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 Argentiinis, 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, Shriner’s 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

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

2:15 – 3 p.m. Panel Discussion

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 ~