



Cure SMA Research and Clinical Care Meeting

Research Meeting Agenda

June 15-17, 2022

	<i>Abstract Number</i>	<u>Wednesday June 15th, 2022</u>
7:00am-8:00am		Research Breakfast with Symposium: SMN Restoration in SMA Sponsored by Biogen
8:30 am to 8:35 am		Welcome Jackie Glasscock, PhD, Director of Research Programs, Cure SMA
8:35-9:55am		Basic Research: Session 1 Moderator: Arthur Burghes
8:35am-8:55am	1	IDENTIFICATION OF NOVEL MIRNAS AS PHARMACODYNAMIC BIOMARKERS IN SPINAL MUSCULAR ATROPHY Arlene D’Silva
8:55am am to 9:15 am	2	SMA-MIRS: MIR-181A-5P, -324-5P, -451A ARE OVEREXPRESSED IN SPINAL MUSCULAR ATROPHY SKELETAL MUSCLE AND SERUM SAMPLES Emanuela Abiusi
9:15am-9:35am	3	DEEP MOLECULAR CHARACTERIZATION OF SMA PATIENTS CARRYING THE C.859G>C VARIANT IN SMN2 Mar Costa-Roger
9:35am to 9:55am	4	THERAPEUTIC POTENTIAL OF AN SMN-INDEPENDENT DRUG THAT MODULATES THE GC-GR-KLF15 SIGNALLING PATHWAY IN SMA MICE Emma Sutton
9:55am to 10:25am		Break
10:25am to 11:45am		Basic Research Session 2 Moderator: Elliot Androphy
10:25am to 10:45am	5	PHARMACOLOGICAL TREATMENT IN VIVO REGULATING EXPRESSION OF THE POTASSIUM CHANNEL KV2.1 IN VULNERABLE MOTOR NEURONS PROVIDES SIGNIFICANT BENEFITS IN A SEVERE SMA MOUSE MODEL Nandhini Sivakumar
10:45am to 11:05am	6	NIFEDIPINE MITIGATES MOTOR NERVE TERMINAL AND MOTOR NEURON PATHOLOGY IN MOUSE MODELS OF SMA Lucia Tabares
11:05am to 11:25am	7	THE ROLE OF STIM1 AND THE STORE-OPERATED CALCIUM ENTRY IN SMA PATHOLOGY Tamás Schmidt
11:25am to 11:45am	8	GV-58 HAS THERAPEUTIC POTENTIAL AS A NOVEL CALCIUM CHANNEL GATING MODIFIER THAT IMPROVES



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		NEUROMUSCULAR TRANSMITTER RELEASE AND STRENGTH IN SPINAL MUSCULAR ATROPHY MODEL MICE Stephen Meriney
11:45am to 1:00pm		LUNCH
1:00pm to 3:20pm		<i>Basic Research Session 3</i> Moderator: Umrao Monani
1:00pm to 1:20pm	9	A CHEMICAL GENETIC SCREEN IDENTIFIES P38MAPK INHIBITION AS A NEUROPROTECTIVE STRATEGY FOR COMBINATORIAL SMA THERAPY Maria Carlini
1:20pm to 1:40pm	10	COMBINATION OF BIO101 WITH ANTISENSE OLIGONUCLEOTIDE THERAPY DEMONSTRATES SYNERGISTIC BENEFICIAL EFFECTS IN SEVERE SMA-LIKE MICE Cynthia Bezier
1:40pm to 2:00pm	11	CENTRAL AND PERIPHERAL DELIVERED AAV9-SMN ARE BOTH EFFICIENT BUT TARGET DIFFERENT PATHOMECHANISMS IN A MOUSE MODEL OF SPINAL MUSCULAR ATROPHY Aoife Reilly
2:00pm to 2:20pm	12	SCAAV9 DELIVERY OF AN SMN LACKING ALPHA-COP-BINDING RESIDUES RESCUES AN SMA MOUSE MODEL Anton Blatnik
2:20pm to 3:00pm		BREAK
3:00pm to 3:20pm	13	NEUROMUSCULAR JUNCTION RECOVERY FOLLOWING SMN RESTORATION IN MOUSE MODELS OF SPINAL MUSCULAR ATROPHY Lyndsay Murray
3:20pm to 3:40pm	14	THE ROLE OF ASTROCYTIC GLUTAMATE TRANSPORTERS IN SMA MOTOR NEURON SYNAPTIC DEFECTS Emily Welby
3:40pm to 4:00pm	15	MULTI-ORGAN PROTEOME LANDSCAPE OF SPINAL MUSCULAR ATROPHY Ines Tapken
4:30pm to 6:30pm		Poster Session A Cocktail Reception Sponsored by Novartis Gene Therapies



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<u>Thursday June 16th, 2022</u>	
7:00am to 8:00am	Researcher Breakfast with Symposium, Explore the Science for an Approved Spinal Muscular Atrophy Treatment and Hear from People living with SMA Sponsored by Genentech
8:30am to 12:00pm	<i>Special Session: Reverse Translation: How do real world observation inform the most pressing basic research questions?</i> Moderator: Charlotte Summer Speakers: Basil Darras, Michelle Farrar, Bakri Elsheikh, Stephen Kolb Panel Discussion
12:00pm to 2:00pm	Lunch and Poster Session B
2:30pm to 4:30pm	Clinical Research Session Moderator: Tom Crawford
2:30pm to 2:50 pm	16 FEASIBILITY OF THE ASSISTED 6 MINUTE CYCLE TEST FOR FATIGUE AND ENDURANCE FOR INDIVIDUALS WITH SMA WHO ARE NON-AMBULATORY Tina Duong
2:50pm to 3:10pm	17 THE SPINAL MUSCULAR ATROPHY CAREGIVER-HEALTH INDICES: DEVELOPMENT AND VALIDATION OF TWO NOVEL CAREGIVER REPORTED OUTCOME MEASURES FOR CLINICAL TRIALS Spencer Rosero
3:10pm to 3:30pm	18 AEROBIC CAPACITY IMPROVES IN ADULTS WITH SPINAL MUSCULAR ATROPHY TREATED WITH NUSINERSEN Jacqueline Montes
3:30pm to 3:50pm	19 BULBAR FUNCTION IN PATIENTS WITH SPINAL MUSCULAR ATROPHY TYPE 1 FOLLOWING ONASEMNOGENE ABEPARVOVEC Katlyn McGrattan
3:50pm to 4:10pm	20 REAL-WORLD FINDINGS FROM THE RESTORE REGISTRY: IMPACT OF NEWBORN SCREENING IN THE UNITED STATES, AND OUTCOMES OF ONASEMNOGENE ABEPARVOVEC TREATMENT IN OLDER PATIENTS WITH SMA Richard Finkel



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4:10pm to
4:30pm

21	<p>TREATMENT EFFECTS AMONG PATIENTS WITH TYPE II AND TYPE III SMA DIRECTLY REPORTED BY PATIENTS AND CAREGIVERS FROM THE TOPAZ CLINICAL TRIAL Hemal Shah</p>
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Friday June 17th, 2022

7:00am to
8:00am

	<p>Research Breakfast with Symposium, Exploring the Evolution of Bulbar Function in Spinal Muscular Atrophy Sponsored by Novartis Gene Therapies</p>
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8:30am to
9:20am

	<p style="text-align: center;">Podium Poster Highlights Moderator: Jackie Glascock</p>
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9:20am to
12:00pm

	<p style="text-align: center;">Clinical Drug Development Session Moderator: Katherine Klinger</p>
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9:20am to
9:40am

22	<p>NUSINERSEN EFFECT IN INFANTS IN THE PRESYMPTOMATIC STAGE OF SMA: 4.9-YEAR INTERIM OF THE NURTURE STUDY Nancy Kuntz</p>
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9:40am to
10:00am

23	<p>RESULTS FROM THE END OF PART A OF THE ONGOING 3-PART DEVOTE STUDY TO EXPLORE HIGHER DOSES OF NUSINERSEN IN SMA Richard Finkel</p>
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10:00am to
10:20am

24	<p>BASELINE CHARACTERISTICS AND INITIAL SAFETY RESULTS IN RESPOND: A PHASE 4 STUDY OF NUSINERSEN IN CHILDREN WITH SMA WHO RECEIVED ONASEMNOGENE ABEPARVOVEC Crystal Proud</p>
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10:20am to
10:40am

25	<p>ONASEMNOGENE ABEPARVOVEC IN PRESYMPTOMATIC SPINAL MUSCULAR ATROPHY: FINAL RESULTS OF SPR1NT STUDY FOR CHILDREN WITH THREE COPIES OF SMN2 Kevin Strauss</p>
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10:40am to
11:00am

26	<p>RAINBOWFISH: PRELIMINARY EFFICACY AND SAFETY DATA IN RISDIPLAM-TREATED INFANTS WITH PRESYMPTOMATIC SPINAL MUSCULAR ATROPHY (SMA) Richard Finkel</p>
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11:00am to
11:20am

27	<p>FIREFISH PARTS 1 AND 2: 36-MONTH SAFETY AND EFFICACY OF RISDIPLAM IN TYPE 1 SPINAL MUSCULAR ATROPHY (SMA) John Day</p>
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11:20am to
11:40am

28	<p>TOPAZ EXTENSION: 24-MONTH EFFICACY AND SAFETY OF APITEGROMAB IN PATIENTS WITH LATER-ONSET SPINAL MUSCULAR ATROPHY (TYPE 2 AND TYPE 3 SMA) Thomas Crawford</p>
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11:40am to
11:50am

Closing Remarks

Jackie Glasscock, PhD, Director of Research Programs, Cure
SMA

11:50am

Research and Clinical Care Meeting Adjourns