



Conference Agenda

ALL CONFERENCE SESSION TIMES LISTED ARE IN CENTRAL STANDARD TIME

Sunday, March 13, 2022

9 AM – 5 PM	Insights in Research Investor Summit (IRIS)
3 – 6 PM	MDA Scholars Networking Session
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 Industry Forum Breakfast Presented by NS Pharma
8:30 – 9:30 AM	Opening and Keynote Address
10 AM – 8 PM	Exhibit Hall and Poster Sessions Open
10 AM – 12 PM	Latest Developments Across the NMD Registry Data Landscape Rayne Rodgers, MPH (Chair) 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 Industry Forum Lunch Presented by Pfizer Industry Forum Lunch Presented by Sarepta
1:30 – 2:30 PM	Lab to Life: LGMD Nicolas Johnson, MD, MS-CI, FAAN (Chair) 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.
1:30 – 3:30 PM	New Gene Discoveries in NMD (CME Accredited Session) Alan Beggs, PhD (Chair) 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) Session will explore implementation of targeted therapies in clinical trials, molecular understanding of FSHD, and how we measure changes in future clinical trials.

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Monday, March 14, 2022 cont.

4 – 5 PM	<p>Lab to Life: MG Henry Kaminski, MD (Chair)</p> <p>Session will review the journey of treatment to patient from identification of unmet need to prescription of the drug and access for all patients.</p>
	<p>Functional/PROs/Composite Clinical Trial Endpoints in DMD (CME Accredited Session) Erik Henricson, PhD, MPH (Chair)</p> <p>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.</p>
5 – 6 PM	<p>Lab to Life: Congenital Myopathies/Dystrophies Chamindra Konersman, MD (Chair)</p> <p>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.</p>
	<p>Best Practices in Respiratory Care (CME Accredited Session) Brad Troxler, MD (Chair)</p> <p>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.</p>
6 – 8 PM	<p>Poster & Networking Reception</p>

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Tuesday, March 15, 2022

7 - 8 AM	<p>Industry Forum Breakfast Presented by Amylyx Industry Forum Breakfast Presented by Fulcrum</p>
8:30 – 10 AM	<p>Mitochondrial Interventions: Small Molecule and Gene Therapy Carlos Moraes, PhD (Chair)</p> <p>Session will explore a few approaches with great clinical potential that use small molecules or genetic manipulation.</p>
	<p>Promoting Diversity in Research Susan Apkon, MD (Chair)</p> <p>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.</p>
	<p>Telehealth Learnings in Clinical Care and Research: Learnings from the Pandemic (CME Accredited Session) Dustin Nowacek, MD (Chair)</p> <p>Session will discuss telehealth experiences, identify the benefits of telehealth, and recognize the limitations of virtual care.</p> <p>When Gut Feelings Aren't Helping, Revisited: Navigating Complicated Ethics in Neuromuscular Care in 2022 (CME Accredited Session) John Brandsema, MD (Chair)</p> <p>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.</p>
10 AM – 8 PM	<p>Exhibit Hall and Poster Sessions Open</p>
10:30 AM – 12 PM	<p>Small Molecule Approaches to Overcoming Muscle Wasting Helen Blau, PhD (Chair)</p> <p>Session will discuss small molecule drugs that counter muscle loss and show promise in treating SMA, DMD, and BMD.</p>
	<p>More Data, Lower Burden – Digital Outcome Measures in Neuromuscular Disease Therapy Development James Berry, MD, MPH (Chair)</p> <p>Session will explore digital outcomes measures in neuromuscular disease therapy development.</p>

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Tuesday, March 15, 2022 cont.

<p>10:30 AM – 12 PM</p>	<p>Natural History and Trial Readiness for Children with Charcot Marie Tooth Disease (CME Accredited Session) Michael Shy, MD (Chair)</p> <p>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.</p> <hr/> <p>Facilitating Multi-Practice Collaboration to Optimize Patient Care (CME Accredited Session) Lora Clawson, MSN, CRNP (Chair)</p> <p>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.</p>
<p>12 – 1:30 PM</p>	<p>Industry Forum Lunch Presented by Genentech Industry Forum Lunch Presented by PTC Industry Forum Lunch Presented by Sarepta</p>
<p>1:30 - 2:30 PM</p>	<p>Building MD Team in NMD (CME Accredited Session) Yaacov Anziska, MD (Chair)</p> <p>Session will address experiences from a range of specialists about coordinating with the neurologist to provide the best care for our MDA neuromuscular patients.</p>
<p>1:30 – 3:30 PM</p>	<p>Clinical Trial Design for Rare Neuromuscular Disorders – Where Are We Headed? Diana Barucha-Goebel, MD (Chair)</p> <p>Session will explore issues common to trials in rare disease such as phenotypic pleiotropy, genetic heterogeneity, combination therapies and n=1 trials.</p> <hr/> <p>Progress in Brain Interface Technology Daniel Rubin, MD, PhD (Co-Chair) Leigh Hochberg, MD, PhD (Co-Chair)</p> <p>Session will review recent progress, with leaders in the field sharing their vision for the clinical applications and future of these important technologies.</p> <hr/> <p>Treatment Decisions for Disorders with Multiple Therapies (CME Accredited Session) Julie Parsons, MD (Chair)</p> <p>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</p>

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Tuesday, March 15, 2022 cont.

<p>2:30 – 3:30 PM</p>	<p>The Resilient Practitioner (CME Accredited Session) Deidre Devier, PhD (Chair)</p> <p>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.</p>
<p>4 – 6 PM</p>	<p>CRISPR – The Next Generation Eric Olson, PhD (Chair)</p> <p>Session will focus on recent advances and future challenges in translating CRISPR gene editing for genetic myopathies from animal models toward human translation.</p> <p>Personalized Medicine/Regulatory Approaches for Rare Disease Teepu Siddique, MD (Chair)</p> <p>Impact of Gene Transfer Therapy on Long-Term Multidisciplinary Care (CME Accredited Session) Emma Ciafaloni, MD</p> <p>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.</p> <p>Business Operations for NMD Clinics (CME Accredited Session) Daragh Heitzman, MD</p> <p>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.</p>
<p>6 – 8 PM</p>	<p>Exhibits & Poster Networking Reception (Exhibit Hall)</p>



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Wednesday, March 16, 2022	
8:30 – 11 AM	Clinical Trial Presentations
11:30 AM – 3 PM	Clinical Trial Presentations