MDA's Statement on the Secretary's Advisory Committee on Heritable Disorders in Newborns and Children voting against moving the Duchenne muscular dystrophy nomination to full evidence review:

February 10th, 2023: Today, the Secretary's Advisory Committee on Heritable Disorders in Newborns and Children (ACHNDC or "the Committee") voted against moving the nomination of Duchenne muscular dystrophy for the Recommended Uniform Screening Panel (RUSP) onto the next stage of review. Rather than the nomination moving on to the "Evidence-based Review" stage, additional supporting evidence must be submitted to the Committee prior to the nomination advancing further.

MDA is disappointed by this vote as we urged the Committee to move the nomination to the next stage. We are undeterred in our efforts to add Duchenne to the RUSP.

Newborn screening is a public health program that screens nearly every newborn in the United States for certain conditions approximately 24 to 48 hours following birth. The conditions screened are serious conditions that if left untreated can have damaging impacts on the infant's health. While newborn screening is a state-run program, the Federal government recommends a "uniform screening panel" to states in which each condition meets a set of criteria related to the viability of screening, the benefits and risks of screening each newborn for the condition, and the availability of follow-up treatment and care upon diagnosis. Already spinal muscular atrophy (SMA) and Pompe disease are included on the RUSP, and most states have both disorders included in their newborn screening panels.

Duchenne muscular dystrophy was nominated in June 2022 by Parent Project Muscular Dystrophy (PPMD) with MDA as a co-sponsor of the package. Following months of initial review, today's vote by the ACHDNC signals that additional evidence is necessary to cover the areas of consideration the Committee must review when evaluating the evidence justifying newborn screening for Duchenne. We, with our partners at PPMD, will work to collect the requested evidence and data so the nomination can move forward as soon as possible.

MDA anticipates remaining active throughout the Committee's review, including testifying at quarterly meetings (just as we did this morning), and providing evidence and information to the Committee, if requested. We will continue to keep the neuromuscular disease community updated as the nomination process continues.