

CNS and Myotonic Dystrophy: Outcomes Measures Development Workshop
Myotonic
September 12, 2019
Philadelphia Convention Center
Philadelphia, PA

8:30 – 8:35 am	Welcome & Workshop Objectives Molly White, Myotonic
8:35 – 9:00 am	Opportunities and Challenges of Targeting the CNS in DM1 and CDM John Day, MD, Ph.D., Stanford University
9:00 – 9:40 am	Panel Discussion #1: Opportunities and Challenges in Targeting the CNS Moderator: Tetsuo Ashizawa, M.D., Houston Methodist Discussants: John Day, M.D., Ph.D., Stanford University, Giovanni Meola, M.D., IRCCS Policlinico San Donato, Nicholas E. Johnson, M.D., VCU, Charles Thornton, M.D., University of Rochester
9:40 – 9:50 am	Industry Perspective on Requirements for CNS Biomarkers Gersham Dent, Ph.D., Biogen
9:50 – 10:00 am	Coffee Break
10:00 - 10:30 am	Measuring RNA in CSF: Insights from the NIH Extracellular RNA Communication Consortium Julie A. Saugstad, Ph.D., Oregon Health Sciences University
10:30 – 11:45 am	Presentations on Potential CNS Biomarkers for DM Moderator: Nicholas E. Johnson <ul style="list-style-type: none">• Extracellular RNA in CSF: Eric Wang, Ph.D., University of Florida• Extracellular RNA in Blood/Urine: Thurman Wheeler, M.D., Massachusetts General Hospital• Imaging: Peg Nopoulos, M.D., University of Iowa
11:45 am	Working Lunch
12:00 – 1:15 pm	Discussion #2: Status of CNS Biomarkers for DM—Getting to Trial Readiness Moderator: John Carulli, Ph.D., Syros Pharmaceuticals Discussants: Eric Wang, Ph.D., University of Florida and Expansion Therapeutics, Doug Kerr, M.D, Ph.D., MBA, GenerationBio, Joe Horrigan, M.D., AMO Pharma
1:15 – 1:30 pm	Industry Perspectives on Requirements for Clinical Trial Outcome Measures Laurence Mignon, Ph.D., Ionis Pharmaceuticals

- 1:30 – 2:30 pm **Presentations on Potential CNS Clinical Endpoints for DM**
Moderator: Chad Heatwole, M.D., University of Rochester
Sleep: Jacinda Sampson, M.D., Ph.D., Stanford University and Valeria Sansone, M.D., Clinico NEMO
Cognition/Behavior/Apathy: Benjamin Gallais, Ph.D., kleCEGEP
NIH Toolbox: Mathula Thangarajh, M.D., Ph.D., Virginia Commonwealth University
- 2:30 – 2:40 pm **Coffee Break**
- 2:40 – 3:15 pm **Panel Discussion #3: Optimization of CNS Endpoints for DM**
Moderator: Sharon Hesterlee, Ph.D., AskBio
Discussants: Workshop participants
- 3:15 – 3:45 pm **Considerations Regarding CNS Endpoints for CDM**
Anne-Berit Ekstrom, M.D., University of Gothenburg
- 3:45 – 4:00 pm **Synthesis and Conclusions**
Tetsuo Ashizawa, M.D., Houston Methodist Neurological Institute