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
<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
<th>Speaker(s)</th>
<th>Location</th>
</tr>
</thead>
<tbody>
<tr>
<td>11 – 11:30 a.m.</td>
<td>Myotonic Dystrophy (DM)</td>
<td>Charles Thornton, M.D., University of Rochester Medical Center</td>
<td></td>
</tr>
<tr>
<td>11:30 – 12 p.m.</td>
<td>Myasthenia Gravis (MG)</td>
<td>James F. Howard, Jr., M.D., FAAN, The University of North Carolina at Chapel Hill</td>
<td></td>
</tr>
<tr>
<td>12 – 1:30 p.m.</td>
<td>Lunch</td>
<td></td>
<td>Independence Center B</td>
</tr>
<tr>
<td></td>
<td><strong>Session #2: Advances in Personalized Medicine</strong></td>
<td></td>
<td>Regency CDEF</td>
</tr>
<tr>
<td></td>
<td><strong>Session Chair:</strong> Eric Olson</td>
<td></td>
<td></td>
</tr>
<tr>
<td>1:30 – 2:10 p.m.</td>
<td>Correction of Duchenne Muscular Dystrophy by Genome Editing</td>
<td>Eric Olson, Ph.D., UT Southwestern Medical Center</td>
<td></td>
</tr>
<tr>
<td>2:10 – 2:50 p.m.</td>
<td>Scaling Up Genomic Diagnosis of Muscle Disorders</td>
<td>Daniel MacArthur, Ph.D., Broad Institute of Harvard and MIT</td>
<td></td>
</tr>
<tr>
<td>2:50 – 3:30 p.m.</td>
<td>Advancing the Role of Digital Health in Neuromuscular Disease</td>
<td>Murray Aitken, MBA, IQVIA Institute for Human Data Science</td>
<td></td>
</tr>
<tr>
<td>3:30 – 4 p.m.</td>
<td>Coffee Break</td>
<td></td>
<td>Independence Center A</td>
</tr>
<tr>
<td></td>
<td><strong>Session #3: MDA Flagship Initiatives</strong></td>
<td></td>
<td>Regency CDEF</td>
</tr>
<tr>
<td>4 – 4:45 p.m.</td>
<td>MDA ONEvoice</td>
<td>Kristin Stephenson, M.H.A., J.D., Muscular Dystrophy Association</td>
<td></td>
</tr>
<tr>
<td>4:45 – 5:30 p.m.</td>
<td>MDA MOVR Registry</td>
<td>Grace K. Pavlath, Ph.D., Muscular Dystrophy Association</td>
<td></td>
</tr>
<tr>
<td>5:30 - 6 p.m.</td>
<td>Break</td>
<td></td>
<td></td>
</tr>
<tr>
<td>6 – 8 p.m.</td>
<td>MDA Champions Networking Reception</td>
<td></td>
<td>Independence Center A</td>
</tr>
</tbody>
</table>
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  
Enterne Argenti, 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

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
Session # 14: Clinical Trials

Session Chairs: Amanda Haidet-Phillips and Laura Hagerty

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

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 ~