

2019 National Limb Girdle Muscular Dystrophy Conference

Hyatt Regency - Chicago, Illinois

The Conference Agenda is Subject to Change



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>	<i>Plaza Ballroom Foyer - East Tower</i>
3:00 – 5:30 PM	GENERAL REGISTRATION - CHECK IN	<i>Plaza Ballroom Foyer - East Tower</i>
7:00 – 9:00 PM	WELCOME RECEPTION	<i>Plaza Ballroom Foyer - East Tower</i>

SATURDAY, AUGUST 31, 2019

8:45 – 9:45 AM	Exhibitor Room Designated Time	<i>Regency Ballroom D - West Tower</i>
8:45 – 10:00 AM	GENERAL REGISTRATION - LATE CHECK IN	<i>Regency Foyer - West Tower</i>
10:00 – 11:15 AM	Plenary Session I: WELCOME AND OVERVIEW OF LGMD: Conference Welcome <ul style="list-style-type: none"> • Kathryn Bryant, National Director, Speak Foundation Overview of LGMD: Symptoms, Genetics, Inheritance, and Incidence <ul style="list-style-type: none"> • Matthew P. Wicklund, MD, University of Colorado, Denver The GRASP LGMD Consortium <ul style="list-style-type: none"> • Nicholas E. Johnson, MD, Virginia Commonwealth University 	<i>Regency Ballroom A-B-C West Tower</i>
11:15 – 11:30 AM	BREAK	
11:30 AM – 12:15 PM	Breakout Sessions 1: Genetic Diagnosis: Why and How <ul style="list-style-type: none"> • Peter B. Kang, MD, University of Florida, Gainesville • Conrad C. Weihl, MD, PhD, Washington University, St. Louis • Monkol Lek, PhD, Yale University • Rich Dineen, MS, CGC, Rush University Medical Center 	<i>Regency Ballroom A-B-C West Tower</i>
	Respiratory Management in LGMD <ul style="list-style-type: none"> • Matthew P. Wicklund, MD, University of Colorado, Denver 	<i>Acapulco - West Tower</i>
	Young Adults with LGMD: School, Career, Life <ul style="list-style-type: none"> • Chris Anselmo, Nicole Henry, Andrea Lane, Jane B. Lockwood 	<i>Toronto - West Tower</i>

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 and CureLGMD2i Foundation 	<i>Regency Ballroom D - West Tower</i>
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 	<i>Regency Ballroom A-B-C West Tower</i>
2:40 – 2:55 PM	BREAK	<ul style="list-style-type: none"> • Afternoon Snacks - Sponsored by Camron'sCure Foundation 	<i>Regency Ballroom D - West Tower</i>
2:55 – 3:45 PM	Plenary Session III:	PATIENT INVOLVEMENT IN CLINICAL TRIAL READINESS: Natural History Studies and Outcome Measures for LGMD <ul style="list-style-type: none"> • Volker Straub MD, University of Newcastle, UK • Meredith James, Physiotherapist, University of Newcastle, UK 	<i>Regency Ballroom A-B-C West Tower</i>
3:45 – 4:00 PM	BREAK		<i>Regency Ballroom D - West Tower</i>
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 	<i>Regency Ballroom A-B-C West Tower</i>
		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 	<i>Acapulco - West Tower</i>
		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, CamronsCure Foundation 	<i>Toronto - West Tower</i>
4:45 – 5:45 PM	Exhibitor Room Designated Time		<i>Regency Ballroom D - West Tower</i>

SATURDAY, AUGUST 31, 2019 – CONTINUED

5:45 – 7:00 PM

BREAK

7:00 PM

BANQUET DINNER

Introduction: The Speak Foundation Initiatives and Advocacy Organizations

- Kathryn Bryant, National Director, Speak Foundation

*Regency Ballroom A-B-C
West Tower*

Keynote Address:

“Lessons from the DMD Community for LGMD Advocacy”

- Pat Furlong, Founding President & CEO, Parent Project for Muscular Dystrophy

Lifetime Achievement Awards Presentation

- Carol Abraham, Chairwoman, National LGMD Conference

Lifetime Achievement Award: Muscular Dystrophy Research

Recipient • Jerry R. Mendell, MD, Nationwide Children’s Hospital

Lifetime Achievement Award: Muscular Dystrophy Advocacy

Recipient • Pat Furlong, Founding President & CEO, Parent Project for Muscular Dystrophy

SUNDAY, SEPTEMBER 1, 2019

8:45 – 9:45 AM

Exhibitor Room Designated Time

Regency Ballroom D - West Tower

9:45 – 10:00 AM

BREAK

10:00 – 11:00 AM

Breakout Sessions 3:

Genetics 101: Understanding Mutations and Test Reports

- Bradley A. Williams, PhD, Jain Foundation

*Regency Ballroom A-B-C
West Tower*

Physical and Occupational Therapy and Mobility Aids

- Meredith James, Physiotherapist, University of Newcastle, UK
- Vovanti Jones, MD, University of Missouri

Acapulco - West Tower

Recreation and Travel with LGMD

- Melanie Bordes, Jenna Keindel, Yumi Shim, Jill Wagner

Toronto - West Tower

11:00 AM – 1:00 PM

LUNCH BREAK

- *Lunch - Sponsored by CureLGMD2i Foundation and ML BioSolutions*

Regency Ballroom D - West Tower

1:00 – 1:25 PM

**Plenary Session IV:
Part 1**

CLINICAL TRIALS AND EMERGING TREATMENTS:

Gene Therapy: The SMA Success Story and Lessons for Future Gene Therapy Treatments

- Jerry R. Mendell, MD, Nationwide Children’s Hospital

*Regency Ballroom A-B-C
West Tower*

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 West Tower</i>
2:15 – 2:30 PM	BREAK	<i>Regency Ballroom D - West Tower</i>
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 West Tower</i>
	Breakout Sessions 4: Cardiac Management in LGMD <ul style="list-style-type: none"> • Elizabeth McNally, MD, PhD, Northwestern University 	<i>Acapulco - West Tower</i>
	Family and Parenting as a Person with LGMD <ul style="list-style-type: none"> • Cassidy Nilles, Rob Roozeboom, Yumi Shim 	<i>Toronto - West Tower</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 West Tower</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 West Tower</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 with Friends and Family <ul style="list-style-type: none"> • This is an unstructured optional event. Chicago offers so much to see and do. Meeting in the Hotel Lobby may provide an opportunity to coordinate doing something fun in Chicago for the afternoon - together with others from the LGMD Community. 	<i>Hotel Lobby - East Tower</i>
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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