



Conference Agenda

ALL CONFERENCE SESSION TIMES LISTED ARE IN CENTRAL TIME

Saturday, March 18, 2023

Saturday, March 18, 2023		
12 – 6 PM	Registration Open for Care Center Directors & Large Exhibit Booth Sponsors	

Sunday, March 19, 2023

Sunday, March 19, 2023		
7 AM – 8 PM	Registration Open	
9 AM – 3 PM	Care Center Directors Meeting	
1 - 5 PM	MDA Trainee Networking Session	
6 - 8 PM	Welcome Reception in Exhibit Hall	Exhibit Hall

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Monday, March 20, 2023

7 AM – 6 PM	Registration Open	
7 - 8 AM	Industry Forum Breakfast presented by Biogen	
	Industry Forum Breakfast presented by PTC	
	Industry Forum Breakfast presented by Reata	
8:30 – 9:30 AM	Opening and Keynote Address Donald S. Wood, PhD President & CEO, MDA Peter Marks, MD, PhD Director of the Center for Biologics Evaluation and Research (CBER) US Food and Drug Administration (FDA)	Trinity Ballroom
9:30 – 9:55 AM	Networking Break	Exhibit Hall
10 AM – 12 PM	General Session	Trinity Ballroom
9:30 AM – 1:30 PM	Exhibit Hall and Poster Sessions Open	Exhibit Hall
12 – 1:30 PM	Industry Forum Lunch Presented by Genentech	
	Industry Forum Lunch Presented by Pfizer	
	Industry Forum Lunch Presented by Sanofi	
	Industry Forum Lunch Presented by Sarepta	
2- 3:30 PM	Ultra-Rare Track	
	LGMD Peter Kang, MD (Chair) Peter Andersen, PhD Kathryn Bryant Douglas Sproule, MD, MSc	
	CMT Michael Shy, MD (Chair) Brett McCray, MD, PhD	
	Myopathies/CMD Alan Beggs, PhD (Chair) Frances Evesson, PhD Michio Inoue, MD, PhD	
	Technology Track	
	Translation of Gene Editing Technologies Melissa Spencer, PhD (Chair) Stephen Cannon, PhD Dongsheng Duan, PhD Dwi Kemaladewi, PhD Eric Olson, MD Afrooz Rashnonejad, MSc, PhD Courtney Young, PhD	

Monday, March 20, 2023 cont.

	DMD Beyond Muscle: A Holistic Approach Track*	
2- 3:30 PM	Cognitive & Behavior Mathula Thangarajh, MD (Chair) V Preethish Kumar, MBBS, MRCP, MRCPE, PhD Alisha Pollastri, PhD Natalie Truba, PhD Bone Health - Where have we been, where are we now, and where are we going? Meilan Rutter, MD (Chair) Nat Nasomyont, MD, MS David Weber, MD, MSCE	
	NMD Care Delivery: Care Throughout the Patient Journey Track*	
	Diagnostic Testing Options Kevin Flanigan, MD (Chair) Diana Bharucha-Goebel, MD Katherine Mathews, MD Steven Moore, MD, PhD	
3:30 – 4:30 PM	Exhibit Hall and Poster Sessions Open	
3:30 – 3:55 PM	Networking Break sponsored by Entrada Therapeutics	Exhibit Hall
	Ultra-Rare Track	
	Mitochondrial Michio Hirano (Chair) Anthony Abbruscato, PharmD, BCPS Alex Dorenbaum, MD Fabian Somers MDA Kickstart Program for Ultra-Rare Gene Therapy Development Sharon Hesterlee, PhD (Chair) Maryna Kolochavina, PharmD, PhD, PMP Ricardo Maseli, MD Panelists: Alan Beggs, PhD; Michio Hirano, MD, PhD; Peter Kang, MD & Michael Shy, MD, PhD	
	Technology Track	
4 - 6 PM	Non-Viral Delivery Approaches for Genetic Therapies Stanley Froehner (Chair) Mahasweta Girgenrath, PhD Thomas Roberts, PhD Nick Whitehead, PhD Using iPS Cells to Model Neuromuscular Disease Rita Perlingeiro (Chair) Penney Gilbert, PhD David Mack, PhD Francesco Saverio Tedesco, PhD	
	DMD Beyond Muscle: A Holistic Approach Track*	
	Digestive Health Impacts from Top to Bottom David Brumbaugh, MD (Chair) Ajay Kaul, MD Katlyn McGrattan, PhD Laura Watne, MS, RD, CSP	

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Monday, March 20, 2023 cont.

	NMD Care Delivery: Care Throughout the Patient Journey Track*	
	Supporting the Patient Throughout Their Journey Ericka P. Greene, MD, MACM, FAAN (Chair) Rebecca Axline, LCSW-S, CSM, APHSW-C Keelie Denson, MD Timothy Lotze, MD	
6 – 8 PM	Poster & Networking Reception	Exhibit Hall

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Tuesday, March 21, 2023

7 AM – 6 PM	Registration Open	
7 - 8 AM	Industry Forum Breakfast presented by Amylyx	
	Industry Forum Breakfast presented by Sanofi	
	Industry Forum Breakfast presented by Sarepta	
8:30 – 11:45 AM	Insights in Research Investor Summit	
8:30 – 10:30 AM	ALS Track	
	ALS Collaborations: A Continuum from Research to Care Melanie Leitner, PhD (Chair) Laura Dalle Pазze Kuldip Dave, PhD Clare Durrett Elisabeth Kilroy, PhD Manish Raisinghani, MBBS, PhD Darleen Sawicki, MSN, NP-BC Fernando Viera, MD	
	Digital Outcomes & Big Data Track	
	Remote Outcomes and Decentralized Studies Jeffrey Statland, MD (Chair) Amir Lahav, ScD Christine McSherry, RN BSN Jeremy Shefner, MD, PhD Paul Strijbos, PhD Ashkan Vaziri, PhD	
	Practical Considerations in Gene Therapy Track*	
	Updates on Gene Therapy in NMD: Current & Emerging Therapies Natalie Goedeker, MSN, CPNP (Chair) Emma Cialfoni, MD, FAAN Richard Finkel, MD Kevin Flanigan, MD Kathryn Wagner, MD, PhD	
	NMD Care Delivery: Care Throughout the Patient Journey Track*	
	Registry Engagement Successes Daragh Heitzman, MD, FAAN (Chair) Molly Colvin, PhD, ABPP R. Bradley Troxler, MD Paul Mehta, MD Rupa Nallamothu	
Leveraging Providers in the Community Craig McDonald, MD (Chair) Molly Colvin, PhD Tina Duong, PT, PhD Erik Henricson, PhD, MPH		
10:30 AM – 1:30 PM	Exhibit Hall and Poster Sessions Open	Exhibit Hall
10:30 – 10:55 AM	Networking Break sponsored by UCB	Exhibit Hall

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Tuesday, March 21, 2023 cont.

11 AM – 12 PM	ALS Track	
	Advances in the Understanding in ALS Stanley Appel, MD (Chair) Clotilde Lagier-Tourenne, MD, PhD Clive Svendsen, PhD	
	Digital Outcomes & Big Data Track	
	Large-Scale Data Approaches to NMD Research Stephan Zuchner, PhD Monkol Lek, PhD Elisabeth Kilroy, PhD Anastasia Wise, PhD	
11 AM – 12 PM	Practical Considerations in Gene Therapy Track*	
	Future of Gene Therapy in NMD: Considerations for Newborn Screening & Clinical Trial Design Natalie Goedecker, MSN, CPNP (Chair) Don Bailey, PhD Katherine Mathews, MD	
	NMD Care Delivery: Care Throughout the Patient Journey Track*	
	Holistic Approach to Transitioning into Adulthood Anne Connolly, MD (Chair) Richard Shell, MD Jamie Twanow, MD	
12 – 1:30 PM	Industry Forum Lunch Presented by Biogen	
	Industry Forum Lunch Presented by Edgewise	
	Industry Forum Lunch Presented by Fulcrum	
	Industry Forum Lunch Presented by NS Pharma	
2 – 5:30 PM	Insights in Research Investor Summit	
2 – 4 PM	ALS Track	
	Gene Therapy in ALS Matthew Harms, MD (Chair) Jennifer Morganroth, MD, MDBA Jennifer Roggenbuck, MS, LGC	
	Digital Outcomes & Big Data Track	
	Lab to Life: CMT Michael Shy, MD (Chair) Joshua Burns, BAppSC, PhD, FFPM RCPS Bruce Conklin, MD Katy Eichinger, PhD, DPT John Svaren, PhD	
	Practical Considerations in Gene Therapy Track*	
	Practical Management of Gene Therapy: Procurement & Administration Natalie Goedecker, MSN, CPNP (Chair) Brent Furbee Kody Lane Graves Crystal Proud, MD	

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Tuesday, March 21, 2023 cont.		
2 – 4 PM	NMD Care Delivery: Care Throughout the Patient Journey Track*	
	Therapy Interventions – Modify Therapy Approaches with Evolving Market Landscape Tina Duong, PT, PhD (Chair) Megan Iammarino, PT, DPT Donovan Lott, PT, PhD, CSCS Jenna Lammers, MSR/PT, CNT, PCS Leslie Nelson, PT, PhD Jacqueline Montes, PT, EdD, NCS Melissa McIntyre, DPT	
3:30 – 4:30 PM	Exhibit Hall and Poster Sessions Open	Exhibit Hall
4 – 4:25 PM	Networking Break sponsored by PepGen	Exhibit Hall
4:30 – 6:00 PM	ALS	
	Industry Updates Suma Babu, MD, MPH, MBBS (Chair) Jinsy Andrews, MD, MSc Melanie Quintana, PhD	
	Digital Outcomes & Big Data	
	Lab to Life: Myositis Tahseen Mozaffar, MD (Chair) Suur Bilicier, MD Teerin Liewluck, MD Thomas Llyod, MD Eleni Tiniakou, MD	
	Practical Considerations in Gene Therapy*	
	Clinical Challenges in the Gene Therapy Era: Roundtable Discussion of Difficult Cases Natalie Goedecker, MSN, CPNP (Chair) Richard Finkel, MD Jerry Mendell, MD Crystal Proud, MD	
	NMD Care Delivery: Care Throughout the Patient Journey*	
	End of Life: Care and Considerations Ambereen Mehta, MD, MPH (Chair) Kirstin Springmeyer, M Div., MS, BCC-PCHAC Lauren Treat, MD	
6 – 8 PM	Poster & Networking Reception	Exhibit Hall

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Conference Agenda

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Wednesday, March 22, 2023

7 AM – 1 PM Registration Open

New, Novel, and Noteworthy: NMD Highlights

We are thrilled to have had an overwhelming number of incredible abstract submissions as we reconvene in 2023. To that end, we have redesigned our Clinical Trail Presentations to include a wider range of topics which also allows for even more live presentations selected directly from the abstract submissions.

This is a final day not to be missed!

8 AM – 4:00 PM Oral Abstract Presentations