**Kelly Maynard**

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 12 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. 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.

Caring for a loved one with a disease such as this forces families to make dramatic changes, both personally and professionally. We were living in our dream home at the time he was diagnosed, but quickly realized our home wouldn’t meet his needs once he was permanently wheelchair bound. So we sold it and spent 6 months living in a tiny rental while looking for a home that would allow us to meet his needs. I was working full time for the Ohio Department of Insurance then, and quickly realized we would have to make employment changes because the level of care he needed made working outside the home impossible. With 10 years to retirement to go, I left state employment and didn’t make a salary for 1.5 years. This was a huge financial hit to my family. We needed help.

And while during this time it felt like our family was in a constant state of transition and upheaval, I realized we were the lucky ones. In the 7 years since my son’s diagnosis, I have assisted hundreds of families across this country, many of them Ohio families, address these same challenges they find themselves facing but with no resources to do so. We live 20 minutes from NCH, a center of excellence in research and care for Duchenne. We have health insurance. Jackson has a Medicaid waiver, which gives us access to home health aides (which I rely on heavily to be able to earn a paycheck), and gives us access to other critical services such as home and vehicle modifications. And, we have an amazing support system of family living within 10 minutes of us.

But what about the families who are living with this same disease 3 counties away who for some reason can’t get access to these services? What about the families whose employers don’t provide health insurance? Or the families that live in counties where there is a critical shortage of home health aides? The families who want to travel to Columbus or Cincinnati for expert care, but can’t afford a $50,000 HC accessible vehicle to transport their children? Disparities in care resulting from where you live is real. And while I don’t have all the answers for what causes these disparities, I do know the only way we’re going to find out and work on solutions is by getting the pertinent stakeholders together to discuss it.

Rare disease patients need a representative voice in health policy. These patients touch every access point of the health care system. The nature of living with a rare disease often means patients and caregivers have to become disease experts themselves. With over 7,000 rare conditions identified by the CDC, it is impossible and unreasonable to expect policymakers to be experts on all of them. For this reason, it’s critical to engage patients and expert providers in all discussions where policy and patient experience intersect. Patients and advocacy organizations should be used as a valuable resource—a FREE one—in the policymaking process. I want to highlight for you a successful example of how this can and does work if the process includes patients:

I was attending a national conference of state Medicaid leaders. I met a state Medicaid director of a state where I knew we had a family experiencing challenges getting access to a newly approved drug due to this state requiring patients to undergo muscle biopsies as part of their coverage criteria for initial authorization, and additional muscle biopsies upon renewal every 6 months. I explained to him that not only is requiring children with a progressive muscle wasting disease to undergo risky surgery that removes muscle tissue unethical, it’s a highly specialized, expensive, and unnecessary procedure. I explained to him there are other methods to determine whether a therapy is actually working for patients, which do not require surgery and paying for specialty protein analysis. He told me he would look into it, and he did. He emailed me not long after to say that his team reviewed the criteria and determined that our suggestions made sense, and that state issued a revised policy of coverage at their next DUR meeting. He thanked me for bringing this to his attention and for saving his state money in the process. His state, like most states, has a process for reviewing drugs that didn’t allow patients and expert providers to weigh in during review.

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 their daily lives.