

2019 National Limb Girdle Muscular Dystrophy Conference

Hyatt Regency - Chicago, Illinois

The Conference Agenda is Subject to Change



Sponsored by SAREPTA THERAPEUTICS

FRIDAY, AUGUST 30, 2019

2:00 – 3:00 PM	EXHIBITOR REGISTRATION <i>(Exhibit tables must be set up by 5:30 pm in Regency Ballroom D)</i>	Plaza Ballroom Foyer
3:00 – 5:30 PM	GENERAL REGISTRATION - CHECK IN	Plaza Ballroom Foyer
7:00 – 9:00 PM	WELCOME RECEPTION	Plaza Ballroom

SATURDAY, AUGUST 31, 2019

8:45 – 9:45 AM	Exhibitor Room Designated Time • Breakfast Snacks - Sponsored by Camron'sCure	Regency Ballroom D
9:45 – 10:00 AM	BREAK	
10:00 – 11:15 AM	Plenary Session I: WELCOME AND OVERVIEW OF LGMD: Conference Welcome • Kathryn Bryant, National Director, Speak Foundation Overview of LGMD: Symptoms, Genetics, Inheritance, and Incidence • Matthew P. Wicklund, MD, University of Colorado, Denver The GRASP LGMD Consortium • Nicholas E. Johnson, MD, Virginia Commonwealth University	Regency Ballroom A-B-C
11:15 – 11:30 AM	BREAK	
11:30 AM – 12:15 PM	Genetic Diagnosis: Why and How • Peter B. Kang, MD, University of Florida, Gainesville • Conrad C. Wehl, MD, PhD, Washington University, St. Louis • Monkol Lek, PhD, Yale University • Rich Dineen, MS, CGC, Rush University Medical Center	Regency Ballroom A-B-C
	Breakout Sessions 1: Respiratory Management in LGMD • Matthew P. Wicklund, MD, University of Colorado, Denver • Mary Ellen Downey, CRT RCP, Millennium Respiratory Services	Acapulco
	Young Adults with LGMD: School, Career, Life • Chris Anselmo, Nicole Henry, Andrea Lane	Toronto

SATURDAY, AUGUST 31, 2019 – CONTINUED

12:15 – 1:50 PM	LUNCH	Exhibitor Room Designated Time <ul style="list-style-type: none"> • Lunch - Sponsored by AskBio 	Regency Ballroom D
1:50 – 2:40 PM	Plenary Session II:	DRUG DEVELOPMENT AND THE PATIENT'S ROLE: Understanding the Drug Development Paradigm <ul style="list-style-type: none"> • Raymond Huml, DVM, IQVIA Power and Purpose of Patient Registries <ul style="list-style-type: none"> • Sarah Emmons, Jain Foundation 	Regency Ballroom A-B-C
2:40 – 2:55 PM	BREAK		
2:55 – 3:45 PM	Plenary Session III:	PATIENT INVOLVEMENT IN CLINICAL TRIAL READINESS: Natural History Studies for Successful Clinical Trials <ul style="list-style-type: none"> • Volker Straub MD, University of Newcastle, UK Outcome Measures: What They Are and Why They Are Important <ul style="list-style-type: none"> • Meredith James, Physiotherapist, University of Newcastle, UK 	Regency Ballroom A-B-C
3:45 – 4:00 PM	BREAK		
4:00 – 4:45 PM	Breakout Sessions 2:	Ask the Experts Panel #1: LGMD Overview, Genetics, and Natural History <ul style="list-style-type: none"> • Raymond Huml, DVM, IQVIA • Meredith James, Physiotherapist, University of Newcastle, UK • Nicholas E. Johnson, MD, Virginia Commonwealth University • Katherine D. Mathews, MD, University of Iowa • Volker Straub, MD, University of Newcastle • Matthew P. Wicklund, MD, Univ of Colorado 	Regency Ballroom A-B-C
		Opportunities for Patient Involvement <ul style="list-style-type: none"> • Lianna R. Orlando, PhD, Muscular Dystrophy Association • Ralph Yaniz, LGMD Day on the Hill, Speak Foundation 	Acapulco
		Children with LGMD: Navigating Care Management, Social Opportunities and Family Health <ul style="list-style-type: none"> • Kelly Brazzo, CureLGMD2i Foundation • Scott Frewing, Kurt+Peter Foundation • Jessica Gerardo, Camron'sCure 	Toronto
4:45 – 5:45 PM		Exhibitor Room Designated Time	Regency Ballroom D

SATURDAY, AUGUST 31, 2019 – CONTINUED

5:45 – 7:00 PM	BREAK	
7:00 PM	BANQUET DINNER	<p>Introduction: The Speak Foundation Initiatives and Advocacy Organizations</p> <ul style="list-style-type: none"> • Kathryn Bryant, National Director, Speak Foundation <p>Keynote Address:</p> <p>“Lessons from the DMD Community for LGMD Advocacy”</p> <ul style="list-style-type: none"> • Pat Furlong, Founding President & CEO, Parent Project for Muscular Dystrophy <p><i>Regency Ballroom A-B-C</i></p> <p>Lifetime Achievement Awards Presentation</p> <ul style="list-style-type: none"> • Carol Abraham, Chairwoman, National LGMD Conference <p>Lifetime Achievement Award: Muscular Dystrophy Research</p> <p>Recipient • Jerry R. Mendell, MD, Nationwide Children’s Hospital</p> <p>Lifetime Achievement Award: Muscular Dystrophy Advocacy</p> <p>Recipient • Pat Furlong, Founding President & CEO, Parent Project for Muscular Dystrophy</p>

SUNDAY, SEPTEMBER 1, 2019

8:45 – 9:45 AM	Exhibitor Room Designated Time <i>Regency Ballroom D</i> <ul style="list-style-type: none"> • Breakfast Snacks - Sponsored by Camron'sCure 	
9:45 – 10:00 AM	BREAK	
10:00 – 10:45 AM	Breakout Sessions 3:	<p>Genetics 101: Understanding Mutations and Test Reports</p> <ul style="list-style-type: none"> • Bradley A. Williams, PhD, Jain Foundation • Monkol Lek, PhD, Yale University <p><i>Regency Ballroom A-B-C</i></p> <hr/> <p>Physical and Occupational Therapy and Mobility Aids</p> <ul style="list-style-type: none"> • Meredith James, Physiotherapist, University of Newcastle, UK • Vovanti Jones, MD, University of Missouri <p><i>Acapulco</i></p> <hr/> <p>Recreation and Travel with LGMD</p> <ul style="list-style-type: none"> • Melanie Bordes, Jenna Keindel, Yumi Shim, Jill Wagner <p><i>Toronto</i></p>
11:00 AM – 1:00 PM	LUNCH BREAK	
1:00 – 1:25 PM	Plenary Session IV: Part 1	<p>CLINICAL TRIALS AND EMERGING TREATMENTS:</p> <p>Gene Therapy: The SMA Success Story and Lessons for Future Gene Therapy Treatments</p> <ul style="list-style-type: none"> • Jerry R. Mendell, MD, Nationwide Children's Hospital <p><i>Regency Ballroom A-B-C</i></p>

SUNDAY, SEPTEMBER 1, 2019 – CONTINUED

1:25 – 2:15 PM	Plenary Session IV: Part 2 Clinical Trials in LGMD <ul style="list-style-type: none"> • Kathryn Wagner, MD, PhD, Kennedy - Krieger Institute Overview of Gene Therapy for LGMD <ul style="list-style-type: none"> • Louise Rodino-Klapac, PhD, Sarepta Therapeutics 	<i>Regency Ballroom A-B-C</i>
2:15 – 2:30 PM	BREAK	
2:30 – 3:15 PM	Participants' Perspectives on Clinical Trials <ul style="list-style-type: none"> • Donavon Decker, Aleksandra Leijenhorst, Dan Pope 	<i>Regency Ballroom A-B-C</i>
	Breakout Sessions 4: Cardiac Management in LGMD <ul style="list-style-type: none"> • Elizabeth McNally, MD, PhD, Northwestern University 	<i>Acapulco</i>
	Family and Parenting as a Person with LGMD <ul style="list-style-type: none"> • Cassidy Nilles, Rob Roozeboom, Yumi Shim 	<i>Toronto</i>
3:15 – 3:30 PM	BREAK	
3:30 – 4:15 PM	Plenary Session V: NOVEL TREATMENT APPROACHES: Which MD treatment approaches are applicable to which LGMD? <ul style="list-style-type: none"> • Jeff Chamberlain, PhD, University of Washington 	<i>Regency Ballroom A-B-C</i>
4:15 – 4:30 PM	BREAK	
4:30 – 5:45 PM	Plenary Session VI: ASK THE EXPERTS PANEL #2: <ul style="list-style-type: none"> • Jeffrey S. Chamberlain, PhD, University of Washington • Elizabeth McNally, MD, PhD, Northwestern University • Jerry R. Mendell, MD, Nationwide Children's Hospital • Louise Rodino-Klapac, PhD, Sarepta Therapeutics • Kathryn Wagner, MD, PhD, Kennedy - Krieger Clinic • Matthew P. Wicklund, MD, Univ of Colorado 	<i>Regency Ballroom A-B-C</i>
5:45 PM	ADJOURN	
MONDAY, SEPTEMBER 2, 2019 – SOCIAL FUN DAY IN CHICAGO!		
12:00 Noon	Gather and Coordinate a Day to Explore Chicago	<i>Hotel Lobby</i>



The National 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

"TOGETHER WE ARE STRONGER"

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