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Chairman Huffman
Ohio Senate
Health Committee

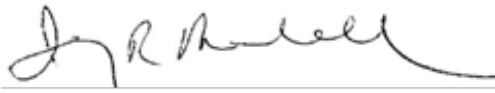
RE: Amendment adding Duchenne Muscular Dystrophy to Newborn Screening Panel

I am writing in support of an amendment hoping to make possible Newborn Screening for DMD in the State of Ohio. Newborn screening for this disease has multiple advantages that I am sure have been presented. I won't reiterate all of the advantages, but I will focus on a major breakthrough that has been evolving and is now virtually at hand. I am referring to gene therapy. On January 4, 2017, we (at Nationwide Children's Hospital) transferred a variant of the DMD gene to our first patient, a 5 years old at the time. Shortly after, we infused the gene in three others, a 4-year-old, 5-year-old and a 6-year-old. These boys are now 5 years since gene transfer and they have changed in every way and have sustained improvement. They are playing with their peers, not falling, climbing stairs rapidly and getting up from the floor without hesitation. These are the most significant findings ever occurring in DMD. To further prove efficacy and safety we have infused the gene in 80 more patients. The positive data from these patients is now at the FDA for commercial approval. The record is outstanding and the study has been sponsored by Sarepta Therapeutics.

These findings change the quality of life and the devastating natural history we have grown accustomed to in DMD. Newborn screening for DMD is important for a long-term influence on the disease. So far, we have treated boys as young as 3 years-old. If we could treat boys with DMD within the first few days or weeks after birth, we could change the disease forever. Not only would this make a difference for the boys but the financial savings avoiding chronic medical care would be an advantage to families, supportive agencies, and State resources that support disabled patients.

As a clinician scientist at Nationwide Children's Hospital, our contribution to this research has been critical. The pride we take is beyond feeling good but it represents saving the lives of children. Please let us collaborate with the State of Ohio enabling early treatment by approval of Newborn Screening for DMD.

Sincerely yours,

A handwritten signature in cursive script, appearing to read "Jerry R Mendell", is written above a horizontal line. The signature is fluid and connected, with a long horizontal stroke at the end.

Jerry R Mendell, MD