Testimony in support of H.B. No. 412: Establish Rare Disease Advisory Council

An orphan disease is defined as a condition that affects fewer than 200,000 people nationwide. While rare diseases individually affect small numbers of people, collectively there are over 7,000 known rare diseases. They affect as many as 25 million Americans – making this a serious public health concern.

Rare diseases are also personal stories and family struggles. I know, because this happened to my family. My younger brother Connor became blinded and paralyzed at 22 by something called neuromyelitis optica spectrum disorder (NMO or NMOSD). It rocked my family's world. It was terrifying to watch my brother unable to move, with top-notch neurologists poking and prodding him, unsure if he would ever walk again. Ironically, I was pursuing my PhD in immunology at the time, and I became obsessed with this neurological autoimmune disease that had hit my brother. I realized I needed to become my family's personal expert on the science of the disease, but I also knew I had to better understand the system and policies that affected healthcare and research funding. All these things would now majorly determine my brother's life.

Thank god, there had been rapid interest, progress and funding into this NMOSD prior to Connor's blindness and paralysis, because he received a swift diagnosis and acute treatment. This was crucial. He was able to gain his eyesight and ability to walk back. Like many rare diseases, NMOSD is a chronic illness that leaves invisible but pernicious symptoms, including permanent nerve damage, for which there are no available treatments. NMO can also come back, and with a vengeance, at any time. To help prevent NMO from returning, Connor, like many other rare disease patients, requires long-term treatment to help reduce relapses or return of disease activity. There were no FDA-approved treatments for NMO at the time, a situation all too common for the rare disease community. Connor, closely connected to the scientific community, knew how important it was to support and contribute to ongoing research. Connor decided to participate in a clinical trial for a potentially promising NMOSD treatment. Connor is one of many rare disease patients who accepts the uncertainty but potential hope of a clinical trial to not only help his disease, but also others living with it.

My mother and I knew we needed to do SOMETHING on behalf of my brother and patients like him, so we started a non-profit named after him- The Connor B Judge Foundation. We aim to promote awareness and research funding related to NMOSD and similar autoimmune diseases. We coordinator annual fundraisers and contribute to ongoing nerve repair research, as well as work with other like-minded organizations, including the National Organization for Rare Disorders and the Ohio Rare Action Network.

Despite all the great work that's been done on behalf of rare diseases, we need more progress. We need: to help fill in the gaps in awareness; improve patient, caregiver and health care provider

education; policies that better protect and work for rare disease patients and their families; and of course, more research funding to find cures.

I write as a scientist, sister and advocate. I learned about how government polices determine healthcare coverage, potential treatment cost, treatment placement on insurance policies and research funding. For all these reasons and more, we need to have a committed ongoing relationship and dialogue with our representatives in government. In at least part to achieve this, a Rare Disease Advisory Council in the Ohio legislature is crucial.

Cordially,

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