

8:00 am to 8:20 am	<p>Welcome and CMO update Mary Schroth, MD, Chief Medical Officer, Cure SMA</p>
8:20 am to 8:40 am	<p>How the availability of new, effective treatment for spinal muscular atrophy alters reproductive decision making Mackenzie Blaile, MS, Colorado Children's Hospital/University of Colorado</p>
8:40 am to 9:00 am	<p>Fat mass assessment in type 1 spinal muscular atrophy: comparison of field and reference methods Ramona Silvana De Amicis, RD, PhD Candidate, University of Milan</p>
9:00 am to 10:30 am	<p>New Therapies Panel Discussion</p> <p>Onasemnogene Abeparvovec Gene-Replacement Therapy for Spinal Muscular Atrophy Type 1: Pivotal Phase 3 Study (STR1VE) Update Adeno-Associated Virus Serotype 9 (AAV9) Antibodies in Patients With SMA Screened for Treatment With Onasemnogene Abeparvovec John Day, MD, Stanford University Medical Center</p> <p>Gene-Replacement Therapy in Spinal Muscular Atrophy Type 1: Long-Term Follow-Up From the Onasemnogene Abeparvovec Phase 1/2 Clinical Trial Jerry Mendell, MD, Nationwide Children's Hospital</p> <p>The AveXis, Inc. Managed Access Program: Single Patient Investigational New Drug Requests for Onasemnogene Abeparvovec Elizabeth Kichula, MD, Children's Hospital of Philadelphia</p>
10:30 am to 10:45 am	<p>Break</p>
10:45 am to 11:30 am	<p>Case Study 1: Management and therapy considerations for a newborn with SMA Moderator: Julie Parsons, MD, Colorado Children's Hospital/University of Colorado Panelists: Alyssa Burgart, MD, MA, FAAP, Lucile Packard Children's Hospital/Stanford University Brian Jackson, MD, MA, Colorado Children's Hospital/University of Colorado</p>
11:30 am to 1:00 pm	<p>Networking Lunch</p>
1:00 pm to 2:00 pm	<p>Musculoskeletal Panel Discussion</p> <p>The positive impact of intensive inpatient rehabilitation in patients with Spinal Muscular Atrophy receiving Nusinersen treatment Caitlin Brady, APRN, CPNP, Children's National Health System</p> <p>20 Year Experience with Segmental Sublaminar Instrumentation for Guided Growth in Early Onset Scoliosis Samuel Rosenfeld, MD, Children's Hospital of Orange County</p> <p>Sagittal Deformities in Children with SMA2 following Spinal Instrumentation Matthew Halanski, MD, Children's Hospital of Omaha</p>
2:00 pm to 2:20 pm	<p>Response of Adults with SMA to Nusinersen John W Day, MD, Stanford University Medical Center</p>



2:20 pm to 2:40 pm	Intrathecal nusinersen in older children and adults with spinal muscular atrophy Aravindhan Veerapandiyan, MD, Arkansas Children's Hospital
2:40 pm to 3:00 pm	Nusinersen Treatment of Older Patients with Spinal Muscular Atrophy Chamindra Konersman, MD, Rady Children's Hospital/University of California, San Diego
3:00 pm to 3:15 pm	Break
3:15 pm to 4:00 pm	Case Study 2: An adult with SMA seeking therapy Moderator: Tom Crawford, MD, Johns Hopkins Medical Center Panelists: Alyssa Burgart, MD, MA, FAAP, Lucile Packard Children's Hospital/Stanford University Brian Jackson, MD, MA, Colorado Children's Hospital/University of Colorado
4:00 pm to 6:00 pm	Poster Session
