

My name is Kelly Maynard. I am the founder and President of Little Hercules Foundation, a national patient advocacy group based in Dublin, Ohio. I am also mother to 13 year old Jackson, who was diagnosed with a progressive, terminal disease called Duchenne muscular dystrophy when he was 5.

Duchenne is a rare and devastating neuromuscular disease that causes all of his muscles to progressively waste away until walking, moving his legs, raising his arms, and feeding himself are no longer possible. Jackson stopped walking at 9.5, and now uses a power wheelchair to ambulate. 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 10 years from now.

Some may ask why a rare disease advisory council is necessary. How is living with a rare disease any different than living with any other health condition? I'd like to address why a rare disease advisory council is a good idea for Ohio.

Receiving a rare diagnosis is itself devastating. Then begins the journey to learn everything you can about what lies ahead, and how to find the best care for the diagnosis. For some, good care is nearby, as is our case living so close to Nationwide Childrens, which is a world's leading research and care center in Duchenne. But for a large portion of those living with Duchenne in the lower half of the country, there is no expertise nearby. Many of these patients and families travel here to Ohio or expert care centers in other states, twice a year. These bi-annual appointments last two days, requiring families to pay expensive travel costs.

A large number of rare disease patients qualify for Medicaid due to the disabling nature of the disease. But not all Medicaid programs cover out of state care. This also results in large out of pocket expenses for patients.

Drug development for rare therapies is very different than for non-rare conditions. Clinical trials tend to have fewer patients, and are often approved by the FDA on an accelerated approval pathway aimed at getting treatments for serious, life-limiting conditions to patients more quickly than a standard approval. This approval pathway is often misunderstood and even rejected by payers, resulting in patients not getting access to these therapies upon FDA approval.

We are currently living in a watershed moment with emerging cell and gene therapies that correct the underlying genetic cause of disease. And while these therapies tend to be expensive on the front end, they are one-dose therapies that are negating the need for a lifetime of care after treatment. Not only are these novel therapies shifting the natural history of formerly fatal conditions, they are transforming how the reimbursement sector pays for them. Several gene therapy trials for Duchenne and many other rare diseases are underway at Nationwide Childrens Hospital. A rare disease advisory council would give Ohio the ability to better understand how the state's resources are utilized and impacted by these therapies.

We advocate for patients and caregivers all across the US; states that have already established a rare disease advisory council utilize it to help shape policymaking. As a result, we see better policy guided by experts in these states. Ultimately, state Medicaid programs and patients are better served for it.

Thank you to Rep Clites and Rep Ginter for recognizing the need for a Rare Disease Advisory Council in Ohio so patients, caregivers and disease experts have a voice in shaping the policy that impacts the 1 in 10 Ohioans living with a rare disease.