

Good morning, Chairman Huffman, Vice Chairman Gavarone, and Ranking Member Antonio thank you for the opportunity to submit my testimony in support of SMA newborn screening.

My name is Stephanie Noll. I am a committee chair for the Ohio, Kentucky, and Indiana chapter of cure SMA. More importantly, I am the mother of a child born with spinal muscular atrophy.

You have previously heard testimony explaining exactly what SMA is and the effects of SMA. However, There's something I want you to hold onto as you hear think of these testimonies. Spinal muscular atrophy is a neuromuscular disease; it does not affect a person's cognition. It does not affect a their ability to think or to learn, it doesn't affect what makes them the unique person that they are. I can best describe it as being held hostage by your own body.

Now let me tell you about my boy!

My son Corey Tiger was born in August of 2012. After a normal and healthy pregnancy, with no family history of any genetic abnormalities, and with no complications at delivery. he was perfect. After six weeks at home I noticed what I thought was a respiratory virus, so I took him to our pediatrician. We were sent straight from there to Cincinnati children's medical center, where we spent the next 42 days. My son underwent countless tests and procedures, with no answers. It was not until Day 31 we received a diagnosis of spinal muscular atrophy type one. By this point Corey's health had deteriorated tremendously. Just three days after receiving his diagnosis, he went into respiratory distress due to a collapsed lung and was intubated and moved to the ICU. Eight days later, I had to lay in a hospital bed holding my son as he took his last breath. SMA stole my son's ability to sit, to hold my fingers, to breathe on his own, and to smile. It took two months and 20 days for SMA to steal my son's life. Spinal muscular atrophy has a rapid progression. If I would have had a diagnosis sooner, we could've implemented the interventions he needed before it was too late. A newborn screening could've made a difference in Corey's life. He would have turned five this past August I should have been hosting a birthday party. Instead, I was taking flowers to his gravesite, and baking a birthday cake, I knew no one would be eating. In 2012, nothing could have saved my son, but now we have reason to hope! The approval of spinraza in December of 2016 has changed the face of SMA. When spinraza is given early, before symptoms onset, these individuals are achieving milestones when they would otherwise be unable to do so. Some of these include the ability to sit independently, stand, or even walk! They are maintaining milestones at ages when they would be expected to lose them. Most importantly, these kids are surviving. I think of the difference this could have made in Corey's life, and the difference it will make in the lives of so many that will follow him. As you're thinking about your support of this issue I want you to remember my son. There are others like him now, and there will be others to come. Now more than ever, early detection is key.

Thank you so much for all of your time today, and I hope you see the difference that we can make together.