



Annual SMA Research and Clinical Care Meeting

Clinical Care Session Agenda

June 5<sup>th</sup>, 2024

	<b>Abstract Number</b>	<b>Presentation Title</b>
8:30 am to 9:00 am		<b>Welcome and Cure SMA Update</b> Mary Schroth, MD, Chief Medical Officer, Cure SMA Lisa Belter, MPH, VP, Research Data Analytics, Cure SMA
9:00 am - 9:15 am	<b>1</b>	<b>Adverse Immune Responses to AAV-based in vivo Gene Therapy</b> Sofia Celli, BA, University of Colorado, Anschutz Medical Campus
9:15 am - 9:30 am	<b>2</b>	<b>Quantitative Evaluation of Upper Extremity Strength and Joint Contractures in Spinal Muscular Atrophy Type II: Implications for Motor Function</b> Timothy Estilow, MS, OTR/L, Children's Hospital of Philadelphia
9:30 am – 10:15 am	<b>3, 4, 5</b>	<b>Panel Discussion: SMA Phenotypes</b> <b>Case series: Incidence of Torticollis and Early Scoliosis in SMA Patients Identified by Newborn Screening</b> Carolyn Kelley, DPT, PCS, Children's Hospital Colorado  <b>Emerging Upper Extremity Phenotype in Children with Spinal Muscular Atrophy</b> AND <b>Anatomic and functional oral deficits: a Feature of the New Natural History of Early Treated 2-copy SMN2 Infants</b> Julie Patterson, MS, OTR/L, Johns Hopkins Hospital
10:15am- 10:35am	<b>6 &amp; 7</b>	<b>Impact of Comorbidities on Mortality in Spinal Muscular Atrophy Patients with Early Onset Scoliosis</b> AND <b>Risk Model for Proximal Junctional Kyphosis Following Spine Deformity Surgery in Spinal Muscular Atrophy\</b> Hiroko Matsumoto, PhD, Boston Children's Hospital Brian Snyder, MD, Boston Children's Hospital
10:35 am -10:55 am		<b>Break</b>
10:55 am -12:00 pm		<b>Reproductive Health in Individuals with SMA: A Panel Discussion</b> Natan Bar-Chama, MD, RMA of New York Loralei Thornburg, MD, University of Rochester Medical Center Megan Dejarnett, Speaker, Author, & Disability Advocate
12:00 pm - 1:00 pm		<b>Lunch</b>



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1:00 pm - 1:15 pm	<b>8</b>	<b>Wearable-derived Patterns of Performance Fatigability During Gait in Spinal Muscular Atrophy</b> Jacqueline Montes, PT, EdD, Columbia University Irving Medical Center
1:15 pm - 1:30 pm	<b>9</b>	<b>Longitudinal Evaluation of Fatigue in Adult Patients with Spinal Muscular Atrophy and the Impact of Treatment with Disease-Modifying Drugs</b> Felipe Franco da Graca, MD, University of Campinas, Brazil
1:30 pm – 1:45 pm	<b>10</b>	<b>Case Series: Profound Improvements in Mouth Opening among patients with SMA following Orastretch Therapy</b> Katlyn McGrattan, PhD, University of Minnesota
1:45 pm - 2:30 pm	<b>11 &amp; 12</b>	<b>Panel Discussion: Anesthesia</b> <b>Anesthesia and Procedure Concerns in the Administration of Nusinersen: Experience of a Large, Single-Institution Cohort</b> Joanne Shay, MD, MBA, Johns Hopkins University <b>Pitfalls in SMA Anesthesia. Nearing 10 years in the Nusinersen era</b> Alan Bielsky, MD, University of Colorado School of Medicine, Children’s Hospital of Colorado
2:30 pm - 2:50 pm		<b>Break</b>
2:50 pm – 3:55 pm		<b>Advocacy in Air Travel for Individuals with SMA: A Panel Discussion</b> Maynard Friesz, VP, Policy & Advocacy, Cure SMA Sarah Bellish, BA, Manager, Policy & Advocacy, Cure SMA Courtney Gushue, DO, Nationwide Children’s Hospital
3:55pm- 4:00pm		<b>Closing Remarks</b>