

# WEDNESDAY

JUNE 9, 2021

10:00 AM – 5:30 PM (CT)

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| 10:00 AM – 11:00 AM | <b>Research &amp; Clinical Care Symposium, Sponsored by Genentech: Explore the Science for an Approved SMA Treatment and Hear from People Living with SMA</b><br><i>Continuing education credit will not be granted for this presentation</i>  |
| 11:00 AM – 11:15 AM | <b>Welcome and CMO Update</b><br>Mary Schroth, MD, Chief Medical Officer, Cure SMA   |
| 11:15 AM – 11:30 AM | <b>Cure SMA Community Database Outcomes</b><br>Lisa Belter, MPH  |
| 11:30 AM – 12:30 PM | <b>Special Session: Ethics</b><br>Moderator: Julie Parsons, MD<br>Panelists: Tom Crawford, MD; John Brandsema, MD; Bakri Elsheikh, MD<br>Ethicist: Brian Jackson, MD, MA   |
| 12:30 PM – 12:45 PM | <b>SINGLE INSTITUTION EXPERIENCE OF NUSINERSEN TREATMENT OF ADULT PATIENTS WITH SPINAL MUSCULAR ATROPHY (SMA)</b><br>Meghan Moore, PT, DPT, NCS  |
| 12:45 PM – 1:00 PM  | <b>FEASIBILITY OF TESTING DIFFERENCES IN SWALLOWING PHYSIOLOGY AND FUNCTION BETWEEN TREATED AND UNTREATED PATIENTS WITH SPINAL MUSCULAR ATROPHY (SMA) 1: NATURAL HISTORY VS. NUSINERSEN</b><br><i>Continuing education credit will not be granted for this presentation.</i><br>Katlyn McGrattan, PhD, CCC-SLP |
| 1:00 PM – 1:15 PM   | <b>CLINICAL EFFICACY AND SAFETY OF RISDIPLAM USE AS COMBINATION THERAPY IN SPINAL MUSCULAR ATROPHY (SMA) PATIENTS POST ONASEMNOGENE-ABEPARVOVEC-XIOI (ONASEMNOGENE) AND/OR NUSINERSEN USE</b><br>Kapil Arya, MBBS, FAAP, FANA, Paul Drake, MD  |
| 1:15 PM – 1:30 PM   | <b>NUSINERSEN BY SUBCUTANEOUS CATHETER FOR SPINAL MUSCULAR ATROPHY (SMA)</b><br>Karlla Brigatti, MS CGC  |
| 1:30 PM – 2:00 PM   | <b>Break</b>   |

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| <p>2:00 PM – 3:00 PM</p> | <p><b>Musculoskeletal Panel</b></p> <p><b>BRACING FOR SCOLIOSIS IN SPINAL MUSCULAR ATROPHY (SMA): IS IT EFFECTIVE?</b><br/>Brian Snyder, MD, PhD</p> <p><b>20-YEAR EXPERIENCE WITH SEGMENTAL SUBLAMINAR INSTRUMENTATION FOR GUIDED GROWTH IN EARLY ONSET SCOLIOSIS</b><br/>Samuel R. Rosenfeld, MD</p> <p><b>HIP PAIN ASSESSMENT IN PATIENTS WITH SPINAL MUSCULAR ATROPHY (SMA): PREVALENCE, INTENSITY, INTERFERENCE, AND ASSOCIATED FACTORS</b><br/>Amy Xu, BS</p> <p><b>DEVELOPMENT OF A COMPETENCY-BASED EDUCATIONAL PROGRAM FOR SPINAL MUSCULAR ATROPHY (SMA) PHYSICAL THERAPISTS (STEP-IN)</b><br/>Jacqueline Montes, PT, EdD</p> |
| <p>3:00 PM – 4:30 PM</p> | <p><b>Special Session: Mental Health Challenges in SMA-Affected Patients and Families: Symptoms, Resources and Solutions</b></p> <p><b>Moderator:</b> Vamshi Rao, MD</p> <p><b>Speakers/Panelists:</b> Pamela Mosher, MD; Vanessa Battista, RN, MS, CPNP-PC, CHPPN; Diane Murrell, LCSW; Al Freedman, PhD</p>  |
| <p>4:30 PM – 5:30 PM</p> | <p><b>SMA Newborn Screening Panel</b></p> <p><b>INTEGRATING A PILOT NEWBORN SCREENING FOR SPINAL MUSCULAR ATROPHY (SMA) INTO THE AUSTRALIAN PUBLIC HEALTHCARE SYSTEM</b><br/>Arlene D'Silva, PhD</p> <p><b>STATEWIDE NEWBORN SCREENING FOR SPINAL MUSCULAR ATROPHY (SMA): THE WISCONSIN EXPERIENCE</b><br/>Mei Baker, MD</p> <p><b>THE CLINICIANS AND FAMILIES EXPERIENCE WITH THE FIRST YEAR OF NEWBORN SCREENING IN COLORADO AND WYOMING</b><br/>Melissa Gibbons, MS, CGC</p> <p><b>INVESTIGATING THE SMA PATIENT COMMUNITY'S ATTITUDES TOWARDS FETAL THERAPY</b><br/>Marisa Schwab, MD</p>  |
| <p>5:30 PM</p>           | <p>Session Adjourns</p>  |