Dear Chairman Cirino, Vice Chair Chavez, Ranking Member Hicks-Hudson and Members of the Senate Finance Committee,

Thank you for the opportunity to provide written interested party testimony on the Senate Amended HB96.

My testimony is to express my concern with the proposed amendment to abolish the Rare Disease Advisory Council (RDAC,) which I have just been re-appointed to serve my third two-year term. Please reverse this proposed amendment.

Not only do I serve on the RDAC as a caregiver, but I am also the President and Founder of Little Hercules Foundation (LHF). LHF is a registered, non-profit 501(c)(3) organization based in Dublin, Ohio. LHF got its start in January 2013 when three moms, two of whom had sons diagnosed with Duchenne Muscular Dystrophy, decided to host events to help fund research. Since then, Little Hercules Foundation has grown into much more. We focus on improving the lives of those diagnosed with Duchenne Muscular Dystrophy.

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 damaged and replaced with connective tissue. DMD is the most common and leading fatal genetic disorder in children, affecting approximately 1 in 5,000 male births with an estimated 300,000 sufferers worldwide today. Currently, there is no cure; DMD is 100% fatal.

DMD is found primarily in boys. The dystrophin gene, which regulates dystrophin production, is located on the X-chromosome. Males, who each have one X- and Y-chromosome, are at risk for X-chromosome related disorders. Duchenne may be unknowingly passed from mother to child. Women can be carriers, but not manifest any symptoms likely due to their second, compensating X-chromosome. In approximately 35% of cases, however, DMD occurs because of a random, spontaneous mutation at formation of the dystrophin gene during pregnancy. Therefore, any child is at risk for having DMD.

DMD is generally diagnosed between the ages of 3-5 when boys start showing muscle weakness and delayed development. It is then that parents or caregivers notice some early signs of DMD such as speech delay, enlarged calf muscles, and challenges with physical tasks such as running, stair-climbing, riding a bike, and balance. Because of my connection to lawmakers and the administrative staff through my RDAC membership, I believe that led to our community advocating for Ohio to become the first state to add DMD to the Newborn Screening Panel through the last budget process.

Additionally, a large Medicaid MCO issued harmful policies for children with DMD and SMA stating they were no longer covering treatments if they otherwise qualified for a currently enrolling clinical trial. Knowing that these types of changes made by MCO's need approval by Medicaid before issuing publicly, I brought it to RDAC's Medicaid representative who addressed the matter before any babies/children were permanently harmed by it. The MCO had not received permission from Medicaid to issue the policy.

While these examples don't exactly meet the main RDAC established goals, it does shows the importance of having this group of experienced advocates and policy professional meeting quarterly to make Ohio a better state for the Rare Disease Community.

Sincerely,

Kelly Maynard