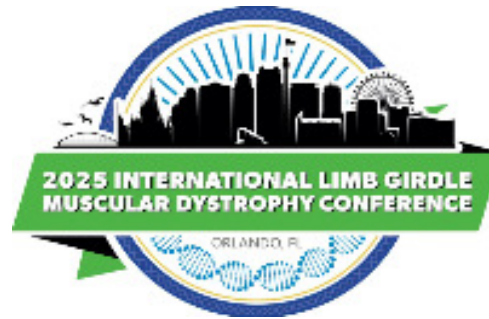


2025 International Limb-Girdle Muscular Dystrophy Conference

JW Marriott Grande Lakes - Orlando, FL

The Conference Agenda is Subject to Change.



Sponsored by



Friday, July 18, 2025

3:00 – 6:00 PM	GENERAL REGISTRATION - CHECK IN	Grande Registration Desk
4:00 – 6:15 PM	Exhibitor Setup	Palazzo Foyer
6:30 – 8:00 PM	WELCOME RECEPTION & MIXER	Valencia / Terrace

Saturday, July 19, 2025

7:00 – 8:00 AM	Late Exhibitor Setup		Palazzo Foyer
8:00 – 11:00 AM	GENERAL REGISTRATION - LATE CHECK IN		Grande Registration Desk
8:00 – 9:00 AM	BREAKFAST SNACKS		Palazzo Foyer
9:30 – 10:30 AM	Session 1:	WELCOME, OVERVIEW, AND CLINICAL CARE: Conference Welcome • Kathryn Bryant Knudson, President, The Speak Foundation Patient Advocacy Organization Introductions • Kathryn Bryant Knudson, President, The Speak Foundation LGMDs: What should patients know to get productive clinical care? • Matthew P. Wicklund, MD, University of Texas Developing Formal Standards of Care for LGMDs • Volker Straub, MD, PhD, University of Newcastle, UK • Meredith James, PhD, University of Newcastle, UK	Palazzo Ballroom
10:30 – 10:45 AM	BREAK		

Saturday, July 19, 2025 - continued

10:45 – 11:45 AM	Session 2: Breakout	Understanding Diagnosis and Genetic Test Reports	Palazzo Ballroom
		• Lee Kugelmann, NMSc, CGC, University of Florida	
		• Conrad (Chris) Weihl, MD PhD, Washington University School of Medicine	
		• Peter Kang, MD, University of Minnesota	
		• Brad Williams and Sara Yemm	
		Building Community Connections: Young Adulthood with LGMD - Ages 18-25 (no parents)	Palazzo A
		• Cerys Davage and Serena Desiderio	
		Raising a Child who has LGMD - Meetup for parents and family members	Palazzo B
		• Rachel DeConti, Faran Day, and Aleksandra Leijenhorst Le Belle	
11:45 AM - 12:00 PM	BREAK		
12:00 – 12:50 PM	LUNCH	<i>Sarepta Therapeutics Symposium</i>	Palazzo Ballroom
		• <i>Sponsored by Sarepta Therapeutics</i>	
12:50 – 1:00 PM	BREAK		
1:00 – 1:45 PM	Session 3: Breakout	How does an idea become a drug? Moving from the lab to clinical trials	Palazzo Ballroom
		• Kevin Campbell, PhD, University of Iowa	
		• Sharon Hesterlee, PhD, Muscular Dystrophy Association	
		• Nicholas E. Johnson, MD, Virginia Commonwealth University	
		Genetic Counselors: Q&A	Palazzo A
		• Lee Kugelmann, NMSc, CGC, University of Florida	
		• Brianna N. Gross, MS, Children's Hospital of Philadelphia	
		• Ashley Cannon, MS, CGC, PhD, University of Alabama	
		Building Community Connections: Adolescents - Meetup for Ages 12-17 (no parents)	Palazzo B
		• Peter Frewing, Brooklyn Garza, and Sammy Brazzo	
		Living with Dominant LGMDs and Treatment Approaches	Palazzo C
		• Jeffrey Statland, MD, Kansas University Medical Center	
		• Victoria Nedza, Patient	
• Natasha Lowery, LGMD-1D DNAJB6 Foundation			

Saturday, July 19, 2025 - continued

1:45 – 2:00 PM	BREAK		
2:00 – 2:45 PM	Session 4: Breakout	Current Drug Development Pathways for LGMDs	Palazzo Ballroom
		• Barry Byrne, MD, PhD, University of Florida	
		• Matthew P. Wicklund, MD, University of Texas	
		Physical Therapy and Rehab: Areas to watch with LGMD	Palazzo A
		• Lindsay Alfano, PT, DPT, PCS, Nationwide Children's Hospital	
		• Vovanti Jones, MD, University of Missouri	
2:45 – 3:00 PM	BREAK		
3:00 – 3:45 PM	Session 5: Breakout	Ask the Expert: Q&A	Palazzo Ballroom
		• Lindsay Alfano, PT, DPT, PCS, Nationwide Children's Hospital	
		• Vovanti Jones, MD, University of Missouri	
		• Volker Straub, MD, PhD, University of Newcastle, UK	
		• Matthew P. Wicklund, MD, University of Texas	
		• Peter Kang, MD, University of Minnesota	
		• Barry Byrne, MD, PhD, University of Florida	
		• Bjarne Udd, MD, PhD, Tampere Neuromuscular Center	
		• Conrad (Chris) Wehl, MD PhD, Washington University School of Medicine	
		Exercise, Weight Management & Nutrition in LGMD	Palazzo A
		• Tina Duong MPT, PhD, Stanford Medicine	
		• Tanja Taivassalo, PhD, University of Florida	Palazzo B
		Navigating Mental Health with Changing Disability	
		• Melissa Grove MS, LPC	
3:45 – 4:00 PM	GROUP PHOTO		
	Palazzo Ballroom		
4:00 – 5:30 PM	BREAK		
5:30 – 6:30 PM	COCKTAIL RECEPTION, AWARDS CEREMONY & SCIENTIFIC POSTER SESSION		Palazzo Ballroom

Sunday, July 20, 2025

8:00 – 9:00 AM	BREAKFAST SNACKS		Palazzo Foyer
9:30 – 10:45 AM	Session 6:	UPDATES FROM CLINICAL TRIALS:	Palazzo Ballroom
		ML Bio Solutions:	
		• Douglas Sproule, MD, MSc, Chief Medical Officer	
		Sarepta Therapeutics:	
		• Louise Rodino-Klapac, PhD, Head of R&D, Chief Scientific Officer	
		AskBio:	
		• Samuel Hopkins, PhD, Senior Vice President of Therapeutics	
		Edgewise Therapeutics:	
		• Joanne M. Donovan, MD, PhD, Chief Medical Officer	
Atamyo Therapeutics:			
• Sophie Olivier, MD, Chief Medical Officer			
10:45 – 11:00 AM	BREAK		
11:00 – 11:50 AM	Session 7: Breakout	Cardiac & Respiratory Management	Palazzo Ballroom
		• Frank Raucci, MD, Virginia Commonwealth University (Cardiac)	
		• Beth McNally, MD, PhD, Northwestern University (Cardiac)	
		• Megan Lowery, MD, University of Florida (Pulmonary)	
		• Amy Fulford, RT, Millennium Respiratory Services	
		Rare Recessive LGMD Subtypes	Palazzo A
		• Christina Pacak, PhD. University of Minnesota	
		• Monkol Lek, PhD, Yale School of Medicine	
		• Bjarne Udd, MD, PhD, Tampere Neuromuscular Center	
		Natural History Studies - What to expect & Why should I participate?	Palazzo B
		Moderator: Carla Zingariello, DO Montefiore/Albert Einstein College of Medicine	
		• Ruby Langeslay, MPH, Virginia Commonwealth University	
		• Kevin Warf, BS, Nationwide Children's Hospital	
		• Sujethra (Suji) Vasu, MD, Caregiver	
		• Dan Pope, Patient	
		Life Hacks for Successful Living with LGMD	Palazzo C
		• Tips & Tricks: Carol Abraham & Victoria Nedza	
• Driving Independence: Cerys Davage & John Faver			

Sunday, July 20, 2025 - continued

11:50 AM - 12:00 PM	BREAK		
12:00 – 12:50 PM	LUNCH	ML BIO UPDATES BBP-418 <i>• Sponsored by ML BIO</i>	Palazzo Ballroom
12:50 - 1:00 PM	BREAK		
1:00 – 1:35 PM	Session 8:	LGMD ADVOCACY OPPORTUNITIES Laying Out the Upcoming LGMD Advocacy: Lessons from the Rare Disease Community <i>• Annie Kennedy, Chief of Policy, Advocacy, and Patient Engagement, Everylife Foundation</i> Drug Approval and Advocacy Readiness for the LGMDs (GROUP PANEL) <i>• Annie Kennedy & Kathryn Bryant Knudson</i> Call to Action 2025 LGMD Day on the Hill <i>• Kathryn Bryant Knudson, President, The Speak Foundation</i>	Palazzo Ballroom
1:35 - 1:45 PM	BREAK		
1:45 – 2:45 PM	Session 9:	EMERGING TREATMENTS Stem Cells + Gene Editing <i>• Simone Spuler, MD, Charité - University Medicine</i> Repurposing Treatments <i>• Mohan Viswanathan, PhD, Massachusetts Institute of Technology</i> Development of Novel Gene Therapy for FKRP Disorders <i>• Jeffrey Chamberlain, PhD, University of Washington</i> Prime Editing <i>• Scot A. Wolfe, PhD, UMass Chan Medical School</i>	Palazzo Ballroom
2:45 – 2:50 PM	Closing Remarks	"We cannot do this without everyone" <i>• Kathryn Bryant Knudson, Brad Williams, and Carol Abraham</i>	Palazzo Ballroom
2:50 PM	ADJOURN		

The International Limb-Girdle Muscular Dystrophy Conference is a project of the Speak Foundation, a 501 (c)(3) tax-exempt public charity since 2008. Learn more at TheSpeakFoundation.com