DAY ON THE HILL

LGMD

LIMB GIRDLE MUSCULAR DYSTROPHY

Critical Incentives are Needed to Get Treatments to Rare Disease Patients

Congress must reauthorize the Rare Pediatric Priority Review Voucher (PRV) Program

- **Give Kids a Chance Act (H.R. 1262/S. 932)** would reauthorize the essential PRV program.
- As many as half of the individuals living with a rare disease are children. Rare pediatric disease (RPD) priority review vouchers (PRVs) offer a crucial incentive to develop therapies for children with devastating and often life-threatening rare conditions.
- The program expired at the end of 2024, and there is now a lot of uncertainty as to its fate, leading to companies' hesitancy to invest and concern among patient communities.
- The program must be reauthorized to meet the significant unmet medical needs in pediatric rare disease communities.
- Since the program's inception in 2012, 53 PRVs have been awarded across 39 rare pediatric diseases. Of these diseases, many typically lead to death before the children reach adulthood and only three have had any FDA-approved treatments before 2012.

Support and Strengthen the Accelerated Approval Pathway

- The Accelerated Approval (AA) pathway was established in 1992 in response to the HIV/AIDS crisis. The AA pathway allows the FDA to use a surrogate endpoint, or biomarker, to evaluate the safety and efficacy of therapies for serious conditions with unmet need.
- Therapies approved through the Accelerated Approval pathway are held to the same rigorous standards as the FDA's traditional pathway, and should not be looked at any differently.
- In recent years, AA has come under attack with some policymakers questioning its utility and some payers declining to cover products approved via AA.
- Protecting and strengthening AA is critically important for the rare disease community.

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