

## 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	<b>Poster Sessions Open</b>
9:30 – 10 AM	<b>Keynote Presentation</b>
10 AM – 6 PM	<b>Exhibit Hall Open</b>
10 – 11 AM	<b>New Biomarkers in Neuromuscular Disease Research</b> Elizabeth McNally, MD, PhD (Chair)
11 AM – 12 PM	<b>MOVR &amp; Real World Evidence</b> Rayne Rodgers, MPH (Chair)
12 – 1:30 PM	<b>Industry Forum Lunch Presented by Alexion Pharmaceuticals</b>
	<b>Industry Forum Lunch Presented by Genentech</b>
	<b>Industry Forum Lunch Presented by Novartis Gene Therapies</b>
1:30 – 3 PM	<b>Axonal Degeneration and Transport Defects</b> Michael Shy, MD (Chair)
	<b>Therapeutic Considerations for Dominant Neuromuscular Diseases</b> Beth Barton, PhD (Chair)
3 – 4:30 PM	<b>Gene Editing in Neuromuscular Disease</b> Eric Olson, PhD (Chair)
	<b>Innovative Modeling in Neuromuscular Disease</b> Jamshid Arjomand, PhD (Chair)
4:30 – 6 PM	<b>Non-viral Delivery in Neuromuscular Disease</b> Stanley C. Froehner, PhD (Chair)
6 – 8 PM	<b>MDA Scholars Virtual Meeting (Invitation Only)</b>
6 – 9 PM	<b>Poster Session</b>

<b>TUESDAY, MARCH 16, 2021</b>	
8 – 9 AM	<b>Keynote &amp; Welcome Presentation</b>
6 AM – 9 PM	<b>Exhibit &amp; Poster Hall Open</b>
9 – 10 AM	<b>Lab-to-Life: Inflammatory Myopathies</b> Thomas Lloyd, MD, PhD (Chair)
10 – 11 AM	<b>Lab-to-Life: Mitochondrial Diseases including Friedreich's Ataxia</b> Carlos Moraes, PhD (Chair)
11 AM – 12 PM	<b>Lab-to-Life: Spinal and Bulbar Muscular Atrophy</b> Kenneth (Kurt) Fischbeck, MD (Chair)
12 – 1:30 PM	<b>Industry Forum Lunch Presented by Amylyx Pharmaceuticals</b>
	<b>Industry Forum Lunch Presented by Biogen</b>
	<b>Industry Forum Lunch Presented by Sarepta Therapeutics</b>
1:30 – 2:30 PM	<b>Lab-to-Life: Congenital MD</b> Kevin Campbell, PhD (Chair)
2:30 – 4 PM	<b>Barriers to Clinical Trial Participation</b> John Day, MD, PhD (Chair)
	<b>Optimizing ALS Care &amp; Support for Patients &amp; Families</b> Lora Clawson (Chair)
	<b>The Changing Regulatory Environment: Impact on Neuromuscular Disease Drug Development</b> James Valentine (Chair)
4:30 – 6 PM	<b>When Gut Feelings Aren't Helping, Revisited: Navigating Complicated Ethics in Neuromuscular Care in 2021</b> John Brandsema, MD (Chair)
	<b>Rare Disease Initiatives</b> Barry Byrne, MD, PhD (Chair)
	<b>Reproductive Health, Family Planning &amp; Parenting</b> Elicia Estrella, MS, CGC, LGC (Chair)
6 – 9 PM	<b>Exhibit &amp; Poster Session</b>

<b>WEDNESDAY, MARCH 17, 2021</b>	
6:30 – 9 AM	<b>Poster Session</b>
9 AM – 6 PM	<b>Exhibit &amp; Poster Hall Open</b>
9 – 10:30 AM	<b>Impact of COVID-19 on Neuromuscular Disease Care</b> Erin Springer, RN, MSN (Chair)
	<b>Measuring Progression of Disease &amp; Selecting Endpoints</b> Erik Henricson, PhD, MPH (Chair)
10:30 AM – 12 PM	<b>Clinical Trials in the Age of COVID-19</b> Jeffrey Statland, MD (Chair)
	<b>Navigating SMA Treatment Decisions</b> Perry Shieh, MD, PhD (Chair)
12 – 1:30 PM	<b>Industry Forum Lunch Presented by Biogen</b>
	<b>Industry Forum Lunch Presented by PTC Therapeutics</b>
	<b>Industry Forum Lunch Presented by Sarepta Therapeutics</b>
1:30 – 3 PM	<b>Telehealth in Neuromuscular Disease Care</b> Mario Saporta, MD, PhD, MBA, FAAN (Chair)
	<b>Therapies Targeting Pulmonary Function in Neuromuscular Disease</b> Jonathan Finder, MD (Chair)
3 – 4:30 PM	<b>Case Studies in Access to New Therapies</b> Claudia Chiriboga, MD, MPH (Chair)
	<b>A Multidisciplinary Approach to Clinical Trials; The Role of Physical and Occupational Therapists in Neuromuscular Research</b> Sally Dunaway Young, PT, DPT (Chair)
4:30 – 6 PM	<b>Cardiomyopathy Across the Neuromuscular Disease Spectrum</b> Linda Cripe, MD (Chair)
	<b>Ethical Considerations in Gene Therapy Trials</b> Kevin Flanigan, MD (Chair)

<b>THURSDAY, MARCH 18, 2021</b>	
9 AM – 12 PM	<b>Oral Presentations from Abstract Submissions</b>
12 – 1:30 PM	<b>Industry Forum Lunch Presented by NS Pharma</b>
	<b>Industry Forum Lunch Presented by Sanofi</b>
	<b>Industry Forum Lunch Presented by Solid Biosciences</b>
1:30 – 6 PM	<b>Oral Presentations from Abstract Submissions</b>
7 PM	<b>Meeting Adjourned</b>