


Annual SMA Research Meeting

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

DAY 1 | WEDNESDAY, JUNE 24, 2026

7:00 AM	Symposium: A Clinical Review of the High Dose Regimen of Nusinersen <i>Sponsored by Biogen</i> 
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RESEARCH MEETING

TIME	ABSTRACT #	SESSION
8:30 AM		Welcome Jackie Glascock, PhD, Chief Scientific Officer, Cure SMA
Genetics of Disease and Modeling Tools Moderator: Arthur Burghes, PhD		
8:40 AM	1	Modeling Neuromuscular Disorders: From Human NMJ Assembloids to a High-Throughput Functional Screening Platform for Drug Discovery Chen Kuchuan, PhD
9:15 AM	2	Excision of Mouse Smn Exon 2b Enhances Smn Function in SMA-Critical Pathways Anton Blatnik, PhD
Pre and Post Symptomatic Treatment Effect Moderator: Lyndsay Murray, PhD		
9:20 AM	3	The Neuropathology of Severe Human SMA and The Effects of Post-symptomatic Administration of Disease Modifying Therapies Maddi Denton, BS
9:40 AM	4	Prenatal Exposure to a Risdiplam-like Compound Partially Rescues the SMNΔ7 Mouse Model of Spinal Muscular Atrophy Without Postnatal SMN Intervention Emma Sutton, PhD
10:00AM	5	Prenatal Intervention in an SMA Mouse Model Rescues Neurodevelopmental Disorders Associated with Spinal Muscular Atrophy Charalambos Demetriou, PhD
10:30 AM		Break
SMN-Deficient Muscle Defects and Muscle-Targeted Therapy Moderator: Rashmi Kothary, PhD		
11:00 AM	6	Characterizing the Reversibility of Cellular and Molecular Defects of SMN-deficient Muscle Elana Molotsky, 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.

TIME	ABSTRACT #	SESSION
1:00 PM	5	2025 State of SMA Lisa Belter, MPH
1:30 PM	6	Neurodevelopment Panel Moderator: Thomas Crawford, MD Thomas Crawford 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 Moderator: Susan Matesanz, MD 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

THURSDAY, JUNE 25 | DAY 2

7:00 AM	<p>Symposium: Expert Perspectives on the Evolving Management of SMA <i>Sponsored by Scholar Rock</i></p> 
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TIME	ABSTRACT #	SESSION
		Treatment and Motor Function Moderator: Allison Ebert, PhD
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 Moderator: Robert Graham, MD Trasie Mead Jaclyn Greenwood Keishla Rolon

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> 
		<p>Clinical Outcome Measures Moderator: Katherine Klinger, PhD</p>
1:10 PM	13	<p>The Shuttle Squat Test: A Practical Tool for Assessing Endurance and Proximal Leg Muscle Strength in Spinal Muscular Atrophy Vincent Carson, MD</p>
1:30 PM	14	<p>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</p>
1:50 PM	15	<p>Long-Term, Real-World Spatiotemporal and Kinetic Gait Monitoring in Spinal Muscular Atrophy Using AI-Sole Kai-Chun Liu, PhD</p>
2:10 PM		Break
		<p>Treatment Outcomes and Biomarkers Moderator: Stephen Kolb, MD, PhD</p>
2:40 PM	16	<p>Long-Term Real-World Outcomes Following Onasemnogene Apeparvovec Monotherapy for Patients with Spinal Muscular Atrophy: Updated Findings from the RESTORE Registry Richard Finkel, MD</p>
3:00 PM	17	<p>Neurofilament Light Chain as a Biomarker in Spinal Muscular Atrophy Charlotte Sumner, MD</p>
3:20 PM	18	<p>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</p>
4:00 PM		<p>Poster Session B: Even Numbered Posters Presented Sponsored by Scholar Rock </p>
6:30 PM		Meet and Greet with Families: Featuring Annual Relay Race

DAY 3 | FRIDAY, JUNE 26

7:00 AM		<p>Symposium: Considerations in SMA Management Across the Lifespan: Newborns to Adults Sponsored by Genentech</p>  <p>A Member of the Roche Group</p>
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TIME	ABSTRACT #	SESSION
8:30 AM		Podium Poster Highlights
		<p>Clinical Therapeutic Development Moderator: Adrian Krainer, PhD</p>

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	Onasemnogene Apeparvovec 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