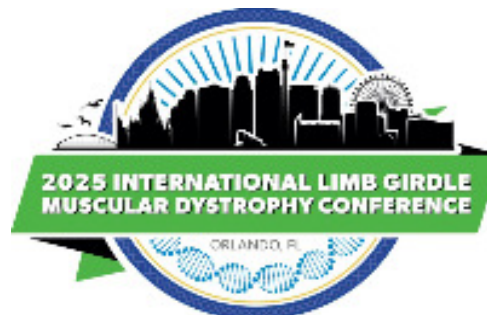


2025 International Limb-Girdle Muscular Dystrophy Conference

JW Marriott Grande Lakes - Orlando, FL

The Conference Agenda is Subject to Change.



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

8:00 – 11:00 AM	GENERAL REGISTRATION - LATE CHECK IN		Grande Registration Desk
8:15 – 9:00 AM	BREAKFAST SNACKS		Palazzo Foyer
9:30 – 10:30 AM	Session 1:	WELCOME, OVERVIEW, AND CLINICAL CARE:	Palazzo Ballroom
		Conference Welcome	
		• Kathryn Bryant Knudson, President, The Speak Foundation	
		LGMD Advocacy and Organization Recognition	
		• 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	
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 Kugelman, 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		
		Life Hacks for Successful Living with LGMD		Palazzo A
		• Tips & Tricks: Carol Abraham and Tori Nedza		
		• Driving Independence: Cerys Davage & John Faver		
		Raising a Child who has LGMD - Meetup for parents and family members		Palazzo C
• Rachel DeConti, Faran Day, and Aleksandra Leijenhorst Le Belle				
11:45 AM - 12:00 PM	BREAK			
12:00 – 12:50 PM	LUNCH	Sarepta Therapeutics Symposium		Palazzo Ballroom
		• Sponsored by Sarepta Therapeutics		
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 Kugelman, NMSc, CGC, University of Florida		
		• Brianna N. Gross, MS, LCGC, Children's Hospital of Philadelphia		
		Living with Dominant LGMDs and Treatment Approaches		Palazzo B
		• Jeffrey Statland, MD, Kansas University Medical Center		
		• Victoria Nedza, Patient		
		• Natasha Lowery, LGMD-1D DNAJB6 Foundation		
Building Community Connections: Meetup for Adolescents - Ages 12-17 (no parents)		Palazzo C		
• Peter Frewing, Brooklyn Garza, and Sammy Brazzo				

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	
Building Community Connections: Meetup for Young Adults - Ages 18-25 (no parents)	Palazzo C		
• Cerys Davage and Serena Desiderio			
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	
		• Barry Byrne, MD, PhD, University of Florida	
		• Vovanti Jones, MD, University of Missouri	
		• Peter Kang, MD, University of Minnesota	
		• Volker Straub, MD, PhD, University of Newcastle	
		• Bjarne Udd, MD, PhD, Tampere Neuromuscular Center	
		• Matthew P. Wicklund, MD, University of Texas	
		Exercise, Weight Management & Nutrition in LGMD	Palazzo A
		• Tina Duong MPT, PhD, Stanford Medicine	
Navigating Mental Health with Changing Disability	Palazzo B		
• 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

8:15 – 9:00 AM	BREAKFAST SNACKS		Palazzo Foyer
9:30 – 10:45 AM	Session 6:	<p>UPDATES FROM CLINICAL TRIALS:</p> <p>ML Bio Solutions:</p> <ul style="list-style-type: none"> • Douglas Sproule, MD, MSc, Chief Medical Officer <p>Sarepta Therapeutics:</p> <ul style="list-style-type: none"> • Louise Rodino-Klapac, PhD, Head of R&D, Chief Scientific Officer <p>AskBio:</p> <ul style="list-style-type: none"> • Samuel Hopkins, PhD, Senior Vice President of Therapeutics <p>Edgewise Therapeutics:</p> <ul style="list-style-type: none"> • Joanne M. Donovan, MD, PhD, Chief Medical Officer <p>Atamy Therapeutics:</p> <ul style="list-style-type: none"> • Sophie Olivier, MD, Chief Medical Officer 	Palazzo Ballroom
10:45 – 11:00 AM	BREAK		
11:00 – 11:50 AM	Session 7: Breakout	<p>Cardiac & Respiratory Management</p> <ul style="list-style-type: none"> • 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 	Palazzo Ballroom
		<p>Rare Recessive LGMD Subtypes</p> <ul style="list-style-type: none"> • Christina Pacak, PhD. University of Minnesota • Monkol Lek, PhD, Yale School of Medicine • Bjarne Udd, MD, PhD, Tampere Neuromuscular Center 	Palazzo A
		<p>Natural History Studies - What to expect & Why should I participate?</p> <p>Moderator: Carla Zingariello, DO Montefiore/Albert Einstein College of Medicine</p> <ul style="list-style-type: none"> • Ruby Langeslay, MPH, Virginia Commonwealth University • Dan Pope, Patient • Sujethra (Suji) Vasu, MD, Caregiver • Kevin Warf, BS, Nationwide Children's Hospital 	Palazzo B
11:50 AM - 12:00 PM	BREAK		
12:00 – 12:50 PM	LUNCH	<p>ML BIO UPDATES BBP-418</p> <ul style="list-style-type: none"> • Sponsored by ML BIO 	Palazzo Ballroom

Sunday, July 20, 2025 - continued

12:50 - 1:00 PM	BREAK		
1:00 – 1:35 PM	Session 8:	<div>LGMD ADVOCACY OPPORTUNITIES</div> <div>Rare Disease and Patient Roundtable Discussion with Amy Comstock Rick from the FDA</div> <div>• Amy Comstock Rick, J.D., CDER's Associate Director for Rare Disease Strategy and the Director of Strategic Coalitions for FDA's Rare Disease Innovation Hub</div> <div>• Kathryn Bryant Knudson, Brad Williams, and Carol Abraham</div> <div>Laying Out the Upcoming LGMD Advocacy: Lessons from the Rare Disease Community</div> <div>• Annie Kennedy, Chief of Policy, Advocacy, and Patient Engagement, Everylife Foundation</div> <div>Call to Action 2025 LGMD Day on the Hill</div> <div>• Kathryn Bryant Knudson, President, The Speak Foundation</div>	Palazzo Ballroom
1:35 - 1:45 PM	BREAK		
1:45 – 2:45 PM	Session 9:	<div>EMERGING TREATMENTS</div> <div>Stem Cells + Gene Editing</div> <div>• Simone Spuler, MD, Charité - University Medicine</div> <div>Repurposing Treatments</div> <div>• Mohan Viswanathan, PhD, Massachusetts Institute of Technology</div> <div>Development of Novel Gene Therapy for FKRPs Disorders</div> <div>• Jeffrey Chamberlain, PhD, University of Washington</div> <div>Prime Editing</div> <div>• Scot A. Wolfe, PhD, UMass Chan Medical School</div>	Palazzo Ballroom
2:45 – 2:50 PM	Closing Remarks	<div>"We cannot do this without everyone"</div> <div>• Kathryn Bryant Knudson, Brad Williams, and Carol Abraham</div>	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

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