Donovan, MD, PhD
10:30 - 10:45 am	**Phase 2 Multiple-Ascending-Dose Study of SRP-5051 PPMO in Patients with DMD Amenable to Exon 51 Skipping: Part A Results** Darko Stevanovic
10:50 - 11:05 am	**Safety, β-Sarcoglycan Expression, and Functional Outcomes From Systemic Gene Transfer of rAAVrh74.MHCK7.hSGCB in LGMD2E/R4** Louise Rodino-Klapac, PhD
11:10 - 11:25 am	**RESCUE-ALS Trial Results: A Phase 2, Randomized, Double-Blind, Placebo-Controlled Study of CNM-Au8 to Slow Disease Progression in ALS** Robert Glanzman, MD, FAAN
11:30 - 11:45 am	**The FIGHT DMD Trial – An FDA Sponsored Study Aimed to Prevent the Cardiomyopathy Associated with DMD with an Oral Thromboxane Receptor Antagonist** Ines Macias-Perez, PhD
11:50 - 12:05 pm	**Annualized Rates of Change from A Phase 2, Randomized, Double-Blind, Placebo-Controlled, 48-Week Study of Losmapimod in Subjects with FSHD: ReDUX4** Rabi Tawil
12:10 - 12:25 pm	**Long-term Follow-up of Cipaglucosidase Alfa/Miglustat in Ambulatory Patients with Pompe disease: An Open-Label Phase I/II Study (ATB200-02)** Barry Byrne, Md, PhD

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Wednesday, March 16, 2022 cont.	
8:30 – 11:15 AM	**Novel Therapeutic Approaches to Treat Neuromuscular Diseases**
8:30 - 8:45 am	**Therapeutic Targeting of a Pathological microRNA in DMD** Anders Naar, PhD
8:50 - 9:05 am	**Use of Homology-Independent Targeted Integration Gene Editing to Correct Proximal Hotspot DMD Gene Mutations** Anthony Stephenson
9:10 - 9:25 am	**Development of a Miniaturized CRISPR Activation for a Single AAV Delivery Method for LAMA2-CMD** Dwi Kemaladewi, PhD
9:30 - 9:45 am	**DUX4 siRNA Optimization for the Development of an Antibody-Oligonucleotide Conjugate (AOC TM) for the Treatment of FSHD** Barbora Malecova, PhD
9:45 - 10:00am	**Break**
10:00 - 10:15am	**Identification of a Novel JNK Inhibitor for an Alternative Treatment of Spinal Muscular Atrophy** Laxman Gangwani
10:15 - 10:30 am	**A Novel Calcium Channel Gating Modifier That Improves Neuromuscular Transmitter Release and Strength in Spinal Muscular Atrophy Model Mice** Stephen Meriney, PhD
10:30 - 10:45 am	**Blocking Translation to Rescue ALS/FTD Phenotypes Associated with C9ORF72 Repeat Expansion** Xin Jiang
10:45 - 11:00 am	**Mineralocorticoid Receptor Signaling in the Microenvironment of Dystrophic Skeletal Muscles** Zachry Howard, PhD
11:00 - 11:15am	**Break**
11:15 AM – 12:00 PM	**Insights into Neuromuscular Disease Mechanism**
11:15 - 11:30am	**TRIM72/MG53 Autoantibodies Represent a Novel Disease Mechanism Compromising Sarcolemmal Membrane Repair in Limb Girdle Muscular Dystrophy** Noah Weisleder, PhD
11:30 - 11:45 am	**Single-cell Transcriptomics Identify Distinct Features of Fibro/Adipogenic Progenitors From Healthy and Dystrophic Muscles** Brian Uapinyoying, PhD
11:45 AM - 12:00 PM	**Use of an Exercise Challenge System to Define a Universal Proteomic Signature of Muscle Injury in Adult Individuals with Diverse Inherited Myopathies** Ben Barthel, PhD

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Wednesday, March 16, 2022 cont.	
8:30 – 12:20 PM	**Strategies to Improve Oligonucleotide Delivery**
8:30 - 8:45 AM	**Enhanced Exon Skipping and Dystrophin Production in a Mouse Model of Duchenne Muscular Dystrophy with EEV-PMO Treatment** Mahasweta Girgenrath, PhD
8:50 - 9:05 AM	**Unlocking the Potential of Oligonucleotide Therapeutics for Duchenne Muscular Dystrophy Through Enhanced Delivery** James McArthur, PhD
9:10 - 9:25 AM	**The Mitochondria-Targeting Peptide Elamipretide Potentiates Dystrophin Expression Induced by an Exon-Skipping Morpholino in the MDX Mouse Model** Dave Brown, PhD
9:30 - 9:45 AM	**A PATrOL™- Enabled Investigational Genetic Therapy for DM1: Mouse Pharmacokinetics, Biodistribution, and CNS Penetration after Systemic Administration** Sandra Rojas-Caro, MD
9:45 - 10:00 AM	**Break**
10:00 - 11:25 AM	**Towards Clinical Trials: IND-enabling Studies and Outcome Measures**
10:00 - 10:15 AM	**Multicentered IND-Enabling Efficacy and Safety Studies are Highly Promising for SMARD1/CMT2S Gene Therapy** Kathrin Meyer, PhD
10:20 - 10:35 AM	**The DMD-HI & DMDCR-HI: Development and Validation of Two Novel Duchenne Muscular Dystrophy Outcome Measures** Jennifer Weinstein, MS
10:40 - 10:45 AM	**Clinical Trial Readiness to Solve Barriers to Drug Development in FSHD (ReSolve): Baseline Characteristics** Jeffrey Statland, MD
10:50 - 11:05 AM	**Clinical Outcome Assessments in Limb Girdle Muscular Dystrophy R1/2A** Stephanie Poelker, PT, DPT
11:10 - 11:25 AM	**Break**
11:25 AM – 12:20 PM	**Diagnosis and Clinical Management for NMDs** Room Location: Hermitage AB
11:25 - 11:40 AM	**Real-World Outcomes of Disease-Modifying Treatments in Pediatric Patients with Spinal Muscular Atrophy: Interim Analysis of a US Chart Review Study** Omar Dabbous
11:45 AM - 12:00 PM	**Meeting the Care Coordination Needs of Complex Therapies for Rare Neuromuscular/Neuro-Genetic Disorders: The Development of a Complex Drug Program** Taylor Schwab
12:05 - 12:20 PM	**Evaluation of the Diagnostic Capabilities of Nanopore Long Read Sequencing and Splice Variant Analysis in Muscular Dystrophies** Christine Bruels, PhD
1 – 3:50 PM	**Highlight Sessions** Room Location: Tennessee Ballroom
1:00 - 1:20 PM	**Relationship of ALS Genes and Single-Nucleotide Polymorphisms with Clinical Outcomes in NurOwn Phase 3 ALS Clinical Trial** Merit Cudkowicz, MD
1:25 - 1:45 PM	**Directed Evolution of a Family of AAV Capsid Variants Enabling Potent Muscle-directed Gene Delivery Across Species** Mohammadsharif Tabebordbar, PhD

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Wednesday, March 16, 2022 cont.	
1:50 - 2:10 PM	**Regulatory T-Lymphocyte and IL-2 Combination Therapy is Safe, Tolerable, and Biologically Active in Persons with Amyotrophic Lateral Sclerosis** Jason Thonhoff, MD, PhD
2:15 - 2:35 PM	**A Collaborative Analysis by Clinical Trial Sponsors and Academic Experts of Anti-Transgene SAEs in Studies of Gene Therapy for DMD** Carsten Bonnemann, MD & Beth Belluscio, MD, PhD
2:40 - 3:00 PM	**A Phase 2 Clinical Trial Evaluating the Safety and Efficacy of Delandistrogene Moxeparvovec (SRP-9001) in Patients with Duchenne Muscular Dystrophy** Jerry Mendell, MD
3:05 - 3:25 PM	**One Year Data From Ambulatory Boys in a Phase 1b, Open-label Study of Fordadistrogene Movaparvovec (PF-06939926) for Duchenne Muscular Dystrophy (DMD)** Russell Butterfield, MD, PhD
3:30 - 3:50 PM	**IGNITE DMD Phase I/II Study of SGT-001 Microdystrophin Gene Therapy for DMD: 2-Year Outcomes Update** Roxana Donisa Dreghici, MD