MDA CLINICAL & SCIENTIFIC CONFERENCE 2021 MARCH 15-18

# Conference Agenda

**ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN DAYLIGHT TIME**

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

| MONDAY, MARCH 15, 2021 Cont. | |
|---|---|
| 3 – 4:30 PM | **Gene Editing in Neuromuscular Disease**<br>Eric Olson, PhD (Chair)<br>Charles Gersbach, PhD<br>Erik Sontheimer, PhD |
| | **Innovative Modeling in Neuromuscular Disease**<br>Jamshid Arjomand, PhD (Chair)<br>Robert Bloch, PhD<br>Clive Svendsen, PhD<br>Mina Gouti, PhD<br>Eleonora Maino, M.Sc, |
| 4:30 – 6 PM | **Non-viral Delivery in Neuromuscular Disease**<br>Stanley C. Froehner, PhD (Chair)<br>Oxana Beskrovnaya, PhD<br>James Dahlman, PhD<br>Melissa Spencer, PhD<br>Nicholas Whitehead, PhD |
| 6 – 8 PM | MDA Scholars Virtual Meeting (Invitation Only) |

MDA CLINICAL & SCIENTIFIC CONFERENCE 2021 MARCH 15-18

# Conference Agenda

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

| TUESDAY, MARCH 16, 2021 Cont. | |
|---|---|
| 2:30 – 4 PM | **Barriers to Clinical Trial Participation**<br>(CME Accredited Session)<br>John Day, MD, PhD (Chair)<br>Kate Eichinger, PT, PhD, NCS<br>Colleen Labbadia, Parent<br>Catherine Seiner, PT, MHS |
| | **Optimizing ALS Clinical Care and Clinical Research in an MDA ALS Care Center in 2021**<br>(CME Accredited Session)<br>Lora Clawson MSN, CRNP (Chair)<br>Kristen Riley, PhD<br>Rachel Slusher, PT, DPT<br>Alpa Uchil, MSN, MPH, CRNP |
| | **The Changing Regulatory Environment: Impact on Neuromuscular Disease Drug Development**<br>James Valentine (Chair)<br>Teresa Buracchio, MD<br>Michelle Campbell, PhD<br>Jeffrey Castellli, PhD<br>Billy Dunn, MD<br>Mindy Leffler<br>Paul Melmeyer<br>Lei Xu, MD, PhD |
| 4:30 – 6 PM | **When Gut Feelings Aren't Helping, Revisited: Navigating Complicated Ethics in Neuromuscular Care in 2021**<br>(CME Accredited Session)<br>John Brandsema, MD (Chair)<br>Alyssa Burgart, MD<br>Tom Crawford, MD<br>Barkri Elsheikh, MD<br>Julie Parsons, MD |
| | **Rare Disease Initiatives**<br>Barry Byrne, MD, PhD (Chair)<br>Alan Beggs, PhD<br>Philip John (PJ) Brooks, PhD<br>Jane Larkindale, Dphil<br>Laura Brod Hameed |
| | **Reproductive Health, Family Planning & Parenting**<br>(CME Accredited Session)<br>Elicia Estrella, MS, CGC, LGC (Chair)<br>Deodonne Bhattarai, Parent<br>Judy Jackson, MS, LCGC<br>Sharyn Lincoln, MS, LCGC<br>Alexandrea Lisi, BA Biology |
| 6 – 9 PM | Dedicated Exhibit Hall & Poster Session<br>*This time is dedicated to viewing Exhibit Booths & Posters exclusively*<br>*Exhibit Booths & Posters to be staffed in real time (if possible)* |

MDA CLINICAL & SCIENTIFIC CONFERENCE 2021 MARCH 15-18

# Conference Agenda

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

**ALL CONFERENCE SESSION TIMES LISTED ARE IN EASTERN DAYLIGHT TIME**

| WEDNESDAY, MARCH 17, 2021 Cont. | |
|---|---|
| 3 – 4:30 PM | **Case Studies in Access to New Therapies**<br>(CME Accredited Session)<br>Claudia Chiriboga, MD, MPH, FAAN (Chair)<br>Anne Connolly, MD, FAAN<br>Basil Darras, MD<br>Matt Harms, MD |
| | **A Multidisciplinary Approach to Clinical Trials; The Role of Physical and Occupational Therapists in Neuromuscular Research**<br>(CME Accredited Session)<br>Sally Dunaway Young, PT, DPT (Chair)<br>Jacqueline Montes, PT, Ed (Chair)<br>Kate Eichinger, PhD, DPT<br>Tim Estilow, OTR/L<br>Sara Feldman, PT, DPT, ATP |
| 4:30 – 6 PM | **Cardiomyopathy Across the Neuromuscular Disease Spectrum**<br>(CME Accredited Session)<br>Linda Cripe, MD (Chair)<br>Andreas Barth, MD, PhD, FAHA<br>Kan Hor, MD<br>Chet Villa, MD |
| | **Ethical Considerations in Gene Therapy Trials**<br>(CME Accredited Session)<br>Kevin Flanigan, MD (Chair)<br>Diana Bharucha-Goebel, MD<br>Robert Brown, MD, DPhil<br>Barry Byrne, MD, PhD<br>Matthew Kendall |
| 6 – 9 PM | Dedicated Exhibit Hall Session<br>*This time is dedicated to viewing Exhibit Booths exclusively*<br>*Exhibit Booths to be staffed in real time (if possible)* |

MDA CLINICAL & SCIENTIFIC CONFERENCE 2021 MARCH 15-18

# Conference Agenda

| THURSDAY, MARCH 18, 2021 | |
|---|---|
| 6:30 AM – 9 AM | Dedicated Exhibit Hall & Poster Session<br>*This time is dedicated to viewing Exhibit Booths & Posters exclusively*<br>*Exhibit Booths & Posters to be staffed in real time (if possible)* |
| 9 AM – 6 PM | Exhibit Hall & Poster Session Open |
| 9 AM - 12 PM | **Oral Presentations from Abstract Submissions Part I** |
| 9:00 – 9:15 AM | **WVE-004, an Investigational Stereopure Antisense Oligonucleotide for the Treatment of Amyotrophic Lateral Sclerosis (ALS) and Frontotemporal Dementia**<br>Yuanjing Liu, PhD |
| 9:15 – 9:30 AM | **Slc20a1/Pit1 and Slc20a2/Pit2 are Essential for Normal Skeletal Myofiber Function and Survival**<br>Clemens Bergwitz, MD |
| 9:30 - 9:45 AM | **Resolution of Causality in Myotonic Dystrophy, Type 1 (DM1) via a PATrOL™-Enabled Therapy**<br>Dietrich Stephan, PhD |
| 9:45 – 10:00 AM | **AAV Gene Therapy for TNNT1-Associated Nemaline Myopathy**<br>Eleonora D 'Ambrosio, MD |
| 10:00-10:15 AM | **Dendrimer-Conjugated GCPII Inhibitor Selectively Targets Activated Macrophages in SOD1G93A Muscle Which Delays Functional Loss and Denervation**<br>Carolyn Tallon, PhD |
| 10:15 – 10:30 AM | **Galectin-1 Improves Sarcolemma Repair and Decreases Inflammatory Response in LGMD2B Models**<br>Pam Van Ry |
| 10:30 – 10:45 AM | **Micro-dystrophin Gene Therapy Delivery and Therapeutic Plasma Exchange in Non-Human Primates**<br>Rachael Potter |
| 10:45-11:00 AM | **A Hotspot in CAPN3 as the Likely Cause of Autosomal-Dominant Calpainopathy (LGMDD4)**<br>Michelle Allen-Sharpley, MD, PhD |
| 11:00 – 11:15 AM | **Molecular Markers of Clinical Severity in Becker Muscular Dystrophy from a Remote Study**<br>Utkarsh Dang, PhD |
| 11:15 – 11:30 AM | **A Pilot Study Using a Novel Wearable Sensor to Examine Physical Activity in Individuals with Charcot Marie Tooth Disease 1A**<br>Katy Eichinger, PT, PhD |
| 11:30 – 11:45 AM | **Quantitative Muscle Analysis in FSHD Using Whole-Body MRI: Composite Muscle Measurements for Cross-Sectional Analysis**<br>Michelle Mellion, MD |
| 12 – 1:30 PM | **Industry Forum Lunch Presented by NS Pharma**<br>A Treatment Option for Patients with Duchenne Muscular Dystrophy Amenable to EXON 53 Skipping |
| | **Industry Forum Lunch Presented by Sanofi**<br>The Challenges of Newborn Screening for Neuromuscular Disorders<br>The Pompe Disease Experience |
| | **Industry Forum Lunch Presented by Solid Biosciences**<br>Real World Outcome Measures in Duchenne Muscular Dystrophy:<br>Current and Novel Assessments of Meaningful Patient Benefit |

| THURSDAY, MARCH 18, 2021 Cont. | |
|---|---|
| 1:30 – 6 PM | **Oral Presentations from Abstract Submissions Part II** |
| 1:30 – 1:45 PM | **Self and Parent Perceptions of Behavior and Quality of Life in Boys with Duchenne Muscular Dystrophy**<br>Anna Jesus, MD Mbe |
| 1:45 – 2:00 PM | **Predictors of Functional Outcomes in Patients with Facioscapulohumeral Muscular Dystrophy**<br>Natalie Katz, MD PhD |
| 2:00 – 2:15 PM | **Apitegromab, a Novel High-Affinity Anti-proMyostatin Monoclonal Antibody for Treating Spinal Muscular Atrophy: Results of a Phase 2 Interim Analysis**<br>Amy Place, PhD, MBA, MS, RD, CLT |
| 2:15 – 2:30 PM | **Predictive Factors of Nusinersen Treatment Response in Infantile-onset SMA: Results from the ENDEAR/SHINE Studies**<br>Laurent Servais, MD, PhD |
| 2:30 – 2:45 PM | **Kinetics of Early Antibody Response and Immunotoxicity Following Zolgensma for the Treatment of SMA Type 1**<br>Stephanie Salbarria |
| 2:45 – 3:00 PM | **SUNFISH Part 2: 24-Month Efficacy and Safety of Risdiplam in Patients with Type 2 or Non-Ambulant Type 3 Spinal Muscular Atrophy (SMA)**<br>Maryam Oskoui, MD, MSc, FRCPC, FAAN |
| 3:00 – 3:15 PM | **Nusinersen in Children with SMA Who Received Onasemnogene Abeparvovec: Design of the Phase 4, Open-Label RESPOND Study**<br>Julie Parsons, MD |
| 3:15 – 3:30 PM | **Long-Term Follow-Up (LTFU) of Onasemnogene Abeparvovec Gene Therapy in Spinal Muscular Atrophy (SMA)**<br>Jerry R. Mendell, MD |
| 3:30 – 3:45 PM | **A Randomized, Double-Blind, Placebo-Controlled, Gene-Delivery Clinical Trial of rAAVrh74.MHCK7. Micro-Dystrophin for Duchenne Muscular Dystrophy**<br>Jerry R. Mendell, MD |
| 3:45 – 4:00 PM | **Lower Long-Term Risk of Death or Permanent Ventilation and First Hospitalization Among Participants with ALS Receiving AMX0035 in the CENTAUR Trial**<br>Sabrina Paganoni, MD, PhD |
| 4:00 – 4:15 PM | **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**<br>Barry Byrne, MD, PhD |
| 4:15 – 4:30 PM | **Onasemnogene Abeparvovec Gene Therapy in Presymptomatic Spinal Muscular Atrophy (SMA): SPR1NT Study Update in Children with 3 Copies of SMN2**<br>Kevin Strauss, MD |
| 4:30 – 4:45 PM | **Safety, β-sarcoglycan Expression, and Functional Outcomes from Systemic Gene Transfer of rAAVrh74.MHCK7.SGCB in Limb Girdle Muscular Dystrophy Type 2E**<br>Louise Rodino-Klapac, PhD |
| 4:45 – 5:00 PM | **Safety and Efficacy of PF-06939926 Gene Therapy in boys with Duchenne Muscular Dystrophy: Update on data from the Phase 1b Study**<br>Beth Belluscio, MD, PhD |
| 5:00 – 5:15 PM | **Early Intervention with Edaravone in Study 19 was Associated with Decreased Hospitalization, Tracheostomy, Ventilation, and Death in Patients with ALS**<br>Benjamin Brooks, MD |
| 5:15 – 5:30 PM | **2.5-years of Vamorolone Treatment in Duchenne Muscular Dystrophy: Results of an Open Label Long-Term Extension**<br>Utkarsh Dang, PhD |

| THURSDAY, MARCH 18, 2021 Cont. | |
|---|---|
| 5:30 – 5:45 PM | **Efficacy and safety of cipaglucosidase alfa/miglustat versus alglucosidase alfa/placebo in late-onset Pompe disease (LOPD): A phase 3 trial (PROPEL)**<br>Tahseen Mozaffar, MD FAAN, FANA |
| 5:45 – 6:00 PM | **Delay in Duchenne Muscular Dystrophy Progression with Eteplirsen: Longer Time to Loss of Ambulation Versus Standard of Care**<br>Joel Iff, PharmD, PhD |
| 6:00 – 6:15 PM | **A novel enhanced delivery oligonucleotide (EDO) therapeutic demonstrates considerable potential in treating myotonic dystrophy type 1**<br>Ashling Holland, PhD |
| 6:15 PM | Meeting Adjourned |