

6:30–7:45 AM	Industry Forum Breakfast	Presented by Biogen	Orlando L-M
8:00–9:00 AM	General Session	Conference Opening R. Rodney Howell, MD, Miller School of Medicine, University of Miami Lynn O'Connor-Vos, Muscular Dystrophy Association	Regency R-S
9:00–10:00 AM	General Session	Keynote Presentation Janet Woodcock, MD, Center for Drug Evaluation and Research (CDER) at the Food & Drug Administration	Regency R-S
Exhibit Hall Open 10:00 AM–8:00 PM			Regency Foyer
10:00–10:30 AM	Break		
10:30 AM–12:00 PM	General Session	FDA Panel Janet Woodcock, MD, Center for Drug Evaluation and Research (CDER) at the Food & Drug Administration Peter Marks, MD, PhD, Center for Biologics Evaluation and Research, Food & Drug Administration John Day, MD, PhD, Stanford University Frank Sasinowski, MS, MPH, JD, Hyman, Phelps & McNamara Kristin Stephenson, MHA, J.D. Muscular Dystrophy Association	Regency R-S
12:00–1:15 PM	Industry Forum Lunch	Presented by AveXis	Orlando L-M
	Industry Forum Lunch	Presented by Sanofi Genzyme	Regency Q
	Industry Forum Lunch	Presented by Sarepta Therapeutics	Florida B-C
1:30–3:30 PM	Clinical Session	Strategies for Managing an MDA Care Center (CME certified) Stan Appel, MD, Houston Methodist Neurological Institute (Chair) Lora Clawson, MSN, CRNP, Johns Hopkins University School of Medicine (Chair) John Day, MD, PhD, Stanford University Katherine Mathews, MD, University of Iowa Michael Shy, MD, University of Iowa	Regency T
	Clinical Session	Genetics for Healthcare Providers (CME certified) Laurie Gutmann, MD, University of Iowa (Chair) Shawna Feely, MS, CGC, University of Iowa Tahseen Mozaffar, MD, University of California Irvine	Regency U
	Scientific Session	Gene-Targeting Therapies 1 Jeffrey Chamberlain, PhD, University of Washington School of Medicine (Chair) Robert Brown, DPhil, MD, University of Massachusetts Medical School (Chair) Robert Burgess, PhD, The Jackson Laboratory Nathalie Clement, PhD, University of Florida Kevin Kaifer, PhD, University of Missouri Ambreen Sayed, PhD, The Jackson Laboratory	Regency P
3:30–4:00 PM	Break		Regency Foyer
4:00–6:00 PM	Clinical Session	What's New in CMT (CME certified) Charlotte Sumner, MD, Johns Hopkins University School of Medicine (Chair) John Svaren, PhD, University of Wisconsin-Madison Stephan Zuchner, MD, PhD, University of Miami School of Medicine	Regency T
		What's New in Inclusion Body Myositis (CME certified) Thomas Lloyd, MD, PhD, Johns Hopkins University School of Medicine (Chair) Namita Goyal, MD, University of California Irvine Steven Greenberg, MD, Harvard Medical School	
	Clinical Session	Practical Implementation of New Therapies (CME certified) Tom Crawford, MD, Johns Hopkins Medicine (Chair) Nancy Kuntz, MD, Northwestern Feinberg School of Medicine (Chair) Jinsy Andrews, MD, MSc, Columbia University Katherine Mathews, MD, University of Iowa Tahseen Mozaffar, MD, University of California Irvine Gil Wolfe, MD, University at Buffalo Jacobs School of Medicine and Biomedical Sciences	Regency P
	Scientific Session	Gene-Targeting Therapies 2 Melissa Spencer, PhD, UCLA (Chair) Gene Yeo, PhD, MBA, University of California San Diego (Chair) Manuela Corti, PT, PhD, University of Florida Charles Gersbach, PhD, Duke University Dwi Kemaladewi, PhD, Children's Hospital of Pittsburgh Afrooz Rashnonejad, PhD, Nationwide Children's Hospital	Regency U
6:00–8:00 PM	Reception	Networking & Poster Reception (odd numbers)	Exhibit Hall

Continuing Medical And Nursing Education

This activity has been planned and implemented in accordance with the accreditation requirements and policies of the Accreditation Council for Continuing Medical Education (ACCME) through the joint providership of Physicians' Education Resource®, LLC and the Muscular Dystrophy Association (MDA). Physicians' Education Resource®, LLC is accredited by the ACCME to provide continuing medical education for physicians.

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6:30–7:45 AM	Industry Forum Breakfast	Presented by PTC Therapeutics	Orlando L-M
8:00–10:00 AM	Clinical Session	What's New in Neuromuscular Diseases (CME certified) Jim Dowling, MD, PhD, The Hospital for Sick Children (Chair) Carsten Bonnemann, MD, National Institutes of Health (Chair) David Beeson, PhD, MA, Oxford University Paula Clemens, MD, University of Pittsburgh Reghan Foley, MD, National Institutes of Health Nicholas Johnson, MD, Virginia Commonwealth University	Regency T
	Clinical Session	Cognitive & Behavioral Aspects of Neuromuscular Diseases (CME certified) John Day, MD, PhD, Stanford University (Chair) Brad Boeve, MD, Mayo Clinic M. Chiara Manzini, PhD, George Washington University Francesco Muntoni, MD, University College London	Regency U
	Scientific Session	Inflammation, Immune Mechanisms, & Therapeutic Approaches Kanneboyina Nagaraju, DVM, PhD, Binghamton University (Chair) Kathrin Meyer, PhD, The Research Institute at Nationwide Children's Hospital (Chair) Alyson Fiorillo, PhD, Children's National Medical Center Henry Kaminski, MD, George Washington University Basil Petrof, MD, McGill University Emiliano Trias, PhD, Institut Pasteur de Montevideo	Regency P
	Scientific Session	Nuclear Membrane Lori Wallrath, PhD, University of Iowa (Chair) Jeff Rothstein, MD, PhD, Johns Hopkins Medicine (Chair) Katherine Bossone, University of the Sciences Peter Meinke, PhD, Friedrich Baur Institute, University Hospital, LMU Munich Stephen Moore, Barrow Neurological Institute	Regency O
Exhibit Hall Open 10:00 AM–8:00 PM			
10:00–10:30 AM	Break		Regency Foyer
10:30 AM–12:30 PM	Clinical Session	Care Across the Lifespan (CME certified) John Brandsema, MD, Children's Hospital of Philadelphia (Chair) Diane Murrell, LCSW, Texas Children's Hospital (Chair) Nancy Glass, MD, MBA, FAAP, Texas Children's Hospital Catherine Lomen-Hoerth, MD, PhD, University of California San Francisco Sarah Stoney, MSW, LSW, The Children's Hospital of Philadelphia James Wymer, MD, PhD, University of Florida	Regency T
	Clinical Session	Technology in Healthcare James Berry, MD, MPH, Massachusetts General Hospital (Chair) Josh Cosman, PhD, Biogen Teresa Arroyo-Gallego, nQ Medical Leigh Hochberg, MD, PhD, Massachusetts General Hospital Balasundaram Kadirvelu, MD, Imperial College London	Regency U
	Scientific Session	Regeneration & Repair Addolorata Pisconti, PhD, Stony Brook University (Chair) Joseph Metzger, PhD, University of Minnesota (Chair) Jason Doles, PhD, Mayo Clinic School of Medicine Katherine Marshall, Johns Hopkins School of Medicine Jeffery Molkenin, PhD, Cincinnati Children's Hospital Medical Center Molly Naylor, University of Michigan	Regency P
	Scientific Session	DNA & RNA Repeat Diseases Don Cleveland, PhD, University of California San Diego School of Medicine (Chair) Joel Chamberlain, PhD, University of Washington School of Medicine (Chair) Gary Bassell, PhD, Emory University School of Medicine Andrew Lieberman, MD, PhD, University of Michigan Marek Napierala, PhD, University of Alabama at Birmingham Katherine Williams, MS, University of California Irvine	Regency O
12:30–1:45 PM	Industry Forum Lunch	Presented by Biogen	Regency Q
	Industry Forum Lunch	Presented by Santhera Pharmaceuticals	Florida B-C
	Industry Forum Lunch	Presented by Sarepta Therapeutics	Orlando L-M
2:00–6:00 PM <i>30 minute break will occur at 3:30 PM</i>	Clinical Session	Clinical Trials (CME certified) John Day, MD, PhD, Stanford University James F. Howard, Jr, MD, University of North Carolina Richard Finkel, MD, Nemours Children's Hospital Michio Hirano, MD, Columbia University Irving Medical Center Eric Hoffman, PhD, Binghamton University Priya Kishani, MD, MBBS, Duke University Medical Center Lutz Mueller, PhD, Roche Sabrina Paganoni, MD, PhD, Massachusetts General Hospital Jeffrey Statland, MD, University of Kansas Medical Center	Regency R-S
6:00–8:00 PM	Reception	Networking & Poster Reception (even numbers)	Exhibit Hall

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8:00–10:00 AM	Clinical Session	Outcome Measures for Neuromuscular Diseases (CME certified) Craig McDonald, MD, University of California, Davis (Chair) Chad Heatwole, MD, MS-CI, University of Rochester (Chair) Lindsay Alfano, DPT, Nationwide Children's Hospital John Day, MD, PhD, Stanford University Samantha LoRusso, MD, The Ohio State University Wexner Medical Center Jeremy Shefner, MD, PhD, Barrow Neurological Institute	Regency T
	Clinical Session	Cardiac & Pulmonary Involvement in Neuromuscular Disease (CME certified) Elizabeth McNally, MD, PhD, Northwestern University Feinberg School of Medicine (Chair) David Birnkrant, MD, MetroHealth Medical Center (Chair) Kenneth Berger, MD, New York University School of Medicine Harold Farber, MD, MSPH, Texas Children's Hospital and Baylor College of Medicine Kan Hor, MD, Nationwide Children's Hospital Jaclyn Omura, MD, Seattle Children's Hospital, University of Washington School of Medicine	Regency U
	Scientific Session	Imaging & Biomarkers Katharine Nicholson, MD, Sean M. Healey & AMG Center for ALS at Mass General (Chair) Michael Shy, MD, University of Iowa (Chair) Suma Babu, MBBS, MPH, Sean M. Healey & AMG Center for ALS at Mass General William Evans, PhD, University of California, Berkeley Christopher Heier, PhD, Children's National Medical Center Thurman Wheeler, MD, Massachusetts General Hospital	Regency P
	Scientific Session	Cell-Based Modeling & Screening Paul August, PhD, ICAGEN (Chair) Fen-Biao Gao, PhD, University of Massachusetts Medical School (Chair) Congshan Sun, PhD, Johns Hopkins Medicine Justin Ichida, PhD, University of Southern California Deok-Ho Kim, PhD, University of Washington Keryn Woodman, PhD, Yale University	Regency O
Exhibit Hall Open 9:00 AM–12:30 PM			
10:00–10:30 AM	Break		Regency Foyer
10:30 AM–12:30 PM	Clinical Session	Physical, Occupational, & Speech Therapy (CME certified) Laurie Sterling, MS, CCC-SLP, BCS-S, Texas Children's Hospital (Chair) Roxanna Bendixen, PhD, OTR/L, University of Pittsburgh Jennifer Chapin, MS, CCC-SLP, University of Florida Melissa Fox, PT, DPT, University of Virginia Health System Linda Lowes, PT, PhD, Nationwide Children's Hospital	Regency T
	Clinical Session	Newborn Screening R. Rodney Howell, MD, Miller School of Medicine, University of Miami (Chair) Barry Byrne, MD, PhD, University of Florida (Chair) John Crowley, Amicus Therapeutics Richard Finkel, MD, Nemours Children's Hospital Kevin Flanigan, MD, Nationwide Children's Hospital Peter Kyriacopoulos, Association of Public Health Laboratories Michael Watson, PhD, American College of Medical Genetics & Genomics Brittany Hernandez, Muscular Dystrophy Association Kristin Stephenson, MHA, JD, Muscular Dystrophy Association	Regency U
	Scientific Session	Protein Homeostasis Wilfried Rossoll, PhD, Mayo Clinic (Chair) Conrad (Chris) Weihl, MD, PhD, Washington University (Chair) Ming Guo, MD, PhD, UCLA School of Medicine Vandana Gupta, PhD, Brigham and Women's Hospital and Harvard Medical School Alysia Mortimer, PhD, Illinois State University Brad Olwin, University of Colorado Boulder	Regency P
	Scientific Session	In Vivo Models & Experimental Therapeutics Kitipong Uaesoontrachoon, PhD, Dalhousie University/AGADA Biosciences (Chair) Laurent Bogdanik, PhD, The Jackson Laboratory (Chair) Virginia Kimonis, MD, University of California Irvine Matthias Lambert, PhD, Boston Children's Hospital and Harvard Medical School Lyndsay Murray, PhD, University of Edinburgh Isabelle Richard, PhD, Genethon	Regency O
12:30 PM	MEETING ADJOURNED		

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