Paul Melmeyer's testimony at the July 29, 2026 FDA Cellular, Tissue, and Gene Therapies Advisory Committee Meeting on Deramiocel:

July 29, 2026 – "Thank you for the opportunity to speak to you today. I am Paul Melmeyer, Executive Vice President of Public Policy and Advocacy at the Muscular Dystrophy Association, and we serve all individuals with neuromuscular diseases, including Duchenne muscular dystrophy, in a variety of ways including advocating for the accelerated development of more and better therapies for the neuromuscular disease patient population. I have no financial relationships to mention.

The Muscular Dystrophy Association does not engage in product specific advocacy, and thus will not make a specific recommendation on this treatment. Instead, I will outline the regulatory approach we expect the FDA and this Advisory Committee to utilize when considering this and all rare neuromuscular disease therapies.

First, we encourage both the Agency and this Advisory Committee to consider carefully and with precision the unique experiences of the neuromuscular disease population that stands to benefit most from a prospective therapy, in this case the non-ambulatory mostly men and boys living with Duchenne. Please consider what alternatives, or lack thereof, that exist for this subpopulation, and which alternatives exist that target the same specific features of living with Duchenne. Please carefully consider what you have heard from our community members both during this Open Public Hearing and in other venues on the meaningfulness of the potential impacts of this therapy. And please ensure that you discuss the community's experiences today, and as you approach the voting questions, that you consider the aspects of the clinical trial most meaningful to the community.

Second, we reiterate the importance of flexibly evaluating whether a prospective neuromuscular disease treatment demonstrates substantial evidence of effectiveness by eschewing rigid two-trial precedents of old and instead embracing the totality of evidence presented. In FDA's Draft Guidance updated just last month, the FDA states that, quote, "all relevant clinical considerations (e.g., disease severity, unmet need, and disease rarity) should inform the extent and types of flexibilities that may be appropriate" end-quote, and continuing, quote "certain flexibilities might be more acceptable for a rare disease that is life-threatening and lacks available therapies", end-quote, which is certainly true for non-ambulatory individuals with a more advanced neuromuscular disease.

Finally, we wish to emphasize many of the FDA's own points published in its 2018 guidance titled "Duchenne Muscular Dystrophy and Related Dystrophinopathies: Developing Drugs for Treatment". The guidance states, quote "FDA encourages sponsors to use endpoints that can assess function across different stages of the disease" endquote, and goes on to mention upper-limb function specifically. The FDA also states, quote "When making regulatory decisions

regarding drugs for dystrophinopathies, FDA will consider patient and caregiver tolerance for risk and the serious and life-threatening nature of these conditions. For example, patients may be willing to tolerate substantial risk of harm if a drug might delay loss of important abilities", endquote.

An "FDA Voices" article posted last month by FDA leaders emphasized the need to listen carefully to the rare disease community, to review rare disease products flexibly, and to act consistently. We appreciate FDA's dedication to our community, and we thank you for the opportunity to testify today."