February 29th, 2024
Health and Government Operations Committee
Chair Joseline Pena-Melnyk
Vice Chair Bonnie Cullison

Dear Chair Pena-Melnyk, Vice Chair Cullison, and Members of the Health and Government Operations Committee,

My name is Hannah Lowe, and I am writing to testify on behalf of my family's story as it relates to House Bill 676.

For families like mine who are facing rare diseases with no treatment or cure, this is an issue we encounter daily. I want to share with you the story of my son, Austin, and how our journey has led us to our own efforts to raise awareness about the urgent need to open new treatment pathways, through the Right to Try for Individualized Treatments Act, which allows patients to access investigational treatments that are individualized just for them.

Austin was born in 2019, and as far as we knew, he was a typical little baby. But when he stopped feeding and gaining weight, and was no longer hitting his developmental milestones of a 5-month old, he was admitted to a local hospital which, fortunately, is near our home.

After weeks of tests, we received the devastating news that Austin had a rare disease called L-CMD, a congenital muscular dystrophy. The disease is so rare that the hospital had never seen a single case, and, at that time, there were only 50 documented cases in the world.

There was no treatment, and there was no cure. In an instant, the life we thought we might enjoy and hopes for our baby boy were shattered. We were told to take Austin home, love him, and cherish the time we had left with him.

But we refused to give up hope. After hundreds of hours of Zoom calls, reading medical articles, meeting with medical experts and sharing conversations with families facing other rare diseases, we have discovered that there is something we can do. We are now collaborating with two research institutions to create a gene therapy for L-CMD.

The technology is there, it just needs to be mapped to the gene that commonly affects our condition. We are now in a race against time to continue to raise money and conduct the research. This is where you can help families like ours.

My home state of Maryland has some of the world's leading facilities and researchers, but the current regulatory framework for developing new medical treatments is slow and cumbersome,

which is not acceptable for families like mine who are facing rare diseases with no treatment or cure. Although it is now federal law that patients have a right to seek these, Maryland's state laws must be changed to better accommodate custom treatments for these devastating illnesses that we battle day in and day out.

We need laws that would allow medical treatments to be developed faster, with appropriate safety measures, to make it legal to seek these individualized treatments. Simply because these treatments do not reach us through the traditional FDA approval pipeline does not mean that we are any less deserving of access to them, especially when they may significantly prolong life with these conditions.

Because our disease has such a small patient population, we can't wait for clinical trials which, even if they do happen, are more than a decade away. Pharmaceutical and biotech firms are typically not interested in treatments where large-scale commercialization is unlikely, so it falls to families like ours to push research and innovation forward.

We need your help to make this Herculean task more attainable. For any of you who are parents, you would do anything for your children. That is what we are doing, too.

Our families and nonprofits are scraping together the money and making breakthroughs happen out of pure grit, determination, and love, but we need your help to put this money to use.

We are not asking for a handout. We are asking for laws that would allow medical treatments to be developed faster, with appropriate safety and efficacy measures, to give hope to families like ours.

Please help us in our fight to find a cure for Austin, other L-CMD patients, and the millions more with other rare diseases. Thank you for your consideration of this important reform.

Thank you,
Hannah Lowe

President
L-CMD Research Foundation