Welcome & Workshop Objectives
Molly White, Myotonic

Opportunities and Challenges of Targeting the CNS in DM1 and CDM
John Day, MD, Ph.D., Stanford University

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

Industry Perspective on Requirements for CNS Biomarkers
Gersham Dent, Ph.D., Biogen

Coffee Break

Measuring RNA in CSF: Insights from the NIH Extracellular RNA Communication Consortium
Julie A. Saugstad, Ph.D., Oregon Health Sciences University

Presentations on Potential CNS Biomarkers for DM
Moderator: Nicholas E. Johnson
- 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

Working Lunch

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

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