Sunday, March 20, 2016

4 p.m.  Registration .......................................................... Regency Foyer

5:30 - 6:30 p.m.  From Clinics to Care Centers: .................................. Regency A, B, C, D, E & F (Ballroom)  
Imagine What We Can Do Together by 2020  
Lauren Webb, AM

6:30 - 8 p.m.  Welcome Reception .......................................................... Independence A

Monday, March 21, 2016

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
<th>Location</th>
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<tr>
<td>6:45 - 7:45 a.m.</td>
<td>Breakfast — General</td>
<td>Independence B</td>
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| 6:45 - 7:45 a.m. | Advanced Practice Providers in the MDA Care Center  
Cynthia Budek, MS, APN, CPNP-PA/AC and Lora Clawson, MSN, CRNP | Potomac III, IV, V & VI                                                   |
| 8 - 8:30 a.m. | Welcome and Opening Remarks       | Regency A, B, C & D                                                          |

**Genetics of Neuromuscular Diseases**

**Session Chairs:** Matthew Harms, M.D., and Matthew Wicklund, M.D.

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<tr>
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<tr>
<td>8:30 - 9:10 a.m.</td>
<td>Overview of Current Genetic Testing Options and Methods</td>
<td>Regency A, B, C &amp; D</td>
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<td>9:10 - 9:50 a.m.</td>
<td>Integrating Genetic Testing into Clinical Practice</td>
<td>Regency A, B, C &amp; D</td>
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<td>9:50 - 10:20 a.m.</td>
<td>Break</td>
<td>Independence A</td>
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<tr>
<td>10:20 - 11:10 a.m.</td>
<td>Progress and Challenges in Genotype — Phenotype Correlations</td>
<td>Regency A, B, C &amp; D</td>
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<td>11:10 - 11:50 a.m.</td>
<td>Approaches to Variant of Unknown Significance and “Next Steps for Negative Exomes”</td>
<td>Regency A, B, C &amp; D</td>
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<tr>
<td>12 - 1:30 p.m.</td>
<td>Lunch</td>
<td>Independence B</td>
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**Caring For Kids And Adults With Neuromuscular Diseases From Day One**

**Session Chairs:** Anne Connolly, M.D., and Tahseen Mozaffar, M.D.

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<tr>
<td>1:30 - 2 p.m.</td>
<td>Guidelines for Care of Patients with LGMD</td>
<td>Regency A, B, C &amp; D</td>
</tr>
<tr>
<td>2 - 2:30 p.m.</td>
<td>Care for Adults with Neuromuscular Disorders in a Multidisciplinary MDA Care Center</td>
<td>Regency A, B, C &amp; D</td>
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R. Brian Sommerville, M.D.
### Monday, March 21, 2016

2:30 - 3 p.m.  Compassionate Care for Children and Adults

Compassionate Care for Children and Adults: What Does a Good Death Look Like?
Joan Rosenbaum, M.D.

3 - 3:30 p.m.  Break

3:30 - 4 p.m.  Pompe, Day No. 1 Through Day No. 30,000

Tahseen Mozaffar, M.D.

4 - 4:30 p.m.  Care of Adults with ALS, Day No. 1

Matthew Harms, M.D.

4:30 - 5 p.m.  Day No. 1 Care of Infants with Muscular Dystrophy and SMA in an Era of Newborn Screening

Anne Connolly, M.D.

5:30 - 7:30 p.m.  Poster Session and Reception

### Tuesday, March 22, 2016

6:15 - 7 a.m.  MDA Team Momentum Fun Run

6:45 - 7:45 a.m.  Breakfast — General

6:45 - 7:45 a.m.  Breakfast

Drug Development: Taking Therapies into the Clinic
Wilson Bryan, M.D.

8 - 8:15 a.m.  Opening Remarks

8:15 - 9:15 a.m.  The Cystic Fibrosis Model as a Paradigm for Multidisciplinary Care: Lessons Learned
Bruce Marshall, M.D.

### Parallel Session #1

**Session Chair:** Vern C. Juel, M.D.

9:15 - 10:15 a.m.  Neuromuscular Ultrasound Presented by AANEM

Lisa Hobson-Webb, M.D.

10:15 - 10:45 a.m.  Break

10:45 am - 12:45 p.m.  Neuromuscular Ultrasound Presented by AANEM (continued)
Parallel Session #2

Session Chair: Lauren Webb, AM

9:15 - 10:15 a.m. Best Practices for Transition Care in the Neuromuscular Care Center . . . . Regency A, B, C & D
Rebecca Boudos, LCSW, Jenny Kean, LCSW, India Peters, and Sindhu Ramchandren, M.D.

10:15 - 10:45 a.m. Break ............................................................. Independence A

Parallel Session #2A

10:45 - 11:45 a.m. Optimizing Clinic Programs and Communication in a Digital World . . . . Regency A, B, C & D
Stephanie Hlywak, Kathryn A. Sabadosa, MPH, and Safi Shareef, M.D.

Parallel Session #2B

11:45 am - 12:45 p.m. Genetic Counseling and Telehealth for Neuromuscular Diseases . . . . Regency A, B, C & D
Sandra Donkervoort, MS, CGC, Sarah N. Pletcher, M.D., and Lauren Webb, AM

Parallel Session #2C

11:45 am - 12:45 p.m. Physical Therapists: Making an Impact in Neuromuscular . . . . . . . Potomac V & VI
Care Centers and Local Communities
Peggy Allred, PT, DPT, Tina Duong, MPT, and Carolyn Kelley, DPT

12:45 - 2 p.m. Lunch ............................................................. Independence B

Parallel Session #3A: Respiratory Care for Neuromuscular Diseases

Session Chairs: Harold Farber, M.D., MSPH, and Noah Lechtzin, M.D., MHS

2 - 2:25 p.m. Breathing Well When Your Muscles Fail: Basic Principles . . . . . . . . . . . . . . Regency A, B, C & D
Harold Farber, M.D., MSPH

2:25 - 3 p.m. The Science — and Art — of Noninvasive Ventilation . . . . . . . . . . . Regency A, B, C & D
for Children with Muscular Dystrophies
Hank Mayer, M.D.

3 - 3:30 p.m. Break ............................................................. Independence A

Noah Lechtzin, M.D., MHS

4 - 4:30 p.m. Scoliosis Repair: Surgical Indications, Demands, Challenges, Complications . . . Regency A, B, C & D
William Phillips, M.D.
Tuesday, March 22, 2016

4:30 - 4:50 p.m.  What Do We Really Accomplish with Scoliosis Repair? ............... Regency A, B, C & D
           The Pulmonary Perspective
           Hank Mayer, M.D.

4:50 - 5:10 p.m.  Invest in the Beginning: A Multidisciplinary Approach ............ Regency A, B, C & D
           to Improving Outcomes in Scoliosis Surgery, The TCH Experience
           Harold Farber, M.D., MSPH

5:10 - 5:30 p.m.  Making a Smooth Handoff Rather than a Fumble: ................. Regency A, B, C & D
           Transitions from Pediatric to Adult Care in Pulmonary Medicine
           Noah Lechtzin, M.D., MHS

Parallel Session #3B: Rehabilitation Issues in Neuromuscular Disorders

Session Chair: Dennis J. Matthews, M.D.

2 - 2:25 p.m.  Introduction ............................................. Potomac III, IV, V & VI
           Dennis J. Matthews, M.D.

2:25 - 3 p.m.  Rehabilitation Principles in Pediatrics: Maximizing Function .... Potomac III, IV, V & VI
           Susan Apkon, M.D.

3 - 3:30 p.m.  Break ...................................................... Independence A

3:30 - 4 p.m.  Exercise ................................................ Potomac III, IV, V & VI
           Joyce Oleszek, M.D.

4 - 4:30 p.m.  Teamwork and Testing .................................... Potomac III, IV, V & VI
           Terri Carry, PT

4:30 - 4:50 p.m.  Advances in Robotics and Adaptive Equipment .................... Potomac III, IV, V & VI
           Dennis J. Matthews, M.D.

4:50 - 5:10 p.m.  Rehabilitation Issues in Adults with Neuromuscular Disorders ... Potomac III, IV, V & VI
           Nanette Joyce, D.O.

Dinner On Your Own

Wednesday, March 23, 2016

6:45 - 7:45 a.m.  Breakfast — General ........................................ Independence B

6:45 - 7:45 a.m.  Breakfast ............................................... Potomac III, IV, V & VI
           Registries and Infrastructure Support for Drug Development
           Ronald Bartek, Paul Mehta, M.D., and Lauren Webb, AM

8 - 8:30 a.m.  Opening Remarks & Poster Awards ............................... Regency A, B, C, D, E & F
Clinical Trials and Late-Breaking News

Session Chair: Valerie A. Cwik, M.D.

8:30 - 8:55 a.m. Effect of Tadalafil, a Phosphodiesterase Type 5 Inhibitor, on Ambulatory Decline in Boys with Duchenne Muscular Dystrophy: Top-Line Results of a Phase 3, Randomized, Placebo-Controlled Trial
   Ronald G. Victor, M.D.

8:55 - 9:20 a.m. Effect of Deflazacort and Prednisone Versus Placebo on Muscle Strength in Boys with Duchenne Muscular Dystrophy Who Have Lost Ambulation: Results from the Deflazacort Clinical Trial Program
   Jordan Dubow, M.D.

9:20 - 9:45 a.m. Antioxidant Therapy in RYR1-Related Congenital Myopathy
   Katherine Meilleur, Ph.D.

9:45 - 10:10 a.m. Decline in Slow Vital Capacity Predicts Respiratory Insufficiency, Use of Assisted Ventilation, Tracheostomy or Death
   Jeremy Shefner, M.D., Ph.D.

10:10 - 10:30 a.m. Expanded Access for Investigational Treatments
   Richard Klein

10:30 - 10:45 a.m. Break

10:45 - 11:10 a.m. CAT-1004, an Oral Agent Targeting NF-kB: Move DMD Trial
   Joanne Donovan, M.D., Ph.D.

11:10 - 11:35 a.m. ACE-083, a Locally-Acting TGF-Beta Superfamily Ligand Trap
   Kenneth M. Attie, M.D.

11:35 a.m.- 12 p.m. BMS-986089: An Anti-Myostatin Adnectin Targeting Duchenne Muscular Dystrophy
   Leslie Jacobsen, M.D.

12 - 12:25 p.m. Ataluren: An Overview of Clinical Trial Results in Nonsense Mutation Duchenne Muscular Dystrophy
   Joseph McIntosh, M.D.

12:30 p.m. Conference Adjourns