


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.

7:00 AM	<p>Symposium: A Clinical Review of the High Dose Regimen of Nusinersen</p> <p>Sponsored by Biogen</p> 
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CLINICAL CARE SESSION			RESEARCH MEETING		
TIME	ABSTRACT #	SESSION	TIME	ABSTRACT #	SESSION
8:30 AM		<p>Welcome</p> <p>Bakri Elsheikh, MD, Cure SMA Medical Advisory Council Chair</p>	8:30 AM		<p>Welcome</p> <p>Jackie Glascock, PhD, Chief Scientific Officer, Cure SMA</p>
			Genetics of Disease and Modeling Tools		
8:45 AM	1	<p>Brief Updates in SMA</p> <p><i>Moderator: Julie Parsons, MD</i></p> <p>Luca Labianca, MD, PhD – Updates in Orthopedics Tina Duong, MPT, PhD – Updates in Technology & SMA Sarah Wright, DO – Updates in Neurocognition</p>	8:40 AM	1	<p>Modeling Neuromuscular Disorders: From Human NMJ Assembloids to a High-Throughput Functional Screening Platform for Drug Discovery</p> <p>Chen Kuchuan, PhD</p>
9:15 AM	2	<p>Multi-System Gastrointestinal and Metabolic Complications in Spinal Muscular Atrophy Across the Lifespan</p> <p><i>Moderator: Julie Parsons, MD</i></p> <p>Ihuoma Eneli, MD Anna Magdalena Banc-Husu, MD</p>	9:15 AM	2	<p>Excision of Mouse Smn Exon 2b Enhances Smn Function in SMA-Critical Pathways</p> <p>Anton Blatnik, PhD</p>
			Pre and Post Symptomatic Treatment Effect		
			9:20 AM	3	<p>The Neuropathology of Severe Human SMA and The Effects of Post-symptomatic Administration of Disease Modifying Therapies</p> <p>Maddi Denton, BS</p>
			9:40 AM	4	<p>Prenatal Exposure to a Risdiplam-like Compound Partially Rescues the SMNΔ7 Mouse Model of Spinal Muscular Atrophy Without Postnatal SMN Intervention</p> <p>Emma Sutton, PhD</p>

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


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

DAY 2 | THURSDAY, JUNE 25, 2026

7:00 AM	<p>Symposium: Expert Perspectives on the Evolving Management of SMA Sponsored by Scholar Rock</p> 
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TIME	ABSTRACT #	RESEARCH MEETING SESSION
Treatment and Motor Function		
8:30 AM	9	Fatigability and Aerobic Capacity: A Comparative Analysis of Risdiplam and Nusinersen Jacqueline Montes, PT, EdD
8:50 AM	10	Evaluating the Waning-effect of Nusinersen Using the SMA Effort Rafael Rodriguez-Torres, PT, DPT
9:10 AM	11	Prodromal Swallowing Deficits in Asymptomatic Infants with Spinal Muscular Atrophy: A Source for Suboptimal Treatment Outcomes Katlyn McGrattan, PhD
9:30 AM	12	Epidural Spinal Cord Stimulation Reduces Intrinsic Gait Abnormalities in SMA Carly Magun, PT, DPT
9:50 AM		Break
10:30 AM		Adults Living with SMA and Parents of Children with SMA Roundtable
11:45 AM		Lunch
12:00 PM		<p>Symposium: From Evidence to Practice: Expanding Options for Adult and Pediatric Patients With SMA Sponsored by Novartis</p> 
Clinical Outcome Measures		
1:10 PM	13	The Shuttle Squat Test: A Practical Tool for Assessing Endurance and Proximal Leg Muscle Strength in Spinal Muscular Atrophy Joelle Williamson Clark, MPH
1:30 PM	14	Rasch Analysis of the Spinal Muscular Atrophy Person-Reported Outcome Measure (SMA-PRO) Daily Activities Domain in Children and Adults with SMA Maria Fragala-Pinkham, PT, DPT, DSc
1:50 PM	15	Long-Term, Real-World Spatiotemporal and Kinetic Gait Monitoring in Spinal Muscular Atrophy Using AI-Sole Kai-Chun Liu, PhD
2:10 PM		Break

Treatment Outcomes and Biomarkers

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

DAY 3 | FRIDAY, JUNE 26, 2026

7:00 AM	<p>Symposium: Considerations in SMA Management Across the Lifespan: Newborns to Adults</p> <p>Sponsored by Genentech</p> <p style="font-size: 24px; font-weight: bold; margin: 0;">Genentech</p> <p style="font-size: 12px; margin: 0;">A Member of the Roche Group</p>
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TIME	ABSTRACT #	RESEARCH MEETING SESSION
8:30 AM		Podium Poster Highlights
Clinical Therapeutic Development		
9:10 AM	19	Safety and Pharmacodynamic Effects of Emugrobart in Combination with Risdiplam in Ambulant and Non-ambulant Participants with SMA: MANATEE Part 1 Laurent Servais, MD, PhD
9:30 AM	20	Final Results from the JEWELFISH Study: 5-year Risdiplam Treatment in Non-treatment-naïve Individuals with SMA Claudia A. Chiriboga, MD
9:50 AM	21	Phase 1 Interim Results Evaluating the Safety, Tolerability, Pharmacokinetics, and Exploratory Efficacy of Salanersen for Spinal Muscular Atrophy Thomas O. Crawford, MD
10:10 AM	22	DEVOTE Part C and ONWARD Integrated Results: Exploring High Dose Nusinersen in Nusinersen-Experienced Participants with Spinal Muscular Atrophy Richard S. Finkel, MD
10:30 AM		Break
11:00 AM	23	Intravenous Onasemnogene Apeparovect Gene Replacement Therapy for Presymptomatic Spinal Muscular Atrophy: Long-Term Follow-Up Analysis Megan A. Waldrop, MD

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

FOR QUESTIONS, PLEASE CONTACT SMARCC@CURESMA.ORG