My name is Kelly Maynard, and I am here to provide supportive context to amendment language introduced by Sen. Stephanie Kunze that impacts rare disease patients living in Ohio. I am the founder and President of Little Hercules Foundation, a patient advocacy group based in Dublin, Ohio.

Seven years ago, my 12 year old son, Jackson, was diagnosed with Duchenne muscular dystrophy. Duchenne is a rare and devastating neuromuscular disease that causes all of his muscles to progressively waste away until walking is no longer possible. Jackson stopped walking at 9.5, and now uses a power wheelchair. Eventually, Duchenne will weaken Jackson’s heart and lungs to the point that ventilation is necessary to breathe before it causes his death, which natural history tells us should be in about ten years from now.

In the United States, a rare disease is defined as a condition that affects fewer than 200,000 people. This definition was created by Congress in the [Orphan Drug Act of 1983](https://www.fda.gov/downloads/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/UCM517741.pdf). Rare diseases became known as orphan diseases because drug companies were not interested in adopting them to develop treatments. The Orphan Drug Act created financial incentives to encourage companies to develop new drugs for rare diseases. The rare disease definition was needed to establish which conditions would qualify for the new incentive programs.

The CDC estimates there may be as many as 7,000 rare diseases, with the FDA approving treatments for approximately 500. Taken individually, these diseases are rare, however, the total number of Americans living with a rare disease is estimated at between 25-30 million.

In recent years, rare disease patients have celebrated the FDA’s efforts to approve treatments for rare conditions at a historically faster pace. But all too often, following FDA approval, patients can experience difficulty accessing approved therapies due to both bureaucratic and process hurdles. Some rare disease patients have even waited 2+ years after approval to start treatment, causing irreparable harm to patients with progressive, debilitating, and life-limiting conditions.

The amendment language introduced by Sen. Kunze seeks to address the following current state policies and procedures:

1. State programs must provide access based on the Medicaid rebate agreement.
   1. Rare disease treatments must be covered from the outset, once a product comes to market, according to its FDA-approved use
   2. States choosing to review an approved rare disease therapy and to develop prior authorization criteria must do so within 90 days of approval while providing coverage during that time according to the FDA-approved use.
   3. Review of an approved therapy must not utilize state resources to reassess the safety and effectiveness as it would be redundant to and could conflict with the responsibilities of the FDA under the FDCA (federal Food and Drug and Cosmetic Act).
   4. Coverage policies must be made public within 90 days and conform to the medically accepted indication and FDA-approved label.
2. State Medicaid decision-making bodies such as the DUR Board and P&T Committees must include a patient representative, and must adopt a drug review process that incorporates testimony from critical stakeholders such as disease experts

Rare disease patients are often negatively impacted by one size fits all, decision making processes that don’t include them as an important voice in the process. Many rare diseases do not enjoy a robust body of research behind them to appropriately address the nuances and complexities of disease pathology, nor provide adequate data on which to make informed policy decisions. In these instances, patients with lived disease experience (on or off drug), caregivers, and experts that actually treat these diseases provide a critical resource that is widely and inexpensively available, yet overlooked. We believe this needs to change, and this change will lead to better informed health policy for all.