

CONFERENCE SCHEDULE



FRIDAY ★ OCTOBER 27, 2023

4:00 – 6:30 PM	General Registration — Check In	Constitution Ballroom Foyer
5:00 – 6:00 PM	Exhibitor Setup	Constitution Ballroom Foyer
6:30 – 8:00 PM	Welcome Reception & Mixer	Constitution Ballroom Foyer

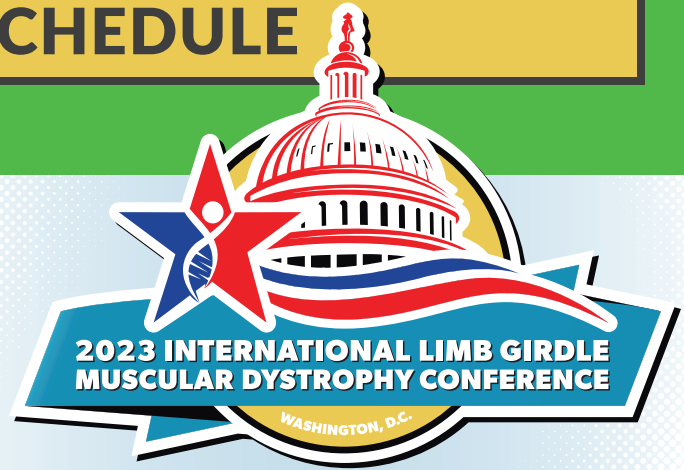
SATURDAY ★ OCTOBER 28, 2023

8:00 – 11:00 AM	General Registration — Late Check In	Constitution Ballroom Foyer
9:00 – 10:30 AM	<p>Plenary Session I:</p> <p>Overview of LGMDs and Natural History Studies:</p> <p>Conference Welcome</p> <ul style="list-style-type: none"> • Kathryn Bryant Knudson, President, The Speak Foundation <p>LGMDs: Overview and Progress in Developing Treatments</p> <ul style="list-style-type: none"> • Nicholas E. Johnson, MD, Virginia Commonwealth University <p>How Natural History Studies Enable Clinical Trials</p> <ul style="list-style-type: none"> • Meredith James, PhD, University of Newcastle, UK <p>Current LGMD Natural History Studies</p> <ul style="list-style-type: none"> • Linda Lowes, PhD, Nationwide Children's Hospital 	Constitution Ballroom
10:30 – 10:45 AM	Break	Constitution Ballroom Foyer
10:45 – 11:45 AM	<p>Plenary Session II:</p> <p>LGMD Diagnosis and Clinical Care:</p> <p>The NIH Diagnostic Program for Neuromuscular Diseases</p> <ul style="list-style-type: none"> • Carsten Bonnemann, MD, National Institutes of Health <p>Clarifying the Genetics of LGMDs</p> <ul style="list-style-type: none"> • Conrad (Chris) Wehl, MD, PhD, Washington University School of Medicine <p>Developing Standards of Care for LGMDs</p> <ul style="list-style-type: none"> • Volker Straub, MD, PhD, University of Newcastle, UK 	Constitution Ballroom
11:45 AM – 12:55 PM	<p>Lunch & Learn</p> <p>Fortify – Launch of a Phase 3 Study and What We've Learned So Far about BBP-418 for LGMD2i/R9</p> <p>Presented by Platinum Sponsor ML BioSolutions</p>	Constitution Ballroom

Grand Hyatt — Washington, D.C. ★ All Conference Session Times are in Eastern Daylight Time (EDT), UTC-4 ★ Conference schedule is subject to change

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

CONFERENCE SCHEDULE



SATURDAY ★ OCTOBER 28, 2023 — *Continued*

12:55 – 1:55 PM	Breakout Session 1:	Managing Respiratory & Cardiac Involvement in LGMDs <ul style="list-style-type: none"> Noah Lechtzin, MD, Johns Hopkins Amy Fulford, RRT, Millennium Respiratory Services Kan Hor, MD, Nationwide Children's Hospital 	Constitution Ballroom
		Ask the Expert: Q&A <ul style="list-style-type: none"> All Speakers from Plenary Sessions I & II 	Cabin John / Arlington
		Family and Parenting as a Person with LGMD <ul style="list-style-type: none"> Chris Carroll, Jessica Evans, PsyD, Yumi Shim 	Wilson / Roosevelt
		Building Community Connections: Children and Teens <ul style="list-style-type: none"> Hosts: Sammi Brazzo, Brooklyn Garza 	Renwick / Bulfinch
1:55 – 2:05 PM	Break		Constitution Ballroom Foyer
2:05 – 3:35 PM	Plenary Session III:	Advocacy Initiatives in LGMD: Overview of Advocacy for Rare Diseases <ul style="list-style-type: none"> Annie Kennedy, Everylife Foundation Collaborating for Advocacy <ul style="list-style-type: none"> Paul Melmeyer, Muscular Dystrophy Association Muscular Dystrophy Coordinating Committee & NIH Support for Research <ul style="list-style-type: none"> Glen Nuckolls, PhD, National Institutes of Health Community Interactions with FDA <ul style="list-style-type: none"> Jennifer Levy, PhD, Coalition to Cure Calpain 3 Brad Williams, PhD, Jain Foundation 	Constitution Ballroom
3:35 – 3:45 PM	Break		Constitution Ballroom Foyer
3:45 – 4:45 PM	Breakout Session 2:	Building Community Connections: 20's - 30's - 40's <ul style="list-style-type: none"> Hosts: Andrea Lane, Julianna Rodrigues 	Cabin John / Arlington
		Living a Fulfilling Life with LGMD <ul style="list-style-type: none"> Melissa Grove MS, LPC 	Wilson / Roosevelt
		Building Community Connections: Adults 50's+ <ul style="list-style-type: none"> Hosts: Carol Abraham, Pat Moeschen 	Renwick / Bulfinch
4:45 – 6:00 PM	Break		

Grand Hyatt — Washington, D.C. ★ All Conference Session Times are in Eastern Daylight Time (EDT), UTC-4 ★ Conference schedule is subject to change

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

CONFERENCE SCHEDULE



SATURDAY ★ OCTOBER 28, 2023 — Continued

6:00 – 8:30 PM

Banquet Dinner

Welcome Back:

- Kathryn Bryant Knudson, The Speak Foundation

Videos: Youth Living with LGMD

Panel Discussion: Individuals Living with LGMD

Keynote Address:

Constitution Ballroom

FDA Fireside Chat with Peter Marks, MD, PhD, CBER, FDA

- Hosts: Kathryn Bryant Knudson & Brad Williams, PhD

LGMD Documentary from Proyecto Alpha: *Nunca Dejes de Moverte* (Never Stop Moving)



FREE Saturday Stream

Keynote Address: "FDA Fireside Chat with Peter Marks, MD, PhD, CBER, FDA"
VirtualEventPortal.com/Videos/LGMDKeynoteSpeaker

SUNDAY ★ OCTOBER 29, 2023

10:00 – 10:50 AM

Breakout Session 3:

Making Gene Therapy More Versatile:

Applying Gene Therapy to LGMDs

- Matthew P. Wicklund, MD, University of Texas in San Antonio

Bespoke Gene Therapy Initiative

- Phillip (PJ) Brooks, PhD, National Institutes of Health

What Will Gene Therapy 2.0 Look Like?

- Sharon Hesterlee, PhD, Muscular Dystrophy Association

Constitution Ballroom

Building Community Connections: Parents of Children with LGMD

- Hosts: Faran Day, Rachel DeConti

Cabin John / Arlington

Enhancing your Clinical Care Experience

- Vovanti Jones, MD, University of Missouri

Wilson / Roosevelt

10:50 – 11:00 AM

Break

Constitution Ballroom Foyer

Grand Hyatt — Washington, D.C. ★ All Conference Session Times are in Eastern Daylight Time (EDT), UTC-4 ★ Conference schedule is subject to change

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

CONFERENCE SCHEDULE



SUNDAY ★ OCTOBER 29, 2023 — *Continued*

11:00 AM – 12:10 PM	Plenary Session IV:	<p>Clinical Trial Updates (Part 1):</p> <p>ML BioSolutions: Update on BBP-418 clinical studies for LGMD 2i/R9</p> <ul style="list-style-type: none"> • Doug Sproule, MD, ML BioSolutions <p>Sarepta Therapeutics: LGMD Program Update</p> <ul style="list-style-type: none"> • Louise Rodino-Klapac, PhD, Sarepta Therapeutics <p>Preliminary Experiences with Atamyo's FKRPs Gene Therapy</p> <ul style="list-style-type: none"> • John Vissing, MD, University of Copenhagen 	Constitution Ballroom
12:10 – 1:10 PM	Lunch & Learn	<p>Relax, Recharge, and Reconnect Patient Networking Lunch Presented by Platinum Sponsor Sarepta Therapeutics</p>	Constitution Ballroom
1:10 – 1:50 PM	Plenary Session V:	<p>Clinical Trial Updates (Part 2):</p> <p>An Update on AskBio's LION-101 Study for the Treatment of LGMD 2i/R9</p> <ul style="list-style-type: none"> • Anh Nguyen, MD, AskBio <p>EDG-5506: A Novel Approach to Protecting Muscle in DMD, BMD, and LGMDs</p> <ul style="list-style-type: none"> • Joanne Donovan, MD, PhD, Edgewise Therapeutics 	Constitution Ballroom
1:50 – 2:00 PM	Break		Constitution Ballroom Foyer
2:00 – 3:00 PM	Plenary Session VI:	<p>Development of New Treatments and New Assessments</p> <p>Developing Cell Therapy for Muscular Dystrophy</p> <ul style="list-style-type: none"> • Peter Kang, MD, University of Minnesota <p>Chaperone Compounds for Rescue of Missense Sarcoglycan Mutations</p> <ul style="list-style-type: none"> • Dorianna Sandonà, PhD, University of Padova <p>Regenerative Medicine in LGMD2A/R1. VTA-100: A Novel Therapeutic Cell-based Approach</p> <ul style="list-style-type: none"> • Michael Molyneaux, MD, Vita Therapeutics <p>Therapeutic Approaches for Dominantly Inherited LGMDs</p> <ul style="list-style-type: none"> • Andrew Findlay, MD, Washington University 	Constitution Ballroom
3:00 PM	Closing Remarks	<ul style="list-style-type: none"> • Kathryn Bryant Knudson, The Speak Foundation 	Constitution Ballroom
3:05 PM	Adjourn		

Grand Hyatt — Washington, D.C. ★ All Conference Session Times are in Eastern Daylight Time (EDT), UTC-4 ★ Conference schedule is subject to change

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