The Regulatory Environment Must Support the Development and Approval of Drugs for Rare and Ultra Rare Diseases

FDA Must Move with the Speed of Science to Get Treatments to Patients Who Need Them

- The FDA has tools and authorities to bring effective treatments to LGMD patients with speed and efficiency, but it must use the tools with which it has been empowered.
- For rare and ultra rare diseases, randomized controlled clinical trials are often not possible, and it is important to evaluate treatments using the tools we do have, like biomarkers, natural history, patient experience data, and real-world evidence, which Congress has supported.

FDA Must Prioritize Patient Focused Drug Development

- Particularly for rare diseases, it is essential that the patient perspective and experience be incorporated into the regulatory framework.
- The risk/benefit analysis for rare diseases is unique, and patients must be at the table informing regulators' decision-making. LGMD patients are the only ones who can provide context on risks/benefits/uncertainty.
- Patient Focused Drug Development (PFDD) is a critical tool for diseases like LGMD that progress irreversibly and lack any treatment options.
- The **BENEFIT Act** would be a positive step, as it would require the FDA to show how patient experience data has been used in the regulatory review process.

Codify Scientific Focused Drug Development (SFDD)

- FDA should gather data and expertise from scientific and medical experts in each disease area as they are weighing approvals.
- FDA reviewers cannot be expected to be experts in all diseases, and a formal process for incorporating expertise from scientists and physicians who are experts must be established.
- The LGMD community has been a leader on this topic, leading an SFDD meeting for LGMD in 2023.
- The **Scientific EXPERT Act (H.R.1532 and S. 822)** would codify this process by establishing a formal mechanism for SFDD.

For Additional Information Regarding Limb Girdle Muscular Dystrophy Day on the Hill, Please Contact Kathryn Bryant Knudson at: Kathryn@TheSpeakFoundation.com.