


# Annual SMA Research & Clinical Care Meeting

JUNE 24-26, 2026 | BOARDWALK INN | ORLANDO, FL

DAY 1 | WEDNESDAY, JUNE 24, 2026

## MORNING SESSIONS - PARALLEL TRACKS

The Clinical Care Session and Research Meeting run concurrently throughout the morning. Attendees may choose between sessions in either track.

<b>7:00 AM</b>	<p><b>Symposium: A Clinical Review of the High Dose Regimen of Nusinersen</b></p> <p>Sponsored by Biogen</p> 
----------------	--

CLINICAL CARE SESSION			RESEARCH MEETING		
TIME	ABSTRACT #	SESSION	TIME	ABSTRACT #	SESSION
8:30 AM		<p><b>Welcome</b></p> <p>Bakri Elsheikh, MD, Cure SMA Medical Advisory Council Chair</p>	8:30 AM		<p><b>Welcome</b></p> <p>Jackie Glascock, PhD, Chief Scientific Officer, Cure SMA</p>
			<b>Genetics of Disease and Modeling Tools</b>		
8:45 AM	1	<p><b>Brief Updates in SMA</b></p> <p><i>Moderator: Julie Parsons, MD</i></p> <p>Luca Labianca, MD, PhD – Updates in Orthopedics Tina Duong, MPT, PhD – Updates in Technology &amp; SMA Sarah Wright, DO – Updates in Neurocognition</p>	8:40 AM	1	<p><b>Modeling Neuromuscular Disorders: From Human NMJ Assembloids to a High-Throughput Functional Screening Platform for Drug Discovery</b></p> <p>Chen Kuchuan, PhD</p>
9:15 AM	2	<p><b>Multi-System Gastrointestinal and Metabolic Complications in Spinal Muscular Atrophy Across the Lifespan</b></p> <p><i>Moderator: Julie Parsons, MD</i></p> <p>Ihuoma Eneli, MD Anna Magdalena Banc-Husu, MD</p>	9:15 AM	2	<p><b>Excision of Mouse Smn Exon 2b Enhances Smn Function in SMA-Critical Pathways</b></p> <p>Anton Blatnik, PhD</p>
			<b>Pre and Post Symptomatic Treatment Effect</b>		
			9:20 AM	3	<p><b>The Neuropathology of Severe Human SMA and The Effects of Post-symptomatic Administration of Disease Modifying Therapies</b></p> <p>Maddi Denton, BS</p>
			9:40 AM	4	<p><b>Prenatal Exposure to a Risdiplam-like Compound Partially Rescues the SMNΔ7 Mouse Model of Spinal Muscular Atrophy Without Postnatal SMN Intervention</b></p> <p>Emma Sutton, PhD</p>

CLINICAL CARE SESSION			RESEARCH MEETING		
TIME	ABSTRACT #	SESSION	TIME	ABSTRACT #	SESSION
			10:00 AM	5	<b>Prenatal Intervention in an SMA Mouse Model Rescues Neurodevelopmental Disorders Associated with Spinal Muscular Atrophy</b> Charalambos Demetriou, PhD
10:30 AM		<b>Break</b>	10:30 AM		<b>Break</b>
			<b>SMN-Deficient Muscle Defects and Muscle-Targeted Therapy</b>		
11:00 AM	3	<b>Neurofilament in Spinal Muscular Atrophy Across the Lifespan</b> <i>Moderator: Pooja Mohan Rao, MD</i> Stephen Kolb, MD Crystal Proud, MD	11:00 AM	6	<b>Characterizing the Reversibility of Cellular and Molecular Defects of SMN-deficient Muscle</b> Elana Molotsky, PhD
11:30 AM	4	<b>Supporting the Mental Health of SMA-affected Individuals and Families: Individual, Community, and Systems Based Approaches</b> <i>Moderator: Pooja Mohan Rao, MD</i> Colette Gramszlo, MD Al Freedman, PhD	11:20 AM	7	<b>Muscle-specific Sensory-motor Dysfunction Induce Masticatory Impairments in Humans and Animal Models of SMA</b> Danny Florez-Paz, PhD
			11:40 AM	8	<b>Muscle-specific Kinase Agonist Antibody, Adimanebart, Coadministered with an SMN2 Splice Modulator Improves Muscle Strength and Voluntary Locomotion in a Mouse Model of Spinal Muscular Atrophy</b> Lyndsay Murray, PhD
12:00 PM	<b>Lunch</b>				


## AFTERNOON - COMBINED RESEARCH AND CLINICAL CARE MEETING

Both tracks come together for the afternoon programming. CME content provided.

TIME	ABSTRACT #	SESSION
1:00 PM	5	<b>2025 STATE OF SMA</b> Lisa Belter, MPH
1:30 PM	6	<b>Neurodevelopment Panel</b> <i>Moderator: Lyndsay Murray</i> Tom Crawford, MD Richard Finkel, MD Charlotte Sumner, MD
2:30 PM		<b>Break</b>
2:50 PM	7	<b>THERAPEUTIC STRATEGIES IN SPINAL MUSCULAR ATROPHY IN AN ERA OF MULTIPLE OPTIONS</b> <i>Moderator: Susan Matesanz, MD</i> John Brandsema, MD Perry Shieh, MD
4:00 PM		<b>CLOSING REMARKS</b> Jennifer Deans, MHA, MS, CCLS, Director, Clinical Care Education Programs
4:30 PM		<b>Poster Session A: Odd Numbered Posters Presented</b>

# DAY 2 | THURSDAY, JUNE 25, 2026

7:00 AM	<p><b>Symposium: Expert Perspectives on the Evolving Management of SMA</b> Sponsored by Scholar Rock</p> 
---------	--

TIME	ABSTRACT #	RESEARCH MEETING SESSION
<b>Treatment and Motor Function</b>		
8:30 AM	9	<p><b>Fatigability and Aerobic Capacity: A Comparative Analysis of Risdiplam and Nusinersen</b> Jacqueline Montes, PT, EdD</p>
8:50 AM	10	<p><b>Evaluating the Waning-effect of Nusinersen Using the SMA Effort</b> Rafael Rodriguez-Torres, PT, DPT</p>
9:10 AM	11	<p><b>Prodromal Swallowing Deficits in Asymptomatic Infants with Spinal Muscular Atrophy: A Source for Suboptimal Treatment Outcomes</b> Katlyn McGrattan, PhD</p>
9:30 AM	12	<p><b>Epidural Spinal Cord Stimulation Reduces Intrinsic Gait Abnormalities in SMA</b> Carly Mueller, PT, DPT</p>
9:50 AM		<b>Break</b>
10:30 AM		<b>Adults Living with SMA and Parents of Children with SMA Roundtable</b>
11:45 AM		<b>Lunch</b>
12:00 PM		<p><b>Symposium: From Evidence to Practice: Expanding Options for Adult and Pediatric Patients With SMA</b> Sponsored by Novartis</p> 
<b>Clinical Outcome Measures</b>		
1:10 PM	13	<p><b>The Shuttle Squat Test: A Practical Tool for Assessing Endurance and Proximal Leg Muscle Strength in Spinal Muscular Atrophy</b> Joelle Williamson Clark, MPH</p>
1:30 PM	14	<p><b>Rasch Analysis of the Spinal Muscular Atrophy Person-Reported Outcome Measure (SMA-PRO) Daily Activities Domain in Children and Adults with SMA</b> Maria Fragala-Pinkham, PT, DPT, DSc</p>
1:50 PM	15	<p><b>Long-Term, Real-World Spatiotemporal and Kinetic Gait Monitoring in Spinal Muscular Atrophy Using AI-Sole</b> Kai-Chun Liu, PhD</p>
2:10 PM		<b>Break</b>

## Treatment Outcomes and Biomarkers

2:40 PM	16	<b>Long-Term Real-World Outcomes Following Onasemnogene Apeparovvec Monotherapy for Patients with Spinal Muscular Atrophy: Updated Findings from the RESTORE Registry</b> Richard Finkel, MD
3:00 PM	17	<b>Neurofilament Light Chain as a Biomarker in Spinal Muscular Atrophy</b> Stephanie Fradette, PharmD
3:20 PM	18	<b>Single-gene NIPT Leads to Earlier Diagnosis and Optimized Treatment of Newborns with Infantile-onset Spinal Muscular Atrophy: A Case Series</b> Karlla W. Brigatti, MS CGC
4:00 PM		<b>Poster Session B: Even Numbered Posters Presented</b>
6:30 PM		<b>Meet and Greet with Families: Featuring Annual Relay Race</b>

## DAY 3 | FRIDAY, JUNE 26, 2026

7:00 AM	<p><b>Symposium: Considerations in SMA Management Across the Lifespan: Newborns to Adults</b></p> <p>Sponsored by Genentech</p> <p style="font-size: 2em; font-weight: bold; margin: 0;">Genentech</p> <p style="font-size: 0.8em; margin: 0;">A Member of the Roche Group</p>
---------	--

TIME	ABSTRACT #	RESEARCH MEETING SESSION
8:30 AM		<b>Podium Poster Highlights</b>
<b>Clinical Therapeutic Development</b>		
9:10 AM	19	<b>Safety and Pharmacodynamic Effects of Emugrobart in Combination with Risdiplam in Ambulant and Non-ambulant Participants with SMA: MANATEE Part 1</b> Laurent Servais, MD, PhD
9:30 AM	20	<b>Final Results from the JEWELFISH Study: 5-year Risdiplam Treatment in Non-treatment-naïve Individuals with SMA</b> Claudia A. Chiriboga, MD
9:50 AM	21	<b>Phase 1 Interim Results Evaluating the Safety, Tolerability, Pharmacokinetics, and Exploratory Efficacy of Salanersen for Spinal Muscular Atrophy</b> Thomas O. Crawford, MD
10:10 AM	22	<b>DEVOTE Part C and ONWARD Integrated Results: Exploring High Dose Nusinersen in Nusinersen-Experienced Participants with Spinal Muscular Atrophy</b> Richard S. Finkel, MD
10:30 AM		<b>Break</b>
11:00 AM	23	<b>Intravenous Onasemnogene Apeparovvec Gene Replacement Therapy for Presymptomatic Spinal Muscular Atrophy: Long-Term Follow-Up Analysis</b> Megan A. Waldrop, MD

11:20 AM	24	<b>Intrathecal Onasemnogene Apeparvovec (OAV101) for Patients with Spinal Muscular Atrophy (SMA): Extended 64-Week Outcomes from the Phase 3 STEER Study</b> Crystal M. Proud, MD
11:40 AM	25	<b>Impact of Baseline Characteristics on Apitegromab Efficacy in Patients with Spinal Muscular Atrophy</b> Jing Marantz, MD, PhD
12:00 PM	26	<b>Post hoc Analyses from the Phase 3 SAPPHIRE Study Evaluating Apitegromab in Patients with Nonambulatory Type 2 or 3 Spinal Muscular Atrophy</b> Jena M. Krueger, MD
12:00 PM		<b>Closing Remarks</b> Jackie Glascock, PhD, Vice President Research, Cure SMA
12:30 PM		<b>Research and Clinical Care Meeting Adjourns</b>
6:30 PM		<b>Family Friendly Researcher Poster Session</b>

**FOR QUESTIONS, PLEASE CONTACT [SMARCC@CURESMA.ORG](mailto:SMARCC@CURESMA.ORG)**