



Written Proponent Testimony HB 130  
Kelly Maynard, President and Co-Founder  
Randi Clites, Rare Disease Policy Director  
June 7, 2023

Chair Lampton, Vice Chair Barhorst, Ranking Member Miranda and members of the House Insurance Committee, thank you for the opportunity to provide written testimony regarding House Bill 130.

Little Hercules Foundation (LHF) is a rare disease advocacy organization serving the Duchenne Muscular Dystrophy community. Duchenne Muscular Dystrophy (DMD) is a rare, progressive, muscle-wasting disease in which those diagnosed are unable to produce dystrophin, a protein essential for the repair and stability of muscle fibers. Without dystrophin, muscle cells are in a state of constant breakdown. DMD is the most common and leading fatal genetic disorder in children; approximately 1 in 5,000 male births affecting an estimated 25,000-30,000 individuals in the US. Currently there is no cure, so DMD is 100% fatal. However, we do have 5 approved treatments that slow progression of the disease, and we anticipate an approved gene therapy to treat 4-5 year olds with DMD later this month.

DMD is generally diagnosed between the ages of 3-5 when boys start showing muscle weakness and delayed gross motor skill development. As the disease progresses, boys living with DMD typically lose the ability to walk between the ages of 8-12. Once children go into a wheelchair permanently, their care needs become increasingly complex necessitating invasive surgeries such as spinal fusion to correct scoliosis and tendon release surgeries to correct joint contractures. Interventions such as oxygen therapy, bone density medications, serial casting, cardiomyopathy preventative medications—the list goes on and on—are necessary. Our testimony is to express LHF's support of HB 130, which will create a prior authorization "gold card," where providers who consistently adhere to evidence-based medicine will be exempted from certain prior authorization requirements.

While 40% of physicians have staff who work exclusively on prior authorizations (per the 2021 AMA Prior Authorization Physician Survey) it is rare in the patient advocacy community to have experts who help a patient with their provider to assist in appealing a denied prior authorization. Little Hercules works with families that receive negative coverage denials from payers for medically necessary medications, equipment, and other medical interventions such as PT, OT, and testing. Much of the time, when these denials occur, the specialty of the health plan reviewer making the final decision has no experience or knowledge of DMD. When peer-to-peer reviews occur, the prescribing expert provider is speaking with someone that is strictly following a health plan policy and has no knowledge of the disease. Access to timely care is of critical importance to rare disease patients whose disease causes major loss of function in a short period of time.

Our DMD community must fight through multiple appeal levels in order to obtain an overturned decision on external appeal with knowledgeable, peer level practitioners making a non-biased review of the facts in the case. Many times in DMD, permanent damage occurs over the time it takes for appeals to happen. It is stated that 18% of physicians reported a life-threatening event or intervention to prevent permanent impairment or damage (per the 2021 AMA Prior Authorization Physician Survey); in DMD and many other progressive, rare diseases, this means loss of muscle that will never be regained. For this reason, Little Hercules Foundation is a strong supporter of HB130.