Parent Project Muscular Dystrophy
JOIN THE FIGHT. END DUCHENNE.

March 22, 2024

Good afternoon, Chairman Carruthers, Ranking Member Liston and the members of the subcommittee.

I am a fellow Ohioan, born and raised in Cincinnati and for the last many years, a resident of Middletown, Ohio

Newborn Screening, Duchenne muscular dystrophy. Why now?

At the age of 34, we had 4 stair-step children. We had moved into what we believed to be our 'forever' home. We moved into this neighborhood because it was within walking distance to schools, within a short distance to the hospital where my husband worked.

At the age of 34, my children were 6, 4, 2 and newborn. While seeing our pediatrician with my son, I expressed concern about my 4-year-old boy, asking my pediatrician why his motor function seemed different somehow from the girls and from the other children in our neighborhood. My pediatrician smiled and said not to worry.

I loved old houses and our 'forever home' as a 3-story rambling house. My 6-year-old was in a neighborhood school with 4 floors. My 4 year old was in a pre-K Montessori school housed in one of our city's lovely old homes, the school on the second floor.

We settled into our lives, our dreams -the old house, the neighborhood school, the pre-k Montessori. The plan was for all of our children to later attend the private high school, within a walking distance of .5 miles.

As my sons grew, my concern deepened. They had little interest in activities such as big wheel or tricycle and struggled on stairs. Doctor after doctor dismissed these worries as in 'they just need to grow up and refine their motor strength'.

Four years passed. At the age of 6, my son attempted (with encouragement) to ride a big wheel. He pushed with all his strength to move the pedals. He cried out in pain. I carried him inside the house that evening, and the following morning went to see a friend, an orthopedic surgeon who greeted me in the waiting area. He looked at my son, his eyes still red from crying and said 'this is Duchenne muscular dystrophy and I'm guessing your younger son carries this same diagnosis. And you, likely a carrier'. I had no family history.

The house, the school, the neighborhood – no longer served our family.

Only five drops of a baby's blood are needed to identify babies at risk for many serious medical conditions. Newborn screening identifies these conditions that can and will affect a child's long-term health or survival. Early detection, diagnosis, and intervention can prevent death or disability and enable children to reach their full potential.

Why now? And why in Ohio?

Because we can. The NBS screening pilot was successful and unfortunately the RUSP failed to understand the need and the urgency.

1. In Ohio, we have incredible expertise and capacity to support these babies and their families and Ohioans live within relatively short driving distances to these hospitals.

   Nationwide Childrens, ranked #9 in Best Children's Hospitals

   Cincinnati Childrens, ranked #3 in Best Children's Hospitals and #1 in Ohio

   Akron Childrens, ranked #22 Best Children's Hospitals Worldwide

2. We have 5 approved therapies for Duchenne and potentially within the next 66 days, a gene therapy approval. On October 26, the PDUFA date for yet another therapy for Duchenne.

3. Early intervention will delay, by years, loss of function and potentially prevent the need for wheelchairs, home modification, handicapped vehicles- lessening the economic and societal burden.

4. Early identification and intervention will enable families to plan for where they live, what schools their children attend, where they work and what type of insurance will be needed.

5. Newborn Screening will identify carrier females who will have the ability to consider reproductive options AND the need to care for themselves as they carry risk of heart disease and weakness.

6. Newborn Screening Saves Lives. Adding Duchenne to Ohio's Newborn Screening panel will change the lives of families and specifically will improve both the quality of life and life span on individuals diagnosed.

In closing, I urge you to add Duchenne to the State's newborn screening panel.

Thank you for listening.

Pat Furlong

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Founding President and Chief Executive Officer