



CCDS Scientific + Patient
SYMPOSIUM
2026 | MIDWAY, UTAH

Yapp



SCAN TO DOWNLOAD
THE SYMPOSIUM APP
AND VIEW A
DETAILED AGENDA
WITH BIOS &
ABSTRACTS

CODE:8CF45V

SCHEDULE OF EVENTS

THURSDAY, JUNE 25	
2:00pm - 5:00pm	KIDS CAMP (DROP OFF BEGINS AT 1:30PM)
2:00pm - 5:00pm	OUTCOME MEASUREMENTS SESSION
<p><i>PAReNts 2.0 Alignment Jam - Integrating Expert Recommendations and Caregiver Input for CCDS Outcome Measurement Tools</i></p> <p>Andreas Schulze, University of Toronto/SickKids - Understanding Variability of Plasma GAA Measurements</p> <p>Jamie Capal, Cincinnati Children's Hospital Medical Center - Measuring Seizures in a Clinical Trial</p> <p>Beth Potter, University of Ottawa; Audrey Thurm, Boston Children's Hospital - Measurement Tools Used in Published Trials</p> <p>Jill Henley, BrainWise - Parent Talk</p> <p>Audrey Thurm, Boston Children's Hospital - Type of Clinical Trials & Risks</p> <p>Celeste Graham, ACD - Report: PAReNts 2.0 Smallest Meaningful Changes</p> <p>Emily Reinhardt, ACD - Report: Community Survey Results</p> <p>Judi Miller, CHOP; Jill Henley, BrainWise - Alignment of Existing Measurement Tools with the Needs of Our CCDS Community</p>	
6:00pm - 7:30pm	DINNER
FRIDAY, JUNE 26	
7:30am - 9:00am	BREAKFAST
8:00am - 9:00am	KIDS CAMP DROP OFF (AM DROP OFF BY 8:10; PM DROP OFFS 8:40-9:00)
9:00am - 10:20am	SESSION 1: OVERVIEW OF CCDS AND TREATMENT TOOLS
<p>Heidi Wallis, ACD - Welcome and Opening Remarks</p> <p>Brittney & Kevin McKittrick - PAReNts 2.0 CTD Spotlight</p> <p>Nicola Longo, UCLA - Overview of Cerebral Creatine Deficiency Syndromes</p> <p>Kim Cecil, Cincinnati Children's Hospital Medical Center - Pediatric Magnetic Resonance Spectroscopy Harmonization</p>	
10:20am - 10:30am	COFFEE BREAK
10:30am - 12:00pm	SESSION 2: MODELS OF CEREBRAL CREATINE DEFICIENCIES
<p>Laura Baroncelli, Neuroscience Institute CNR - Preclinical Models of CTD: Strengths, Limitations and Translational Opportunities</p> <p>Aleksander Bogoniewski, UCLA - Elucidating the Neurodevelopmental and Metabolic Mechanisms of GAMT Deficiency Using Stem Cell-Derived Model Systems</p> <p>Emil Alexov, Clemson University - Cracking the Code: Mapping the Full Translocation Pathway of Creatine Across the Creatine Transporter</p>	
12:00pm - 1:00pm	LUNCH
1:00pm - 1:45pm	SESSION 2 CONTINUED
<p>Hana Young - PAReNts 2.0 GAMT Spotlight</p> <p>Hanna Polari, Revvity - Plenary Talk: Towards Expanded Newborn Screening for GAMT Deficiency</p>	

FRIDAY, JUNE 26 CONTINUED	
1:45pm - 3:20pm	SESSION 3: ADVANCES IN THERAPEUTIC DEVELOPMENT FOR CCDS – I
<p>Preeti Vyas, Johns Hopkins University - Novel Glucose Dendrimer Creatine Conjugates for Intracellular Delivery of Creatine in CTD Alex Edwin, Stanford University - Beyond the Transporter: Developing Small Molecule Creatine Delivery Strategies for CTD Chin-Yi Chen, Virginia Tech - Focused Ultrasound-Mediated Blood-Brain Barrier Opening Enhances Delivery of Creatine to the Brain for CTD Carmon Phillips - PAREnts 2.0 GAMT Spotlight</p>	
3:20pm - 3:30pm	COFFEE BREAK
3:30pm - 4:30pm	KEYNOTE SPEAKER: EMIL KAKKIS, ULTRAGENYX <i>The Development of Rare Disease Therapies for Neurologic Genetic Diseases</i>
5:30pm - 6:30pm	POSTER SESSION
6:30pm - 8:00pm	DINNER & KIDS' ACTIVITY WITH RESEARCHERS
SATURDAY, JUNE 27	
7:30am - 9:00am	BREAKFAST (KIDS CAMP DROP OFF 8:30-8:55)
8:00am - 9:00am	KIDS CAMP DROP OFF (AM DROP OFF BY 8:10; PM DROP OFFS 8:40-9:00)
9:00am - 10:20am	SESSION 4: COGNITIVE OUTCOMES IN CLINICAL TRIALS
<p>Sangeetha Iyer, ACD - Opening Remarks Ashlea Pfannenstiel - PAREnts 2.0 CTD Spotlight Audrey Thurm, Boston Children's Hospital - Update on Parents Advancing REsearch NeTworks 2.0 Aurore Curie, Lyon University Hospital - CREAT_criteria: A Prospective Study in Male and Female CTD Patients to Determine the Most Relevant Outcome Measures</p>	
10:20am - 10:30am	COFFEE BREAK
10:30am - 12:00pm	SESSION 5: ADVANCES IN THERAPEUTIC DEVELOPMENT FOR CCDS – II
<p>Gerry Lipshutz, UCLA - Restoring Creatine in the Brain in GAMT and CTD: Recent Achievements and an FDA Path Forward Alanna Schepartz, UC Berkeley - Mini-protein Guided Delivery of Bio-active Substances Sara Biagiotti, University of Urbino - A Novel Therapeutic Strategy for GAMT Based on RBCEVs Loaded with IVT-GAMT mRNA</p>	
12:00pm - 1:00pm	LUNCH
1:00pm - 2:30pm	SESSION 6: CCDS – DEFINING PATIENTS, MEASURING DISEASE, TRACKING OUTCOMES
<p>Melissa Evans - PAREnts 2.0 CTD Spotlight Carole Chehowah - Xtraordinaire Update Israel Abebe Admasu, Boston Children's Hospital - Establishing Novel Functional Biomarkers for Creatine Deficiency Syndromes Emily Reinhardt, ACD - PAREnts 2.0 Project / Registry Sarah Donoghue, Murdoch Children's Research Institute - Creatine Supplementation in an Adult Female Following Diagnosis: Clinical and Biochemical Progression</p>	
2:30pm - 2:40pm	COFFEE BREAK
2:40pm - 3:35pm	SESSION 6 CONTINUED
<p>Kimberly Morris - PAREnts 2.0 CTD Spotlight Thomas Joudinaud, Ceres Brain Therapeutics - CBT101 - Poised to enter Phase 2 clinical trial in CTD Judith Miller, Children's Hospital of Philadelphia - Clinical Trial Development Lessons from Vigilant and Related Studies</p>	
3:35pm - 4:20pm	PANEL DISCUSSION: CCDS CLINICAL TRIAL READINESS - THERAPEUTIC STRATEGIES & OUTCOME MEASURES
<p>Judith Miller (CHOP), Nicola Longo (UCLA), Andreas Schulze (University of Toronto/SickKids), Thomas Joudinaud (Ceres Brain Therapeutics) Richard Collis (Ultragenyx)</p>	
4:20pm - 4:30pm	CLOSING COMMENTS: DAN COLLER, ACD BOARD CHAIR
5:30pm - 9:00pm	CHAMPIONS FOR A CURE GALA
SUNDAY, JUNE 28 (OPTIONAL FAMILY DAY)	
7:30am - 9:00am	GRAB & GO BREAKFAST
12:30pm - 1:30pm	FAMILIES' LUNCH AT THE GAZEBO