	Monday (8/26/19)		Tues (8/27/19)		Wed (8/28/19)		Thurs (08/29/19)		Friday (8/30/19)
8:15	Check-in/Welcome/introductions		Principles of Gene Editing Angela Lek, PhD		Molecular Pathogenesis of the Myotonic Dystrophies (DM1 and DM2) Nicolas Wein, PhD		Congenital Myopathies Megan Waldrop, MD		Mitochondrial Myopathies Michio Hirano, MD
9:00	The Dystrophinopathies: Clinical and Genetics Kevin Flanigan, MD		Principles of Gene Therapy Afrooz Rashnonejad, PhD		FSHD Genetics and Pathogenesis Nizar Saad, PhD		Sarcolemma in Health and Disease Noah Weislieder, PhD		Autoimmune Neuropathies Adam Quick, MD
9:45	:45 Break		Break		Break		Break		Break
10:00	DMD Pathophysiology Jill Rafael-Fortney, PhD		FSHD and DM1 Clinical Bakri El-Sheikh		SMA Clinical Megan Waldrop, MD		Anne Connolly, MD Congenital Myasthenic Syndromes 10:00a - 10:30a		Determining the cause of weakness in critically ill patients Mark Rich, MD, PhD
Tues only: 10:30			- Dak						
10:45	Intro to Neuromuscular Pathology Daniel Boue, MD, PhD				ALS: Clinical and Genetic Features Stephen Kolb, MD, PhD		SMA: Therapeutics Arthur Burghes, PhD		NT-3 Therapy for CMT Zarife Sahenk, MD, PhD
Tues only: 11:00			The LGMDs						<u> </u>
11:30	Overview of the Design of Muscle for Force Production and Transmission and Defects that result Disease Lee Sweeney, PhD		Matt Wicklund, MD Note: <u>11:00 to 11:45 AM</u>		Muscle stem cell populations in development and disease Emanuela Gussoni, PhD		Disorders of Glycosylation Paul Martin, PhD		Inflammatory/Acquired Myopathies Miriam Freimer, MD
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Tues & Thurs only) 12:15	Lunch		Clinical and Translational Research Seminar: Bertrand Fontaine, MD, PhD Muscle Channelopathies: from bench to patient care and discoveries" Insitute of Myology, Paris		Lunch		Child Health Research Center Seminar: Perry Shieh, MD, PhD Gene Therapy for Myotubular Myopathy David Geffen School of Medicine at UCLA Ronald Reagan UCLA Medical Center		Lunch
Friday only: 12:30									PPMD Amanda Wilkinson, RN, BSN 12:45
1:00	Bus to OSU (selected wet labs)		Bus to OSU (selected wet labs)		Bus to OSU (selected wet labs)		Bus to OSU (selected wet labs)		Career Development for the Young
	Clinical track	Lab track (wet labs)	Clinical track	Lab track (wet labs)	Clinical track	Lab track (wet labs)	Clinical track	Lab track (wet labs)	Investigator: insights from the NIH and
1:15	Glycogenoses Chad Hoyle, MD	Muscle Histopathology (NCH) Melanie Herring Megan Berger	Adult Cases from the Clinic/Clinical Vignettes Bakri Elsheikh, MD Matt Wicklund, MD	Seahorse Lab Kedryn Baskin, PhD (OSU) BRT 380	Bone Health in NMD Garey Noritz, MD	- Electroporation of DNA into	The Floppy Infant Reghan Foley, MD	In vivo mouse electrophysiology and physiology lab Dave Arnold, MD (OSU) 207 Rightmire Hall Detection Methods for Muscle Post-translational Modification Brandon Biesiadecki, PhD (OSU) 5199 Graves Hall Gene Delivery Techniques and Tissue Harvesting Kaltnin Meyer, PhD (NCH) WA2109	private foundations Tom Cheever, PhD
2:00	Emery-Dreifuss / Iaminopathies Albert Tsao, MD				Cardiac Care in the Muscular Dystrophies Linda Cripe, MD	Federica Accornero DHLRI 519	Pediatric Cases from the Clinic/Clinical Vignettes Reghan Foley, MD Perry Shieh, MD, PhD		Career Development for the Young Investigator: the view from academia Dennis Durbin, MD
2:45	Break	myoblasts from mice ill Rafeel-Fortew, PhD (OSU) 410 Hamilton Hall (OSU) 410 Hamilton Hall Generation Techniques for Muscle Physiology Lab	Break	Introduction to Flow Cytometry to Study Muscle Progenitor Cells Emanuela Gussoni, PhD (NCH) WA2525 Zebrafish Wet Lab Sharon Amacher, PhD (OSU) Rightmire	Break	electrophysiology and physiology lab Dave Arnold, MD (OSU) 207 Rightmire Hall Introduction to Flow Cytometry to Study Muscle Progenitor Cells Emanuela Gussoni, PhD (NCH) WA3525			Assessment/Feedback/Dismissal
3:00	Use and Interpretation of Genetic Testing Matthew Pastore, MS, CGC		Adult Cases Continued (see above)		Neuromuscular Junction Bakri Elsheikh, MD				
3:45			Clinical Outcomes Measures Linda Lowes, PhD Lindsay Alfano, DPT		Ventilation for NMD Grace Paul, MD				
4:30	Bus to OSU DHLRI 165		Bus from OSU		Bus to OSU DHLRI 165			Bus from OSU	
5:00 PM Keynote	Keynote (@OSC, DHLRI 165) "New insights into gene expression regulation in muscle". Federica Accornero, PhD Ohio State University		Keynote (@NCH) Genome Interpretation and Diagnosis of Muscular Dystrophies Monkol Lek, PhD Yale University		Keynote (@OSC, DHLRI 165) Bench to Bedside Stories-Gene Therapy for Neurodegenerative Diseases Kathrin Meyer, PhD NCH Center for Gene Therapy		Keynote (@NCH) Emerging Therapeutics for Muscle Disease Jerry Mendell, M.D. NCH Center for Gene Therapy		
	Introductory Mixer (7:30 pm) Hilton Hotel							ner (7:30 pm) Meridien	