



Annual SMA Research & Clinical Care Meeting

June 5-7, 2024

	<i>Abstract Number</i>	<u>Wednesday June 5th, 2024</u>
7:30 AM		Research & Clinical Care Symposium: Unveiling the Practical Considerations of Gene Replacement Therapy Sponsored by Novartis
9:00 AM		Welcome Jackie Glascock, PhD, Vice President, Research, Cure SMA
9:05 AM		Basic Research: Block 1
9:05 AM	1	ROLE OF DLC1 LSOFORM 1 IN SPINAL MUSCULAR ATROPHY PATHOGENESIS AND TREATMENT Martin Cheung
9:25 AM	2	MUNC13-1 LOCAL TRANSLATION MITIGATES PRESYNAPTIC PATHOLOGY AND RESCUES MOTOR FUNCTION IN SPINAL MUSCULAR ATROPHY Mehri Moradi
9:45 AM	3	SMN ASSOCIATION WITH ALPHA-COP IS NOT CRITICAL TO SMA PATHOGENESIS Kaitlyn Kray, BS, MS
10:05 AM	4	SM-PROTEIN RINGS ARE ASSEMBLED ON MRNAS AND THESE MRNAS ARE LESS ABUNDANT IN SMA MICE Anton Blatnik, PhD
10:25 AM		BREAK
11:00 AM		Basic Research: Block 2
11:00 AM	5	SMN PROTEOME IDENTIFY UNCHARTED RNA METABOLISM PATHWAYS Olivier Binda, PhD
11:20 AM	6	SINGLE-NUCLEI RNA SEQUENCING REVEALS DEFECTS IN DIFFERENT CELL TYPES IN THE DELTA7 SMA MODEL Yongchao Ma, PhD
11:40 AM	7	IS SMA A DEVELOPMENTAL DISEASE? Federica Genovese, MSc
12:00 PM		LUNCH
1:00 PM		Basic Research: Block 3
1:00 PM	8	NEURODEVELOPMENTAL DISORDERS AND CORRELATION WITH MRI BRAIN ABNORMALITIES IN SPINAL MUSCULAR ATROPHY TYPE 1 Rodrigo Holanda Mendonça, MD, PhD
1:20 PM	9	DEVELOPMENT, VULNERABILITY AND RECOVERY OF INTRAMUSCULAR MOTOR AXONS IN MOUSE MODELS OF SMA Laura Comley, PhD



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1:40 PM

10	INVESTIGATING COMBINATIONAL THERAPEUTICS TO ENHANCE AXON REGENERATION IN MOUSE MODELS OF SMA Lyndsay Murray, PhD
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2:00 PM

11	CHARACTERIZING THE REVERSIBILITY OF CELLULAR AND MOLECULAR DEFECTS OF SMN-DEFICIENT MUSCLE Elana Molotsky, PhD
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2:20 PM

BREAK	
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2:40 PM

<i>Basic Research: Block 4</i>	
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2:40 PM

12	B-ADRENERGIC SIGNALING IS IMPAIRED IN CARDIOMYOCYTES IN A MOUSE MODEL OF SPINAL MUSCULAR ATROPHY Cecelia Mangione, BS
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3:00 PM

13	LIVER SMN RESTORATION RESCUES SMN2B/- MOUSE MODEL OF SMA: THE KEY TO RESCUE WHOLE-BODY PATHOLOGY? Emma Sutton, PhD
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3:20 PM

14	STEM CELL-DERIVED ASTROCYTES DEMONSTRATE REDUCED TENEURIN-4 LEVELS AND PERISYNAPTIC ASTROCYTE PROCESS DEFECTS IN SPINAL MUSCULAR ATROPHY Allison Ebert, PhD
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4:30 PM

Poster Session A: Odd Numbered Posters Presented	
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		<u>Thursday June 6th, 2024</u>
	<i>Abstract Number</i>	
7:30 AM		Research & Clinical Care Symposium: The Evolving SMA Landscape: Approaches and Considerations in the Era of Disease-Modifying Therapies Sponsored by Genentech
9:00 AM		Special Session: The Importance of Understanding Remaining Unmet Needs in SMA
9:00 AM		INTRODUCTION TO THE SESSION: THE SMA POPULATION Lisa Belter, MPH
9:20 AM		THE ADULT SMA EXPERINENCE Panel: Doug McCullough, Jaclyn Greenwood, Ryan Manriquez
10:10 AM		CLINICAL CARE: THE UNIQUE NEEDS OF ADULTS WITH SMA Bakri Elsheikh, MD
10:30 AM		REGENERATION AFTER CHRONIC DENERVATION Sophie Rengarajan, MD, PhD
10:50 AM		BREAK
11:10 AM		SMA AND AGING: LESSONS FROM SARCOPENIA Roger Fielding, PhD
11:30 AM		WHAT WE'VE LEARNED FROM SMA DRUG APPROVALS & HOW THAT SHAPES FURTHER DRUG DEVELOPMENT? TBA
11:50 AM		PANEL DISCUSSION All speakers
12:20 PM		LUNCH
1:20 PM		<i>Clinical Research</i>
1:20 PM	15	SWALLOWING PHYSIOLOGY AND FUNCTION IN UNTREATED PATIENTS WITH SPINAL MUSCULAR ATROPHY TYPE 1: ESTABLISHING NATURAL HISTORY REFERENCE VALUES Katlyn McGrattan, PhD
1:40 PM	16	NEWBORN SCREENING IN SMA MOTOR FUNCTION PROJECT (NBS-SMART): ACHIEVING CONSENSUS ON MOTOR FUNCTION OUTCOMES Kristin J. Krosschell, PT, DPT
2:00 PM	17	MOTOR UNIT INDEX (MUNIX) AS A BIOMARKER OF PROGRESSION IN 5Q-SPINAL MUSCULAR ATROPHY TYPES 3 AND 4 Rodrigo Holanda Mendonça, MD, PhD
2:20 PM		BREAK
2:40 PM	18	SMA COMMUNITY-BASED EXPERIENCE WITH THE 12-TIER FUNCTIONAL ABILITY SCALE FOR EVOLVING SPINAL MUSCULAR ATROPHY Meghan Moore Burk, PT, DPT



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3:00 PM

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RAPID RISDIPLAM INITIATION IN NEWBORNS WITH SPINAL MUSCULAR ATROPHY (SMA): A MULTICENTER, RETROSPECTIVE COHORT STUDY

Natalie Goedeker DNP, CPNP

3:20 PM

20

REAL-WORLD OUTCOMES FOLLOWING ONASEMNOGENE ABEPARVOVEC IN PATIENTS WITH SPINAL MUSCULAR ATROPHY AND INVASIVE VENTILATORY SUPPORT: FINDINGS FROM THE RESTORE REGISTRY

Richard Finkel, MD

3:40 PM

21

PRECLINICAL PHARMACOLOGY OF A 15-PGDH INHIBITOR, MF-300, THAT INCREASES SPECIFIC MUSCLE FORCE IN SMN DELTA 7 AND SMN1C/C MOUSE MODELS OF SPINAL MUSCULAR ATROPHY

Micah Webster

4:00 PM

Poster Session B: Even Numbered Posters Presented

6:30 PM

Meet and Greet with Families: Featuring Annual Relay Race



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	<i>Abstract Number</i>	<u>Friday June 7th, 2024</u>
7:30 AM		Research & Clinical Care Symposium: Insights Into Evolving Needs of SMA Patients as They Age: Claims Data and Expert Opinion Sponsored by Biogen
9:00 AM		Podium Poster Highlights
9:40 AM		Clinical Drug Development
9:40 AM	22	SAFETY AND TOLERABILITY OF ONASEMNOGENE ABEPARVOVEC FOR PATIENTS WITH SPINAL MUSCULAR ATROPHY WEIGHING ≤17 KG AND ≤24 MONTHS OLD: PHASE 4 OFELIA STUDY Nayla Mumneh, MD
10:00 AM	23	SAFETY AND EFFICACY OF INTRAVENOUS ONASEMNOGENE ABEPARVOVEC IN PEDIATRIC PATIENTS WITH SPINAL MUSCULAR ATROPHY: FINDINGS FROM THE PHASE 3B SMART STUDY Hugh McMillan, MD, MSc, FRCPC, FAAN
10:20 AM	24	INTERIM CLINICAL, NEUROFILAMENT, AND SAFETY RESULTS FROM THE OPEN-LABEL PHASE 4 RESPOND STUDY EVALUATING NUSINERSEN IN CHILDREN WITH SPINAL MUSCULAR ATROPHY PREVIOUSLY TREATED WITH ONASEMNOGENE ABEPARVOVEC Crystal Proud, MD
10:40 AM	25	FIREFISH PARTS 1 AND 2: 5-YEAR EFFICACY AND SAFETY OF RISDIPLAM IN TYPE 1 SMA Maria Mazurkiewicz-Beldzińska, MD, PhD
11:00 AM	25	STUDY DESIGN AND STATUS UPDATE ON THE SYNAPSE-SMA PHASE 2 TRIAL OF NMD670 IN AMBULATORY ADULTS WITH SPINAL MUSCULAR ATROPHY TYPE 3 Vera Kiyasova, MD, PhD
11:20 AM	26	TALDEFGROBEP ALFA: PRECLINICAL AND CLINICAL DATA SUPPORTING THE PHASE 3 RESILIENT STUDY IN SPINAL MUSCULAR ATROPHY Lindsey Lair, MD
11:40 PM		Closing Remarks Jackie Glascock, PhD, Vice President Research, Cure SMA
12:00 PM		Research and Clinical Care Meeting Adjourns
6:30 PM		Family Friendly Researcher Poster Session