GET READY FOR THE

2022 LGMD GLOBAL ADVOCACY SUMMIT

LIMB GIRDLE MUSCULAR DYSTROPHY

At the **2022 LGMD Global Advocacy Summit**, a first for the limb-girdle community, you will learn directly from pharmaceutical companies about the clinical trials coming in 2022 and 2023. Additionally, you will hear from patient advocacy leaders from around the world about the regulatory drug process in both the U.S. and Europe. You will also have the opportunity to listen to LGMD patients and caregivers share with the invited companies about what matters most to them in trials and in drug development. **You do not want to miss it!**

SUMMIT SCHEDULE | MAY 20, 2022 | 12:00 PM – 7:00 PM

12:00 PM

Welcome
European Advocacy

Learn about updates on drug development and the regulatory process in Europe, as well as the various structures that exist in Europe for patient advocacy and how you can get involved.

- **Rob Camp** | Director, International Patient Collaboration, EUPATI (Spain)
- **Aleksandra Leijenhorst** | Chair, Stichting Spierkracht (Netherlands) and Coordinator, LGMD CAB (Europe)


Rob Camp


Aleksandra Leijenhorst

1:00 PM

International Updates

Hear the latest LGMD updates from AFM-Téléthon.

- **Mandine Casado** | Expert Patient, GI LGMD, AFM-Téléthon (France)
- **Mélanie Bordes** | Expert Patient, GI LGMD, AFM-Téléthon (France)

Hear updates about The Quality Project by GFB Onlus, which seeks to improve the quality of life for patients living with sarcoglycanopathies.

- **Carles Sánchez Riera, PhD** | Researcher, Sapienza University of Rome (Italy)


Mandine Casado


Mélanie Bordes


Carles Sánchez Riera, PhD

1:10 PM

Roundtable Discussion 1

In this unique discussion, five individuals living with LGMD from around the globe will share what matters most to them in clinical trials and drug development.

- **Moderator:** Kathryn Bryant Knudson | Founder/CEO, The Speak Foundation (USA)
- Brandi Benton (USA)
- Melissa Grove (USA)
- Kathryn Newnham (England)
- Carles Sánchez Riera, PhD | Researcher, Sapienza University of Rome (Italy)
- Joshua Thayer (USA)

ALL TIMES ARE IN EASTERN DAYLIGHT TIME (EDT), UTC -4. | Summit schedule is subject to change.

WATCH THE LIVESTREAM EVENT AT

WWW.MALONE-MEDIA.COM/VIDEOS/LGMDSUMMIT

SUMMIT SCHEDULE | MAY 20, 2022 | 12:00 PM – 7:00 PM

1:45 PM US Advocacy

In this session, you will gain insight on the various structures that exist in the United States for patient advocacy so you can be an effective voice for LGMD. You will also learn about the drug development and regulatory process from start to finish and find out how you can be involved.

- **Paul Melmeyer** | Vice President, Public Policy and Advocacy, Muscular Dystrophy Association (MDA) (USA)
- **Sharon Hesterlee** | Chief Research Officer, Muscular Dystrophy Association (MDA) (USA)


Paul Melmeyer


Sharon Hesterlee, PhD

Q&A

- **Brad Williams, PhD** | Director of Research & Diagnostic Innovation, Jain Foundation (USA)
- **Sarah Shira Emmons** | Director of Global Patient Outreach & Community Strategies, Jain Foundation (USA)

2:45 PM US Updates

Hear updates from the Principal Investigator of the GRASP-LGMD Consortium about active studies and clinical trials.

- **Nicholas Johnson, MD, MSci, FAAN** | Virginia Commonwealth University; Principal Investigator, GRASP-LGMD Consortium (USA)


Nicholas Johnson, MD, MSci, FAAN

Research Updates for LGMD2B/R2

Listen to research updates and information about preclinical progress for LGMD2B/R2.

- **Douglas E. Albrecht, PhD** | Co-President, Jain Foundation (USA)
- **Laura Rufibach, PhD** | Co-President, Jain Foundation (USA)

3:00 PM Roundtable Discussion 2

In our second roundtable discussion for the Summit, you will hear from four more individuals who are ready to share their voices with the companies designing clinical trials and developing treatment drugs for LGMD.

- **Moderator:** Kathryn Bryant Knudson | Founder/CEO, The Speak Foundation (USA)
- **Moderator:** Sarah Foye | Founder/Organizer, Team Titin (USA)
- **Mélanie Bordes** | (France)
- **Rachel DeConti** | (USA)
- **John Faver** | (USA)
- **Keisha Greaves** | (USA)

3:30–7:00 PM Pharmaceutical Updates

3:30 PM Sarepta Therapeutics, Inc.

Sarepta will present updates on their gene therapy development programs for multiple LGMD subtypes.

- **Louise Rodino-Klapac, PhD** | Executive Vice President, Head of R&D and Chief Scientific Officer (USA)


Louise Rodino-Klapac, PhD

4:00 PM Atamyo Therapeutics

Atamyo will share on their upcoming gene therapy trial for LGMD2I/R9, as well as their plans for their next programs for other LGMDs.

- **Sophie Olivier, MD** | Chief Medical Officer (France)


Sophie Olivier, MD

4:30 PM Vita Therapeutics

Vita Therapeutics will offer information about their platform and plans for LGMD2A/R1, as well as their goal to file a first-in-human IND study later this year.

- **Douglas Falk, MS** | Cofounder and Chief Executive Officer (USA)
- **Steve Brooks, MD, MBA** | Senior Vice President, Clinical Affairs (USA)


Doug Falk, MS


Steve Brooks, MD, MBA

5:00 PM Asklepios BioPharmaceutical, Inc. (AskBio)

Hear from the AskBio team about their current clinical study (LION-CS101) of an investigational gene therapy for individuals with a confirmed genetic diagnosis of LGMD2I/R9.

5:30 PM ML Bio Solutions

The team at ML Bio Solutions will be sharing on their development of BBP-418, potentially the first oral treatment for patients with LGMD2I/R9.

- **Doug Sproule, MD, MSc** | Chief Medical Officer (USA)


Doug Sproule, MD, MSc

6:00 PM Edgewise Therapeutics

Edgewise Therapeutics will share about their current pipeline of precision therapeutics that regulate key muscle proteins to address LGMD.

- **Alan Russell, PhD** | Chief Scientific Officer (USA)


Alan Russell, PhD

6:30 PM Myogenica

Hear from a professor at the University of Minnesota about preclinical studies to enable a phase 1 clinical trial of iPSC-derived myogenic progenitors for the treatment of muscular dystrophies.

- **Rita Perlingeiro, PhD** | Cofounder (USA)


Rita Perlingeiro, PhD

7:00 PM Closing

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