

2019 CNS Outcome Measures Development Workshop
Thursday, September 12, 2019
8:30 AM – 4:00 PM

Philadelphia Convention Center
1101 Arch St, Philadelphia, PA
Room 113 AB

- 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 Neurological Institute
Discussants: John Day, M.D., Ph.D., Stanford University, Giovanni Meola, M.D., IRCCS Policlinico San Donato, Nicholas E. Johnson, M.D., Virginia Commonwealth University, Charles Thornton, M.D., University of Rochester
- 9:40 – 9:50 am **Requirements for CNS Biomarkers – Industry Perspective**
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 **Potential CNS Biomarkers for DM**
Moderator: Nicholas E. Johnson, Virginia Commonwealth University
Presentations:
- Extracellular RNA in CSF: Eric Wang, Ph.D., University of Florida
 - Extracellular RNA in Serum/Urine: Thurman Wheeler, M.D., Massachusetts General Hospital
 - Brain Imaging: Peg Nopoulos, M.D., University of Iowa
- 11:45 am Working Lunch
- 12:00 – 1:20 pm **Status of CNS Biomarkers for DM—Getting to Trial Readiness**
Moderator: John Carulli, Ph.D., Syros Pharmaceuticals
Discussants: Matthew Disney, Ph.D., Scripps Florida and Expansion Therapeutics, Douglas Kerr, M.D, Ph.D., MBA, GenerationBio, Joseph Horrigan, M.D., AMO Pharma

- 1:20 – 1:30 pm **Requirements for Clinical Trial Outcome Measures – Industry Perspective**
Laurence Mignon, Ph.D., Ionis Pharmaceuticals
- 1:30 – 2:30 pm **Potential CNS Clinical Endpoints for DM**
Moderator: Chad Heatwole, M.D., University of Rochester
Presentations:
Sleep: Jacinda Sampson, M.D., Ph.D., Stanford University and Valeria Sansone, M.D., Clinico NEMO
Cognition/Behavior/Apathy: Benjamin Gallais, Ph.D., CEGEP
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