

# THURSDAY

JUNE 10, 2021

Basic and Clinical Research  
10:00 AM – 4:00 PM (CT)

10:00 AM – 11:00 AM	<b>Biogen Research Symposium</b>
11:00 AM - 11:05 AM	<b>Welcome</b> Jackie Glascock, PhD
<b>Special Session: Rehabilitation and Regenerative Therapies/Technologies</b>	
11:05 AM – 11:20 AM	<b>Introductory Remarks</b> Moderator, Joe Lewcock, PhD
11:20 AM – 11:40 AM	<b>TITLE TO BE ANNOUNCED</b> Joe Lewcock, PhD
11:40 AM – 12:00 PM	<b>TITLE TO BE ANNOUNCED</b> Michael Coleman, PhD
12:00 PM – 12:20 PM	<b>TITLE TO BE ANNOUNCED</b> Richard Robitaille, PhD
12:20 PM – 12:40 PM	<b>TITLE TO BE ANNOUNCED</b> Ahmet Hoke MD, PhD
12:40 PM – 1:00 PM	<b>Closing Remarks from Moderator and Panel Discussion</b>
1:00 PM – 2:00 PM	<b>Poster Session A</b>
<b>Basic Research Session</b>	
2:00 PM – 2:15 PM	<b>ASSEMBLY OF HIGHER-ORDER SMN OLIGOMERS IS ESSENTIAL FOR METAZOAN VIABILITY AND REQUIRES AN EXPOSED STRUCTURAL MOTIF PRESENT IN THE YG ZIPPER DIMER</b> Kushol Gupta, PhD
2:15 PM – 2:30 PM	<b>POST-TRANSLATIONAL MODIFICATIONS REGULATE FUNCTIONS OF THE SURVIVAL OF MOTONEURON (SMN) PROTEIN</b> Nora Tula Detering
2:30 PM – 2:45 PM	<b>CHROMATIN CONTROL HELPS THE EFFECTS OF NUSINERSEN ON SMN2 EXON 7 ALTERNATIVE SPLICING</b> Alberto Kornblihtt, PhD
2:45 PM – 3:00 PM	<b>RESTORATION OF SMN EXPRESSION IS REQUIRED IN MULTIPLE TISSUES FOR COMPLETE RESCUE IN A PRE-CLINICAL MODEL OF SPINAL MUSCULAR ATROPHY (SMA)</b> Aoife Reilly
3:00 PM – 3:15 PM	<b>COMPARISON OF NEUROMUSCULAR JUNCTION PATHOLOGY IN FOUR MOUSE MODELS OF SPINAL MUSCULAR ATROPHY (SMA) INDICATES DISTINCT PATTERNS OF SELECTIVE VULNERABILITY</b> Victoria Zimmer
3:15 PM – 3:30 PM	<b>IMPAIRMENT OF THE NEUROTROPHIC SIGNALING HUB B-RAF CONTRIBUTES TO MOTONEURON DEGENERATION IN SPINAL MUSCULAR ATROPHY (SMA)</b> Niko Hensel
3:30 PM – 3:45 PM	<b>THERAPEUTIC POTENTIAL OF REDUCING KRUPPEL-LIKE FACTOR 15 (KLF15) ACTIVITY IN SPINAL MUSCULAR ATROPHY (SMA)</b> Emma Sutton
3:45 PM – 4:00 PM	<b>AAV9-MEDIATED GENE THERAPY RESCUES THE NOVEL IGHMBP2D564N/D564N SPINAL MUSCULAR ATROPHY (SMA) WITH RESPIRATORY DISTRESS TYPE 1 (SMARD1) MOUSE MODEL</b> Caley Smith
4:00 PM	<b>Session Adjourns</b>

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# FRIDAY

JUNE 11, 2021

**Basic and Clinical Research  
10:00 AM – 4:20 PM (CT)**

RESEARCHER MEETING AGENDA

<b>10:00 AM – 11:00 AM</b>		<b>Novartis Gene Therapies Research Symposium</b>
<b>Clinical Research Session</b>		
<b>11:00 AM – 11:15 AM</b>	<b>NEWBORN SCREENING FOR SPINAL MUSCULAR ATROPHY (SMA) IN THE UNITED STATES: EARLY FINDINGS FROM THE RESTORE REGISTRY</b> Laurent Servais, MD, PhD	
<b>11:15 AM – 11:30 AM</b>	<b>BEYOND COPY NUMBER TO FURTHER RESOLVE GENOTYPE-PHENOTYPE DISCORDANCES: RESULTS OF SEQUENCING THE ENTIRE SMN2 GENE IN SMA PATIENTS WITH A NEW AFFORDABLE AND FAST METHOD</b> Eduardo Tizzano	
<b>11:30 AM – 11:45 AM</b>	<b>LONG-TERM NATURAL HISTORY OF DYNAMOMETER MEASURED MUSCLE POWER IN UNTREATED SPINAL MUSCULAR ATROPHY (SMA) TYPES 2 AND 3</b> Thomas Crawford, MD	
<b>11:45 AM – 12:00 PM</b>	<b>EVALUATING PERCEIVED FATIGUE WITHIN AN ADULT SMA POPULATION</b> Lisa Belter, MPH	
<b>12:00 PM – 12:15 PM</b>	<b>RESPIRATORY MUSCLE FATIGABILITY IN PATIENTS WITH SPINAL MUSCULAR ATROPHY (SMA)</b> Kim Kant-Smits, MSc	
<b>12:15 PM – 12:30 PM</b>	<b>ENTRUSTABLE PROFESSIONAL ACTIVITIES FOR PHYSICAL THERAPISTS WORKING WITH INDIVIDUALS WITH SPINAL MUSCULAR ATROPHY (SMA)</b> Jean Fitzpatrick Timmerberg	
<b>12:30 PM – 12:45 PM</b>	<b>INCREASED MOTOR UNIT RESERVE CAPACITY DURING ENDURANCE PERFORMANCE IN SPINAL MUSCULAR ATROPHY (SMA) PATIENTS TREATED WITH PYRIDOSTIGMINE</b> Laura E. Habets	
<b>12:45 PM – 1:00 PM</b>	<b>PRESERVED SWALLOWING FUNCTION IN INFANTS WHO INITIATED NUSINERSEN TREATMENT IN THE PRESYMPTOMATIC STAGE OF SMA: RESULTS FROM THE NURTURE STUDY</b> Enrico Bertini, MD	
<b>1:00 PM – 2:00 PM</b>	<b>Poster Session B</b>	
<b>Clinical Drug Development Session</b>		
<b>2:00 PM – 2:15 PM</b>	<b>TOPAZ: A PHASE 2 STUDY TO EVALUATE THE EFFICACY AND SAFETY OF APITEGROMAB (SRK-015) IN PATIENTS WITH LATER-ONSET SPINAL MUSCULAR ATROPHY (TYPE 2 AND TYPE 3 SMA): TOPLINE RESULTS</b> Thomas Crawford, MD	
<b>2:15 PM – 2:30 PM</b>	<b>SIX- AND 14-MONTH RESULTS OF SPINRAZA IN ADULTS WITH SPINAL MUSCULAR ATROPHY (SAS) STUDY</b> Craig M. Zaidman, MD	

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Continued

2:30 PM – 2:45 PM	<b>PROSPECTIVE OPEN-LABEL STUDY OF NUSINERSEN TREATMENT FOR ADULTS WITH SPINAL MUSCULAR ATROPHY (SMA): LONGER TERM RESULTS</b> Bakri Elsheikh, MD
2:45 PM – 3:00 PM	<b>NUSINERSEN IN LATER-ONSET SPINAL MUSCULAR ATROPHY: WALKING DISTANCE AND FATIGUE IN CS2/12 AND SHINE PARTICIPANTS</b> Jacqueline Montes, PT
3:00 PM – 3:15 PM	<b>PART A RESULTS FROM THE ONGOING DEVOTE STUDY TO EXPLORE HIGH-DOSE NUSINERSEN IN SMA</b> Richard Finkel, MD
3:15 PM – 3:30 PM	<b>ONASEMNOGENE ABEPARVOVEC GENE THERAPY IN PRE-SYMPTOMATIC SPINAL MUSCULAR ATROPHY (SMA): SPR1NT STUDY UPDATE IN CHILDREN WITH 2 AND 3 COPIES OF SMN2</b> Kevin Strauss, MD
3:30 PM – 3:45 PM	<b>LONG-TERM FOLLOW-UP (LTFU) OF ONASEMNOGENE ABEPARVOVEC GENE THERAPY IN SPINAL MUSCULAR ATROPHY (SMA)</b> John Day, MD, PhD
3:45 PM – 4:00 PM	<b>RAINBOWFISH: A STUDY OF RISDIPLAM IN INFANTS WITH PRESYMPTOMATIC SPINAL MUSCULAR ATROPHY (SMA)</b> Richard Finkel, MD
4:00 PM – 4:15 PM	<b>JEWELFISH: SAFETY, PHARMACODYNAMIC AND EXPLORATORY EFFICACY DATA IN NON-NAIVE PATIENTS WITH SPINAL MUSCULAR ATROPHY (SMA) RECEIVING TREATMENT WITH RISDIPLAM</b> Claudia Chiriboga, MD
4:15 PM – 4:20 PM	<b>Closing Remarks</b> Jill Jarecki, PhD
4:20 PM	<b>Session Adjourns</b>