

2018 MDA[®] Clinical Conference

Muscular Dystrophy Association • #MDAClinicCon • mda.org

Program Agenda

Sunday, March 11, 2018

- 4 – 6:30 p.m. Registration Regency Foyer
- 5 – 6 p.m. Keynote Speaker Regency CDEF
Changing the Playing Field for Children with Neuromuscular Disease: Clinical Gene Therapy for SMA and DMD
Jerry R. Mendell, M.D., Nationwide Children's Hospital
- 6 – 8 p.m. Champions Welcome Reception Independence Center A

Monday, March 12, 2018

- 6 – 6:45 a.m. MDA Team Momentum Fun Run/Muscle Walk Lobby
- 7 – 7:50 a.m. Breakfast Independence Center B
- 7 – 7:50 a.m. United States National Amyotrophic Lateral Sclerosis (ALS) Registry Breakfast Potomac III IV V VI
Paul Mehta, M.D., National ALS Registry, CDC/Agency for Toxic Substances and Disease Registry
- 8 – 8:30 a.m. Opening Session Regency CDEF

Session #1: What's New In Regency CDEF
Session Chair: **Rabi Tawil**

- 8:30 – 9 a.m. *Amyotrophic Lateral Sclerosis (ALS)*
James D. Berry, M.D., M.P.H., Massachusetts General Hospital
- 9 – 9:30 a.m. *Duchenne Muscular Dystrophy (DMD)*
Kathryn Wagner, M.D., Ph.D., Kennedy Krieger Institute
- 9:30 – 10 a.m. *Facioscapulohumeral Muscular Dystrophy (FSHD)*
Rabi Tawil, M.D., University of Rochester Medical Center
- 10 – 10:30 a.m. Coffee Break Independence Center A
- 10:30 – 11 a.m. *Spinal Muscular Atrophy (SMA)*
Richard S. Finkel, M.D., Nemours Children's Hospital

11 – 11:30 a.m. *Myotonic Dystrophy (DM)*
Charles Thornton, M.D., University of Rochester Medical Center

11:30 – 12 p.m. *Myasthenia Gravis (MG)*
James F. Howard, Jr., M.D., FAAN, The University of North Carolina at Chapel Hill

12 – 1:30 p.m. Lunch Independence Center B

Session #2: Advances in Personalized Medicine Regency CDEF
Session Chair: Eric Olson

1:30 – 2:10 p.m. *Correction of Duchenne Muscular Dystrophy by Genome Editing*
Eric Olson, Ph.D., UT Southwestern Medical Center

2:10 – 2:50 p.m. *Scaling Up Genomic Diagnosis of Muscle Disorders*
Daniel MacArthur, Ph.D., Broad Institute of Harvard and MIT

2:50 – 3:30 p.m. *Advancing the Role of Digital Health in Neuromuscular Disease*
Murray Aitken, MBA, IQVIA Institute for Human Data Science

3:30 – 4 p.m. Coffee Break..... Independence Center A

Session #3: MDA Flagship Initiatives Regency CDEF

4 – 4:45 p.m. *MDA ONEvoice*
Kristin Stephenson, M.H.A., J.D., Muscular Dystrophy Association

4:45 – 5:30 p.m. *MDA MOVR Registry*
Grace K. Pavlath, Ph.D., Muscular Dystrophy Association

5:30 - 6 p.m. Break

6 – 8 p.m. MDA Champions Networking Reception Independence Center A

Tuesday, March 13, 2018

7 – 8 a.m. Breakfast Independence Center B

Sessions #4, 5 and 6 are Concurrent Sessions

Session #4: Best Practices in Cardiac Care Regency EF
Session Chair: Elizabeth McNally

- 8 – 8:30 a.m. *Cardiac Management for the Muscular Dystrophy Lifespan*
Elizabeth McNally, M.D., Ph.D., Northwestern University, Feinberg School of Medicine
- 8:30 – 9 a.m. *Cardiac Screening, Surveillance and Management in the Myotonic Dystrophy Patient*
Jennifer Strande, M.D., Ph.D., Medical College of Wisconsin
- 9 – 9:30 a.m. *Management of Neuromuscular-Associated Cardiomyopathy*
Pradeep P.A. Mammen, M.D., F.A.C.C., FAH, UT Southwestern Medical Center
- 9:30 – 10 a.m. *Arrhythmia Monitoring and Arrhythmia Management in Muscular Dystrophies*
Andreas Barth, M.D., Ph.D., Johns Hopkins University, School of Medicine

Session #5: Best Practices in Physical Therapy in SMA Regency CD
Session Chair: Jacqueline Montes

- 8 – 8:30 a.m. *Best Practices for Rehabilitation Management for Type 1 Spinal Muscular Atrophy (SMA)*
Allan M. Glanzman, P.T., D.P.T., PCS, The Children's Hospital of Philadelphia
- 8:30 – 9 a.m. *Best Practices in Rehabilitation for Spinal Muscular Atrophy (SMA) in Patients with Later Onset SMA*
Leslie Nelson, P.T., UT Southwestern Medical Center
- 9 – 9:30 a.m. *Considerations for Rehabilitation Management for Adult Spinal Muscular Atrophy (SMA)*
Sally Dunaway Young, P.T., D.P.T., Columbia University Medical Center
- 9:30 – 10 a.m. *Exercise in Spinal Muscular Atrophy*
Jacqueline Montes, P.T., Ed.D., NCS, Columbia University Medical Center

Session #6: Best Practices in Bone Health Washington Room
Session Chair: Mathula Thangarajh

- 8 – 8:30 a.m. *Partnering for Better Bone Health Outcomes in Neuromuscular Diseases*
Mathula Thangarajh, M.D., Children's National Health System
- 8:30 – 9 a.m. *Partnering for Better Bone Health Outcomes in Neuromuscular Diseases*
Susan D. Apkon, M.D., Seattle Children's Hospital
- 9 – 9:30 a.m. *Bone Health: PM&R View*
Dennis Matthews, M.D., Children's Hospital Colorado

9:30 – 10 a.m. *Clinical Management in Bone Health: Current Approach and Future Directions*
Alison Boyce, M.D., National Institutes of Health

10 – 10:30 a.m. Coffee Break..... Independence Center A

Sessions #7 and 8 are Concurrent Sessions

Session #7: Best Practices in Technology in Health Care.....Regency EF
Session Chair: Laura Hagerty

10:30 – 11 a.m. *Accelerating Discovery in Neurological Disorders with Cognitive Computing*
Elenee Argentinis, J.D., Watson Health Life Sciences Solutions

11 – 11:30 a.m. *Telemedicine in Health Care*
James D. Berry, M.D., M.P.H., Massachusetts General Hospital

11:30 – 12 p.m. *Answer ALS*
Jeffrey D. Rothstein, M.D., Ph.D., Johns Hopkins University, School of Medicine

Session #8: Best Practices in Dysphagia/Nutrition..... Regency CD
Session Chair: Becky Hurst-Davis

10:30 – 11 a.m. *Nutrition in Neuromuscular Disease*
Becky Hurst-Davis, M.S., RD, CSP, CD, CNSC, University of Utah, School of Medicine

11 – 11:30 a.m. *Dysphagia in Neuromuscular Disease*
Kiera Berggren, SLP, University of Utah, School of Medicine

11:30 – 12 p.m. *Nutritional Considerations in Neuromuscular Disease*
Elizabeth Miller, M.S., RD, Shriners' Hospital for Children, Salt Lake City

12 – 1:30 p.m. Lunch Independence Center B

Sessions #9, 10 and 11 are Concurrent Sessions

Session #9: Newborn Screening.....Regency EF
Session Chair: R. Rodney Howell

1:30 – 2:15 p.m. *Newborn Screening*
R. Rodney Howell, M.D., Miller School of Medicine, University of Miami

2:15 – 3 p.m. *Panel Discussion*
Panelists:
Rebecca Abbott, March of Dimes
Peter Kyriacopoulos, Association of Public Health Laboratories
Paul Melmeyer, National Organization for Rare Disorders
Mike Watson, Ph.D., FACMG, ACMG, Foundation for Genetic & Genomic Medicine

3 – 3:30 p.m. Coffee Break..... Independence Center A

3:30 – 4 p.m. **Robert Griggs, M.D., FAAN**, University of Rochester Medical Center

4 – 4:30 p.m. **Barry Byrne, M.D.**, University of Florida
4:30 – 5 p.m. **Kathryn Swoboda, M.D.**, Massachusetts General Hospital

Session #10: Augmentative Communication (Workshop)..... Washington Room
Session Chair: John M. Costello

1:30 – 3 p.m. *ALS and AAC and Early Engagement in Assessment, System Design and Implementation*
John M. Costello, M.A., CCC-SLP, Boston Children’s Hospital

Session #11: Pediatric/Adult Transition (Panel Discussion) Regency CD
Session Chair: Diane V. Murrell

1:30 – 3 p.m. *Transition from Pediatric to Adult-Based Health Care for Youth with Neuromuscular Disease*

Panelists:

Lauren Elman, M.D., University of Pennsylvania Medical Center
Diane V. Murrell, M.S.W., LCSW, Texas Children’s Hospital
Sarah Stoney, M.S.W., LSW, The Children’s Hospital of Philadelphia

3 – 3:30 p.m. Coffee Break..... Independence Center A

Sessions #9, 12 and 13 are Concurrent Sessions

Session #12: Respiratory Health..... Regency CD
Session Chairs: John Hansen-Flaschen and Venessa Holland

3:30 – 4 p.m. *Home Assisted Ventilation*
John Hansen-Flaschen, M.D, University of Pennsylvania, School of Medicine

4 – 4:30 p.m. *Transitions to Home Mechanical Ventilation*
Craig Dale, R.N., Ph.D., University of Toronto

4:30 – 5 p.m. *Respiratory Management of the Neuromuscular Patient with Airway Clearance Devices and Lung Volume Recruitment*
Venessa Holland, M.D., M.P.H., Houston Methodist Neurological Institute

Session #13 Care Coordination (Panel Discussion)..... Washington Room
Session Chair: Mona Shahbazi

3:30 – 5 p.m. *Best Practices in Care Coordination*

Panelists:

Rebecca Axline, LCSW-S, Houston Methodist Neurological Institute
Susan Nease, DNP, ANP, Medical University of South Carolina
Mona Shahbazi, N.P., M.S.N., Hospital for Special Surgery

5 – 5:30 p.m. Break

5:30 – 8 p.m. Poster Session and Reception..... Independence Center A

Wednesday, March 14, 2018

7 – 8 a.m. Breakfast Independence Center B

Session # 14: Clinical Trials Regency CDEF

Session Chairs: **Amanda Haidet-Phillips and Lianna Orlando**

8 – 8:30 a.m. *Suppressing Neuroinflammation: ALS Therapy Comes of Age*
Stanley H. Appel, M.D., Houston Methodist Neurological Institute

8:30 – 8:50 a.m. *AVXS-101 Phase 1 Gene Replacement Therapy Clinical Trial in SMA Type 1: Continued Event-Free Survival and Achievement of Developmental Milestones*
Doug Sproule, M.D., AveXis, Inc.

8:50 – 9:10 a.m. *Correlations between MRI and Functional Measurements in DMD Boys: Baseline data from the Phase 2 Domagrozumab Study*
Sarah Sherlock, Ph.D., Pfizer

9:10 – 9:30 a.m. *Updated Results from ATB200-02: a First-in-Human, Open-Label, Phase 1/2 Study of ATB200 Co-Administered with AT2221 in Adults with Pompe Disease*
Tahseen Mozaffar, M.D., University of California, Irvine

9:30 – 10 a.m. *Nusinersen in Infants Who Initiate Treatment in a Presymptomatic Stage of SMA: Interim Efficacy and Safety Results from the Phase 2 NURTURE Study*
Darryl De Vivo, M.D., Columbia University Medical Center

10 – 10:30 a.m. Coffee Break Regency Foyer

10:30 – 10:50 a.m. *Golodirsen Induces Exon Skipping Leading to Sarcolemmal Dystrophin Expression in Patients with Genetic Mutations Amenable to Exon 53 Skipping*
Elizabeth Smyth, FNP-BC, Sarepta Therapeutics, Inc.

10:50 – 11:10 a.m. *CALLISTO: A Phase 1 Open-Label, Sequential Group, Cohort Study of Pharmacokinetics and Safety of Omigapil in LAMA2 and COL6-Related Dystrophy Patients*
A Reghan Foley, M.D., NINDS, NIH

11:10 – 11:30 a.m. *Biomarkers for Muscular Diseases – Data Supporting Glutamate Dehydrogenase (GLDH) as a Specific Biomarker of Liver Damage*
Jane Larkindale, Ph.D., Critical Path Institute/FARA

11:30 – 11:50 a.m. *A Phase 2 Study of AMO-02 (tideglusib) in Congenital and Childhood Onset Myotonic Dystrophy Type 1*
Joseph Horrigan, M.D., AMO Pharma Ltd.

~ Conference Concludes ~