Rare Disease Speech 06/28/22

Nicky Hosey

Good Morning - My name is Nicky Hosey I am 26 years old and live in Washington NC. I am the mom to twin 10-month-old girls (1 being the reason I am unable to attend today) and a four-year-old, red headed, blue eyed and outgoing little boy named Wade Hosey.

Wade has a rare disease called Spinal Muscular Atrophy. Most have never heard of this disease that is the leading genetic cause of death among infants. SMA as we call it progressively destroys motor neurons in your brain stem and spinal cord that controls

all muscular activity including speaking, walking, swallowing and even breathing.

When Wade was a year old, he was not walking like his friends. Doctors would always say “just give it time he’s a lap baby”. It wasn’t until he was 16 months that I pushed hard for another opinions. Wade was referred to a physical therapy and from there sent to a pediatric neurologist. After months of waiting for genetic panels to come back it was determined he has SMA. Mind you at that point I had never heard of this disease. If you do a quick google search of Spinal Muscular Atrophy the first thing you will see is a picture of a child who is in a wheelchair has a feeding tube, trached and vented. You scroll down and you read “most children with the disease do not live to be 2 years of age”. How was that possible my baby was 19 months at this point?

After we received an official diagnosis, we started looking at treatment options. We found a

new drug called Zolgensma. Zolgensma is a one-time gene therapy that must be giving before the age of two. Wade is missing a gene called SMN-1. This gene therapy would replace the nonworking SMN-1 gene with a new working gene. The treatment would continuously produce the SMN protein in one single IV treatment. The drug was rather new and gene therapy sounds like something from a sci-fi movie. We knew this was the treatment we wanted. I fought for my child. We started at Vidant from there went to Duke and finally ended up at Children's Hospital of the Kings Daughter in Norfolk VA until we found a doctor who would administer the drug. We fought long and hard to get Blue Cross Blue Shield to pay for Wade's treatment. After being denied we went social. We started a campaign called "Walking with Wade" We got the attention of national media outlets, lawyers, and billionaire philanthropist. I must take a moment to thank Keith Kidwell- our local representative who was instrumental in staying on top of BSBC to help

our Wade. What many do not understand is during this short 2-3 months of fighting Wade was losing muscular strength faster than we could imagine. His hands would shake - think an older person with Parkinson's disease. He could not bear any weight on his legs in fact crawling because incredibly difficult. If you were to watch him crawl his legs just laid behind him almost as a snake. The day came for Wade to get his Gene therapy at Children's Hospital. He was surrounded by his family. He watched TV played with his paw patrol toys and took a nap. One hour and thirty minutes later we drove home. Two days after his gene therapy Wade crawled on the couch by himself.

It is now two years later; Wade has no respiratory issues. Wade walks 100% unassisted. No wheelchair, no walker. He can stand up in a room unassisted. Wade goes to therapy and swim therapy every week where his therapist cannot believe how far he has come. He has mastered riding a bike and his new goal is to jump. We are very lucky to be in North Carolina which is one of the states that have added SMA to the newborn screening test. My hope is that we have made this road a little bit easier for other children with SMA.