**PRESS RELEASE**

**NEW 'VOICE OF THE PATIENT' REPORT HIGHLIGHTS UNMET MEDICAL NEEDS OF SIX SUBTYPES OF LIMB-GIRDLE MUSCULAR DYSTROPHY**

**Falls Church, Virginia (May 15, 2023)** – Today the Limb-Girdle Muscular Dystrophy (LGMD) Coalition, a partnership of six LGMD patient organizations, released the Voice of the Patient report summarizing outcomes from the September 2022 Externally-Led Patient Focused Drug Development Meeting (EL-PFDD). This meeting focused on LGMD subtypes LGMD2A/R1, LGMD2C/R5, LGMD2D/R3, LGMD2E/R4, LGMD2F/R6, and LGMD2i/R9. The goal of the report is to provide the U.S. Food and Drug Administration (FDA), clinicians, medical product developers, and academic researchers an opportunity to hear perspectives from individuals with LGMD on the health effects, daily impacts, treatment goals, and decision factors considered when seeking out or selecting a treatment. The meeting was conducted as a parallel effort to FDA's EL-PFDD initiative, a commitment under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V) to more systematically gather patients' perspectives on their conditions and available therapies to treat their conditions.

The meeting included 29 speakers and panelists who are living with LGMD2A/R1, LGMD2C/R5, LGMD2D/R3, LGMD2E/R4, LGMD2F/R6, and LGMD2I/R9, as well as caregivers of individuals living with these six subtypes. An additional 136 patients and family members responded to a live survey and over 250 written comments were submitted. In total, over 600 people attended the meeting, including over 27 FDA representatives, 48 drug company representatives, and over 400 LGMD patients and family participants. Common themes that emerged throughout the meeting included: (1) LGMDs are progressive diseases, but symptoms progress at different rates, and many individuals are severely affected; (2) People living with LGMDs urgently need better treatments; (3) LGMDs affect multiple body systems; (4) Individuals with LGMD experience discrimination and social exclusion, (5) Current treatments do not change the course of the diseases; (6) Many individuals are eager to participate in clinical trials.

Six patient organizations, jointly referred to as the LGMD Coalition, partnered together to organize the event: Coalition to Cure Calpain 3, CureLGMD2i, the Kurt+Peter Foundation, the LGMD2D Foundation, the McColl-Lockwood Laboratory for Muscular Dystrophy Research, and the Speak Foundation. The Voice of the Patient report and a recording of the meeting are available at https://LGMDPFDD.com. "It is our hope that drug developers and regulators will read the report and incorporate patients' and caregivers' perspectives when developing and reviewing new therapies to best meet the needs and expectations of our community," shared Coalition to Cure Calpain 3 Scientific Director Jennifer Levy.

The LGMD Coalition is appreciative for support from our sponsors, Sarepta Therapeutics, AskBio, Edgewise Therapeutics, ML BioSolutions, and Vita Therapeutics. "We wish to thank our many partners from advocacy and professional organizations, drug companies, federal agencies, and universities from across the world who

attended our meeting," said Kathryn Bryant, founder of the Speak Foundation. "We are extremely grateful to all the individuals in the LGMD community who contributed to the meeting's success by watching online, providing oral and written testimony, and responding to the survey questions. We believe that their engagement in this process provided insights into these subtypes of LGMD which will help the research community develop effective therapies."

**About LGMD**

LGMD is a term used for a group of rare neuromuscular diseases which are inherited and cause muscle weakness and wasting. The muscles most affected are those closest to the body (proximal), specifically the muscles of the shoulders, upper arms, pelvic area, and thighs. More than 30 subtypes of LGMD exist. Together, LGMDs have an estimated prevalence of about 2 in every 100,000 individuals. There is currently no cure for any subtype of LGMD.

**About the LGMD Coalition**

The LGMD Coalition is a group of six 501(c)3 nonprofit organizations -- Coalition to Cure Calpain 3, CureLGMD2i, the Kurt+Peter Foundation, the LGMD2D Foundation, the McColl-Lockwood Laboratory for Muscular Dystrophy Research, and the Speak Foundation – that collaborated to host an LGMD EL-PFDD focused on subtypes LGMD2A/R1, LGMD2C/R5, LGMD2D/R3, LGMD2E/R4, LGMD2F/R6, and LGMD2I/R9. Learn more at https://LGMDPFDD.com.