MDA testimony in front of the Secretary's Advisory Committee on Heritable Disorders in Newborns and Children on the Duchenne muscular dystrophy nomination – 1/29/24

Thank you for the opportunity to comment on the ongoing review of Duchenne muscular dystrophy for consideration for the Recommended Uniform Screening Panel. I am Paul Melmeyer, Vice President, Public Policy and Advocacy, at the Muscular Dystrophy Association. MDA is proud to serve the Duchenne, spinal muscular atrophy, and Pompe communities along with many other rare neuromuscular disease communities. And on a note of celebration, SMA is now screened for in all 50-states and DC, an incredible milestone for the SMA community.

First and foremost, we are very grateful for the Committee's continued full evidence review of the Duchenne nomination, particularly the work of Dr. Kemper and his team as well as the technical expert panel, on which MDA is represented. We look forward to continuing to contribute to the evaluation during the quarterly ACHDNC meetings, the TEP, and any other appropriate venue.

The treatment landscape for Duchenne is only becoming more favorable. With about six months of experience the Duchenne clinical field now has in dosing Elevydis to 4 and 5-year-olds with Duchenne, we are very pleased that while access challenges have arisen, to our knowledge, every barrier has been surmounted and each eligible boy prescribed Elevidys has successfully obtained the gene therapy.

Access challenges have included Medicaid agencies slow-walking the addition of Elevydis to their formularies, commercial plans considering Elevydis to be experimental (despite the FDA approval proving otherwise), self-insured plans carving out gene therapies in their entirety, and facilities needing to purchase the therapy and hope for reimbursement under buy-and-bill practices, thus placing a heavy financial burden on the clinics.

Nonetheless, through strong advocacy from the community, and from groups like the Little Hercules Foundation, PPMD, and MDA, each of these barriers have been overcome. In the last several weeks, we convened many of the prescribing clinicians to discuss gene therapy access challenges (among other topics) and clinicians were certainly more positive towards the access environment for these safe and effective therapies than they were previously. Moreover, within our gene therapy support groups, we hear stories of boys running for the very first after taking Elevydis.

With Agamree soon hitting the market, the potential expansion of Elevydis's label beyond 4-and-5 year olds this year, and additional therapies advancing through the pipeline, the landscape of treatments for those with Duchenne has never looked brighter. Thank you.