

Featured Speakers



Volker Straub, MD

Director, John Walton Muscular Dystrophy
Research Centre, Newcastle University,
Newcastle upon Tyne, England, UK.



Peter B. Kang, MD

Pediatric Neuromuscular Neurologist
and Physician-Scientist,
University of Minnesota Medical School, USA.



Tahseen Mozaffar, MD

Professor of Neurology and Laboratory
Medicine and Vice Chair for Research,
Department of Neurology at
University of California, Irvine, USA.



Katherine D. Mathews, MD

Director, Iowa Neuromuscular Program.
Professor of Pediatrics-General Neurology,
University of Iowa, USA.



Nicholas E. Johnson, MD

Associate Professor and Vice Chair of Research,
Virginia Commonwealth University, USA.

Friday | September 17, 2021

All conference session times are in Eastern Daylight Time (EDT), UTC -4. | Conference schedule is subject to change.

12:00 – 12:15 PM Welcome

- Kathryn Bryant Knudson | Conference Administrator
- Brad Williams, PhD | Conference Chairman
- Carol Abraham | Conference Vice Chairwoman

12:15 – 12:45 PM Overview of Limb-Girdle Muscular Dystrophies

An overview of what LGMDs are, their genetics, and their symptoms. Included will be an update on what has happened in the limb-girdle community since our last conference in 2019.

- Volker Straub, MD | Newcastle University, UK

12:45 – 12:50 PM My LGMD Story: Aric Hershley | USA

12:50 – 1:00 PM Break

1:00 – 2:20 PM Updates on Clinical Trials and Emerging Treatments

An overview of treatments being developed for various LGMD subtypes and clinical trials which are being organized to test them.

- Moderator: Jenn Levy, PhD | Coalition to Cure Calpain 3, USA
- GRASP LGMD Panel Discussion
- Peter B. Kang, MD | University of Minnesota, USA
- Tahseen Mozaffar, MD | University of California, Irvine, USA
- Katherine D. Mathews, MD | University of Iowa, USA
- Nicholas E. Johnson, MD | Virginia Commonwealth University, USA

2:20 – 2:30 PM Break

2:30 – 3:10 PM Patient-Focused Session: Planning for the Future

How can a person with LGMD live their best life? People living with LGMD and caregivers share their strategies for navigating different aspects of life.

- Moderator: Melissa Grove, MS, LPC | Legacy Counseling Center, USA
- Aleksandra Leijenhorst | Netherlands
- Jasmine Robinson | USA
- Joshua Thayer | USA
- Kae Tran | Canada

3:10 – 3:15 PM Break

3:15 – 4:00 PM Patient-Focused Session: Caregivers – Who, What and How?

Individuals living with LGMD share their experiences of hiring caregivers to assist with their activities of daily living.

- Moderator: Carol Abraham | Coalition to Cure Calpain 3, USA
- Mark Barnett | USA
- Anne Brastow | USA
- Andrew Robertson | UK
- Bliss Welch | USA

4:00 – 5:00 PM Community Reception



Join a Conference Community Reception Room

Zoom link:

<https://us02web.zoom.us/j/86934608410?pwd=bW9Zb3NmU0NsUnNYZnNLZkdGcJlUJUZz09>

Meeting ID: **869 3460 8410**

Passcode: **770112**

Questions? Visit: www.TheSpeakFoundation.com

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Conference Schedule



Saturday | September 18, 2021

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11:30 AM – 12:00 PM



Introduction to Gene Therapy (Led by Sarepta Therapeutics)

In this 30-minute presentation, Sarepta will be presenting introductory gene therapy concepts. This will be an interactive presentation with the opportunity for the patient community to assess their current knowledge of gene therapy and expand their knowledge base.

- Allison Kreuzer, PhD | Director, Patient Affairs, Sarepta Therapeutics, Inc., USA

12:00 – 1:00 PM



Sarepta Therapeutics LGMD Pipeline Update

In this 60-minute presentation, Sarepta will be sharing an update on their pre-clinical and clinical LGMD pipeline and facilitating a short Q&A with the patient community following their presentation.

- Louise Rodino-Klapac, PhD | Executive Vice President, Chief Scientific Officer, Sarepta Therapeutics, Inc., USA

1:00 – 1:30 PM

Break

1:30 – 1:35 PM

My LGMD Story: Tatyana Vyatkina | Russia

1:35 – 1:45 PM

LiftSeat® Demonstration

1:45 – 1:50 PM

"Neurologist of the Year" Award

1:50 – 2:00 PM

Break

Keynote Session

2:00 – 3:00 PM

Regulatory Aspects of Drug Development and the Patient's Role

Former FDA Commissioner (2017-2019) discusses challenges in developing therapies for rare diseases involving new, cutting-edge technologies such as gene and cell therapies, and gene editing. Dr. Gottlieb will also discuss how people living with LGMD can contribute towards the development of treatments.

- Keynote Speaker: Scott Gottlieb, MD | Former FDA Commissioner, USA

3:00 – 3:45 PM

Patient Interaction with the FDA

Learn how individuals living with rare conditions such as LGMD can communicate their experiences and priorities with the FDA.

- James Valentine, JD, MHS | Associate, Hyman, Phelps & McNamara, P.C., USA

3:45 – 3:50 PM

Patient-Focused Drug Development (PFDD) Introduction for Patient Engagement

A Patient-Focused Drug Development (PFDD) meeting with the FDA is being organized for several LGMD subtypes. Discover how the LGMD community can participate.

3:50 – 4:00 PM

Break

4:00 – 5:00 PM

Patient-Focused Session: Maintaining Bone Health, Flexibility & Function

Loss of flexibility and decreased bone density are common complications impacting mobility in LGMD. Clinicians in these areas of practice share strategies for managing these challenges.

- Moderator: Carol Abraham | Coalition to Cure Calpain 3, USA
- Linda Lowes, PT, PhD | Nationwide Children's Hospital, USA
- Meredith James, PT | Physiotherapist, University of Newcastle, UK
- Philip Zeitler, MD | Children's Hospital Colorado, USA

Featured Speakers



Louise Rodino-Klapac, PhD
Executive Vice President, Chief Scientific Officer,
Sarepta Therapeutics, Inc., USA.



Keynote Speaker Scott Gottlieb, MD

Scott Gottlieb is a physician and served as the 23rd Commissioner of the U.S. Food and Drug Administration. He is a resident fellow at the American Enterprise Institute and a partner at the venture capital firm New Enterprise Associates. Dr. Gottlieb is an aggressive advocate for advancing public health through developing and implementing innovative approaches to improving medical outcomes, reshaping healthcare delivery, and expanding consumer choice and safety. Dr. Gottlieb is widely published in leading medical journals, and is a regular contributor to CNBC and Face the Nation. Fortune Magazine recognized him as one of the "World's 50 Greatest Leaders" in 2018 and 2019.



James Valentine, JD, MHS
Hyman, Phelps & McNamara, USA.



Linda Lowes, PT, PhD
Principal Investigator, Center for Gene Therapy,
Nationwide Children's Hospital, USA.



Meredith James, PT
Clinical Physiotherapist, John Walton Muscular
Dystrophy Research Centre, Newcastle, UK.



Philip Zeitler, MD
Professor of Pediatrics, University of Colorado
Anschutz Medical Campus, USA.

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Featured Speakers



Anh Nguyen, MD
VP, Sector Lead, Musculoskeletal,
AskBio, USA.



Sharon Hesterlee, PhD
Chief Research Officer,
Muscular Dystrophy Association, USA.



Jorge Bevilacqua, MD, PhD
Professor, Department of Neurology
and Neurosurgery, Hospital Clínico
Universidad de Chile, Chile.



Conrad (Chris) Weihl, MD, PhD
Professor of Neurology,
Washington University School of Medicine,
St. Louis, Missouri, USA.



Monkol Lek, PhD
Assistant Professor of Genetics,
Yale University School of Medicine, USA.

Sunday | September 19, 2021

All conference session times are in Eastern Daylight Time (EDT), UTC -4. | Conference schedule is subject to change.

12:00 – 1:30 PM



Sponsored Session: AskBio

In this 90-minute presentation, AskBio will introduce and give an overview of their patient advocacy program and present the pre-clinical data supporting the clinical advancement of LION-101. They will also offer an inside look from a GRASP LGMD Investigator into LGMD gene therapy trials, and into the perspectives and challenges of living with LGMD2i from a patient family.

- Anh Nguyen, MD | AskBio, USA
- Nicholas E. Johnson, MD | Virginia Commonwealth University, USA
- Craig Norton | USA
- CJ Norton | USA
- Sharon Hesterlee, PhD | Muscular Dystrophy Association, USA

1:30 – 1:35 PM

My LGMD Story: Szymczak Family | USA

1:35 – 1:55 PM

Break

1:55 – 2:00 PM

My LGMD Story: Andrew Robertson | UK

2:00 – 4:00 PM

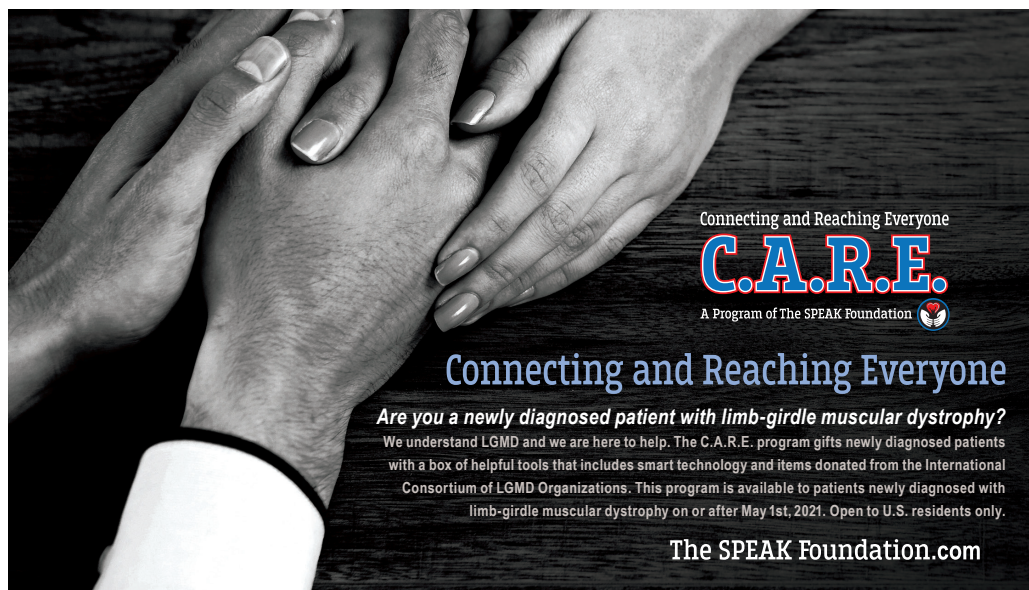
Diagnostic Landscape for LGMDs

Correctly diagnosing LGMDs and other conditions causing similar symptoms is often challenging. Clinicians and geneticists describe the diagnostic process, with patients sharing their journeys.

- Moderator: Laura Rufibach, PhD | Jain Foundation, USA
- Jorge Bevilacqua, MD, PhD | University of Chile, Chile
- Conrad (Chris) Weihl, MD, PhD | Washington University School of Medicine, USA
- Monkol Lek, PhD | Yale University School of Medicine, USA

Patient Panel:

- Mark Barnett | USA
- Kyle Harrington | USA
- Jenna Keindel | Canada
- Andrea Klein | USA
- Noni Uribe | USA



Connecting and Reaching Everyone
C.A.R.E.
A Program of The SPEAK Foundation

Connecting and Reaching Everyone

Are you a newly diagnosed patient with limb-girdle muscular dystrophy?
We understand LGMD and we are here to help. The C.A.R.E. program gifts newly diagnosed patients with a box of helpful tools that includes smart technology and items donated from the International Consortium of LGMD Organizations. This program is available to patients newly diagnosed with limb-girdle muscular dystrophy on or after May 1st, 2021. Open to U.S. residents only.

The SPEAK Foundation.com

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Conference Schedule

Monday | September 20, 2021

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11:15 AM – 12:00 PM

Global LGMD Updates: India, Egypt, and Latin America

LGMD is a global disease affecting all parts of the world. Leading clinicians from different regions discuss diagnosis, research, and opportunities for advocacy.

- Moderator: Sarah Emmons | Jain Foundation, USA
- Satish Khadilkar, MD | Bombay Hospital Institutes of Medicine, India
- Rasha el Sherif, MD, PhD | Myocare National Foundation, Egypt
- Jorge Bevilacqua, MD, PhD | University of Chile, Chile

12:00 – 1:30 PM



Sponsored Session: GFB ONLUS

In this 90-minute presentation, GFB Onlus will share information about the clinical determinants of disease progression in patients with beta-sarcoglycan gene mutations, a quality of life study for patients with alpha-sarcoglycan, beta-sarcoglycan, and gamma-sarcoglycan mutations, CFTR correctors in sarcoglycanopathies, and unraveling muscle differences to approach sarcoglycanopathies.

- Yvan Torrente, MD, PhD | University of Milan, Italy
- Claudia Paniga | GFB Onlus, Italy
- Dorianna Sandonà, MD, PhD | University of Padua, Italy
- Carles Sánchez Riera, PhD | Spain

1:30 – 1:35 PM

My LGMD Story: Brandi Benton | USA

1:35 – 1:45 PM

LiftSeat® Demonstration

1:45 – 2:00 PM

Break

2:00 – 4:00 PM

Respiratory and Cardiac Complications and the Importance of Screening

Respiratory and cardiac complications are associated with many forms of LGMD.

It is vitally important for individuals with LGMD to receive proper screening and symptom management.

- Moderator: Kathryn Bryant Knudson | Conference Administrator
- Matthew P. Wicklund, MD | University of Colorado, USA
- Oren Kupfer, MD | University of Colorado, USA
- Elizabeth McNally, MD, PhD | Northwestern University, USA

4:00 – 4:30 PM

Conference Wrap-Up

LGMD Patient Network



The Speak Foundation is assembling the first-ever LGMD Patient Network and YOU are invited to be included. Signing up is absolutely free, confidential, and can be done easily by visiting [TheSpeakFoundation.com](https://www.TheSpeakFoundation.com). Don't Miss Out — Join Our LGMD Patient Network Today!

[TheSpeakFoundation.com](https://www.TheSpeakFoundation.com)

Featured Speakers



Satish Khadilkar, MD

Dean, Medical Faculty of Bombay Hospital
Institute of Medical Sciences.
Head of the Department of Neurology,
BHIMS, Mumbai, India.



Rasha el Sherif, MD, PhD

Founder of Myocare Neuromuscular Foundation.
TGOC LGMD Subgroup Lead in TREAT-NMD, Egypt.



Yvan Torrente, MD, PhD

Professor, Università degli Studi, Milan.
Co-Founder, UNISTEM Center,
Università degli Studi, Milan, Italy.



Claudia Paniga

Interpreter, GFB Onlus, Italy.



Dorianna Sandonà, MD, PhD

Professor, Biomedical Sciences,
University of Padua, Italy.



Carles Sánchez Riera, PhD

Postdoctoral Researcher,
University of La Sapienza, Rome, Italy.



Matthew P. Wicklund, MD

Professor of Neurology,
University of Colorado, USA.



Oren Kupfer, MD

Pediatric pulmonologist, University of Colorado
School of Medicine and Children's Hospital,
Colorado, USA.



Elizabeth McNally, MD, PhD

Cardiologist, Center for Genetic Medicine
at Northwestern, Chicago, Illinois, USA.

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