

# PROFESSIONAL TRACK AGENDA - Friday, September 14, 2018

Time	Professional Sessions
7:30 AM – 8:00 PM	<p align="center"><b>Professionals' Breakfast</b> <i>Governor's Chamber D &amp; E</i></p>
8:00 AM – 9:00 AM	<p align="center"><b>Drug Development Roundtable – Natural History Data Updates</b> Moderator: Kathie Bishop, PhD Speakers: Ami Mankodi, MD: "Results from the Multicenter Observational Study of Myotonic Dystrophy Type 1"; Nicholas E. Johnson, MD: "Update on the Trial Readiness and Endpoint Assessment in CDM1 Study"; Cynthia Gagnon, PhD: "Results from a 9-Year Longitudinal Study in DM1 Patients" <i>Governor's Chamber D &amp; E</i></p>
9:00 AM – 9:20 AM	<p align="center"><b>Population-Based Prevalence Study in Myotonic Dystrophy</b> Moderator: Kathie Bishop, PhD Speaker: Nicholas E. Johnson, MD: "Population-Based Prevalence Study in Myotonic Dystrophy Type-1 and Type-2" <i>Governor's Chamber D &amp; E</i></p>
9:20 AM – 11:00 AM	<p align="center"><b>Current Advances in DM2 Research</b> Moderator: Laura Ranum, PhD Speakers: Andy Berglund, PhD: "Blocking Transcription in DM2"; Chad Heatwole, MD: "Measuring Patient-Reported Disease Burden in DM2"; Laura Ranum, PhD: "RAN Translation and DM2"; Charles Thornton, MD: "STOPP-DM2"; Lukasz Sznajdar, PhD: "Intron Retention in DM2" <i>Governor's Chamber D &amp; E</i></p>
11:00 AM – 11:10 AM	<p align="center"><b>Coffee Break</b> <i>Governor's Chamber D &amp; E</i></p>
11:10 AM – 12:30 PM	<p align="center"><b>Molecular &amp; Imaging Biomarker Development for DM Studies</b> Moderator: Charles Thornton, MD Speakers: Donovan Lott, PhD: "Development of Muscle MRI Endpoints as a Potential Biomarker for DM1"; Peg Nopoulos, PhD: "MRI Studies on Brain Structure and Function in DM1 Patients"; Charles Thornton, MD: "Updates on the Development of a Muscle Splicing Biomarker Assay"; Thurman Wheeler, MD: "Extracellular RNA as a Biomarker for Myotonic Dystrophy" <i>Governor's Chamber D &amp; E</i></p>
12:30 PM – 1:30 PM	<p align="center"><b>Lunch</b> <i>Governor's Chamber D &amp; E</i></p>
1:30 PM – 1:50 PM	<p align="center"><b>Clinical Efficacy of Mexiletine</b> Moderator: Tom Cooper, MD Speaker: Chad Heatwole, MD: "Clinical Efficacy Trial of Mexiletine for Myotonic Dystrophy Type 1" <i>Governor's Chamber E</i></p>
1:50 PM – 3:00 PM	<p align="center"><b>DM Disease Burden: Results from Recent Research</b> Moderator: Tom Cooper, MD Speakers: Oliver Tobin, MD: "Myotonic Dystrophy Burden of Disease Analysis"; Katharine Hagerman, PhD: "Report from the Christopher Project"; Elizabeth Ackermann, PhD: "Myotonic Family Registry Update and Outcomes from MDF CNS Workshop"; Natalie Street, MS, CGC: "Updates on Myotonic Dystrophy Data Collection in the MD-STAR Net" <i>Governor's Chamber E</i></p>
3:00 PM – 3:50 PM	<p align="center"><b>Poster Session and Research &amp; Vendor Showcase</b> <i>Governor's Ballroom &amp; Lobby</i></p>
4:00 PM – 5:00 PM	<p align="center"><b>Gene Editing and Therapy Development</b> Moderator: Darren Monckton, PhD Speakers: Darren Monckton, PhD: "Summary of the MDF Genome Editing Workshop"; Eric Wang, PhD: "Gene Therapy and Gene Editing for DM – Progress, Promise, and Considerations"; Ranjan Batra, PhD: "Transcriptome Editing for Therapeutic Treatment of Myotonic Dystrophy" <i>Governor's Chamber E</i></p>