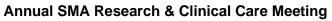


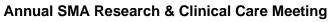
Annual SMA Research & Clinical Care Meeting

	Abstract Number	Wednesday June 28th, 2023
7:00 AM		Research Breakfast with Symposium
7.00 7 ((v)		Sponsored by Biogen: SMN Restoration in Adults Living with SMA
8:30 AM		Welcome
		Jackie Glascock, PhD, Vice President, Research, Cure SMA
8:35 AM		Basic Research: Session 1
		Moderator: Elliot Androphy, MD
8:35 AM	1	TOWARDS A PRENATAL THERAPY FOR PATIENTS WITH SEVERE SPINAL MUSCULAR ATROPHY - PRECLINICAL EXPERIMENTS IN MOUSE AND SHEEP MODELS
		Beltran Borges, MD
8:55 AM	2	TARGETING HISTONE ACETYLATION SPECIFICALLY TO SMN2 INTRON 6 USING A CRISPR-DEAD CAS9 STRATEGY COOPERATES WITH NUSINERSEN TO PROMOTE EXON 7 INCLUSION Alberto Kornblihtt, PhD
9:15 AM	3	A PERMANENT GENETIC TREATMENT FOR SPINAL MUSCULAR ATROPHY USING BASE EDITORS Christiano Alves, PhD
9:35 AM	4	MOTOR UNIT RECOVERY FOLLOWING AAV9-SMN TREATMENT IN A MOUSE MODEL OF SPINAL MUSCULAR ATROPHY
		Laura Comley, PhD
9:55 AM	5	SURVIVAL MOTOR NEURON (SMN) GENE THERAPY: ADDRESSING CONCERNS OF SENSORIMOTOR TOXICITY
10:15 AM		Maria Balch, PhD
		Coffee Break
10:45 AM		Basic Research Session 2 Moderator: Rashmi Kothary, PhD
10:45 AM	6	CEREBELLAR STRUCTURAL, GLIAL, AND NEURONAL DEFECTS IN A MOUSE MODEL OF SPINAL MUSCULAR ATROPHY Nicholas Cottam, BS
11:05 AM	7	CEREBELLAR PATHOLOGY IN TWO MOUSE MODELS FOR SPINAL MUSCULAR ATROPHY Florian Gerstner, MS
11:25 AM	8	HUMAN SPINAL CORD ORGANOID MODELS OF SPINAL MUSCULAR ATROPHY EXHIBIT EARLY NEURODEVELOPMENTAL DEFECTS
		Natalia Rodriguez-Muela, PhD
11:45 AM		Lunch
1:00 PM		Basic Research Session 3 Moderator: Adrian Krainer, PhD



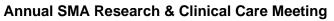


1:00 PM	9	ONE RING TO RULE THEM ALL: ARE SM-RING ASSOCIATED FUNCTIONS OF SMN CENTRAL TO THE
		DEVELOPMENT OF SMA?
4.00 DM	40	Anton Blatnik, PhD RAPID CYTOPLASMIC PHASE SEPARATION OF SMN IN
1:20 PM	10	RESPONSE TO CELLULAR STRESS
		Oliver Gruss, PhD
1:40 PM	11	MIR34 CONTRIBUTES TO SPINAL MUSCULAR ATROPHY
		(SMA) AND AAV9-MEDIATED DELIVERY OF MIR34A
		AMELIORATES THE MOTOR DEFICITS IN SMA MICE
		Tai-Heng Chen, MD, PhD
2:00 PM	12	NUCLEOLIN, AN SMN INTERACTING PROTEIN, BINDS WD- 40 DOMAIN OF ACOP THROUGH ITS DILYSINE MOTIF
		Timra Gilson, PhD
2:20 PM		Break
-		SMA PROTECTIVE MODIFIER PLASTIN 3 IS
2:40 PM	13	EPIGENETICALLY REGULATED BY THE MACROSATELLITE
		DXZ4 AND THE TRANSCRIPTION REGULATOR CHD4
		Brunhilde Wirth, PhD
3:00 PM	14	MITIGATING EFFECTS OF A SYNAPTIC CHAPERONE IN
		INTERMEDIATE SMN2B/- MODEL MICE
		Yoon Ra Her, PhD
3:20 PM	15	COMBINATORIAL THERAPY USING A ZOLGENSMA-LIKE DRUG WITH MIFEPRISTONE TO TARGET WHOLE-BODY
		PATHOLOGIES IN THE SMN2B/- MOUSE MODEL OF SMA
		Emma Sutton, PhD
4:30 PM		Poster Session A: Odd Numbered Posters Presented
1.00 1 101		Thursday June 29 th , 2023
		Thursday build 25 , 2020
7:00 AM		Researcher Breakfast with Symposium
		Sponsored by Genentech: Addressing the Evolving Needs of
		the SMA Community: The Next Phase of Evidence Generation
8:30 AM		Special Session: The State of SMA – Towards Improved
0.00.484		Therapeutics and Care Part 1: The Evolving SMA Patient Population: Unmet Needs
8:30 AM		and impact of Early Treatment
		Moderator: Charlotte Sumner, MD
8:30 AM		INTRODUCTION TO THE SESSION
0.00 /		Charlote Sumner, MD and Stephen Kolb, PhD
8:40 AM		SMA IN THE UNITED STATES: PERSPECTIVES FROM
		MULTIPLE REAL WORLD DATA
		SOURCES
0.00.414		Lisa Belter, MPH and Sarah Whitmire WHERE ARE THEY NOW? THE SMA NEWBORN
9:00 AM		SCREENING IDENTIFIED INFANTS'
		CLINICAL JOURNEY





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		Crystal Proud, MD
9:20 AM		WHAT'S NEXT? DRUGS IN THE PIPELINE
		Thomas Crawford, MD
9:40 AM		Part 1: Panel Discussion
10:20 AM		Coffee Break
10:40 AM		Part 2: The Importance of Understanding SMA pathobiology
		and Identification of New
		Druggable Targets
40.40.414		Moderator: Stephen Kolb, MD, PhD WHAT GENETICS AND BIOCHEMISTRY HAVE TOLD US
10:40 AM		ABOUT SMA AND SMN: WHERE WE GO FROM HERE
		Arthur Burghes, PhD
11:00 AM		THE ROLE OF SMN AT THE NEUOMUSCLAR JUNCTION:
11.007.001		KNOWN FUNCTIONS AND
		REMAINING QUESTIONS
		Umrao Monani, PhD
11:20 AM		UNDERSTANDING NEURONAL VULNERABLILITY: LESSONS FROM MOUSE MODELS
		Lyndsay Murray, PhD
11:40 AM		Part 2: Panel Discussion
		Lunch
12:20 PM		
1:20 PM		Clinical Research Session Moderator: Kathy Klinger, PhD
4.00 DM	16	ASSESSING PERCEIVED FATIGABILITY IN ADOLESCENTS
1:20 PM	16	AND ADULTS WITH SMA; RESULTS FROM A PILOT STUDY
		Rafael Rodriguez-Torres, DPT
1:40 PM	17	MUSCLE ULTRASOUND IN CHILDREN WITH SMA TREATED
		WITH EARLY MONO- OR DUAL THERAPY
		Susan E. Matesanz, MD
2:00 PM	18	SERUM NEUROFILAMENT LEVELS AND MAXIMUM ULNAR CMAP IN THE REAL-WORLD SETTING TO MONITOR
		IMPACT OF MOLECULAR THERAPIES ON CLINICAL
		OUTCOMES IN INFANTS WITH SMA INDICATE A
		POTENTIAL PROTECTIVE EFFECT OF NUSINERSEN
		DOSING PRIOR TO IV ONASEMNOGENE DOSING IN
		VULNERABLE SMA INFANTS
		Kathryn Swoboda, MD
2:20 PM	19	PRELIMINARY RESULTS FROM AN ONGOING PILOT CLINICAL TRIAL TESTING THE EFFECTS OF SPINAL CORD
		STIMULATION ON MOTOR FUNCTION IN HUMANS WITH
		TYPE 3 SMA
		Marco Capogrosso, PhD
2:40 PM	20	RISDIPLAM EXPERIENCE FOLLOWING
		ONASEMNOGENE ABEPARVOVEC IN INDIVIDUALS





		WITH SPINAL MUSCULAR ATROPHY: A
		MULTICENTER CASE SERIES
		Melissa D Svoboda, MD
3:00 PM	21	EVALUATION OF MOLECULAR RESPONSE TO RISDIPLAM
0.00 1 101		IN SMA PATIENTS THROUGH THE ANALYSIS OF SMN2
		TRANSCRIPT LEVELS ON PERIPHERAL BLOOD
		Emanuela Abiusi, MD
4:00 PM		Poster Session B: Even Numbered Posters Presented
		Friday June 30th, 2023
7:00 AM		Research Breakfast with Symposium
7.100 7		Sponsored by Novartis Gene Therapies: How Far We've Come
		with SMA: Two Patients' Journeys
8:30 AM		Podium Poster Highlights
		Moderator: Jackie Glascock, PhD
9:40 AM		Clinical Drug Development Session
0.10,441		Moderator: Samuel Pfaff, PhD
9:40 AM	22	INTERIM RESULTS FROM THE ONGOING RESPOND STUDY
		EVALUATING NUSINERSEN IN CHILDREN WITH SPINAL
		MUSCULAR ATROPHY PREVIOUSLY TREATED WITH
		ONASEMNOGENE ABEPARVOVEC
		Crystal Proud, MD
10:00 AM	23	FIREFISH PARTS 1 AND 2: 4-YEAR EFFICACY AND SAFETY
		OF RISDIPLAM IN TYPE 1 SPINAL MUSCULAR ATROPHY
		Giovanni Baranello, MD SUNFISH PARTS 1 AND 2: 4-YEAR EFFICACY AND SAFETY
10:20 AM	24	DATA OF RISDIPLAM IN TYPES 2 AND 3 SPINAL
		MUSCULAR ATROPHY (SMA)
		John W. Day MD, PhD
40.40 AM	25	EFFECT OF APITEGROMAB ON PEDI-CAT AND PROMIS-
10:40 AM	25	FATIGUE QUESTIONNAIRE AT 36-MONTHS IN PATIENTS
		WITH TYPE 2 AND NONAMBULATORY TYPE 3 SPINAL
		MUSCULAR ATROPHY
		Thomas O. Crawford, MD
11:00 AM	26	EFFECT OF APITEGROMAB ON MOTOR FUNCTION AT 36-
TT.00 AW	20	MONTHS IN PATIENTS WITH TYPE 2 AND
		NONAMBULATORY TYPE 3 SPINAL MUSCULAR ATROPHY
		Thomas O. Crawford, MD
11:20 AM		Closing Remarks
		Jackie Glascock, PhD, Vice President Research, Cure SMA
11:30 AM		Research and Clinical Care Meeting Adjourns