Paul Melmeyer's testimony at the FDA Cellular, Tissue, and Gene Therapies Advisory Committee Meeting on SRP-9001:

May 12, 2023 – "Thank you for the opportunity to speak to you today. I am Paul Melmeyer, 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 participate in product specific advocacy, and thus will not make a specific recommendation on this treatment. Instead, I will outline the flexible regulatory approach we expect the FDA and this Advisory Committee to utilize when considering this and all rare neuromuscular disease therapies.

First, we urge the FDA to flexibly and consistently use the accelerated approval pathway for approving rare neuromuscular disease treatments when proving clinical effectiveness in heterogeneous, often slowly progressing, neuromuscular diseases in a timely manner is not possible. Most neuromuscular diseases, including DMD, are irreversible in their progression and consequently the muscle damaged and lost while waiting for new therapeutic approvals cannot be regained upon later approval of the therapy. As Dr. Peter Marks, Director of CBER, recently stated at MDA's Clinical and Scientific Conference, "We can't be so careful about our approvals under accelerated approval that we prevent potentially lifesaving therapies from getting to market in a timely manner."

We understand some have called for more infrequent use of the accelerated approval pathway, but to do so may essentially halt all possibility of safe and effective treatments reaching some neuromuscular diseases, an absolutely unacceptable result. We urge the Agency to continue to flexibly apply the accelerated approval pathway in rare neuromuscular diseases while utilizing the authorizations pertaining to post-market confirmatory trials enacted by Congress last year.

Second, we reiterate the various ways in which substantial evidence of effectiveness can be demonstrated. FDA has elsewhere stated that "our regulations allow for regulatory flexibility to expedite the development, evaluation, and marketing of new therapies intended to treat persons with life-threatening and severely debilitating illnesses, especially where no satisfactory alternative therapy exists". FDA has demonstrated several recent examples of flexibly using the accelerated approval pathway and subsequent confirmatory trials to support approval of neuromuscular disease treatments, and we encourage the Agency to continue to do so.

Finally, the FDA has a well-established record of approving treatments for serious and life-threatening rare diseases without the traditional level of proof of effectiveness required in more common or less serious diseases. Analyses have shown that at least two-thirds of rare disease drugs are approved by the Agency flexibly considering whether the effectiveness evidence is adequate. These flexibilities have been reiterated by the three most recent PDUFA reauthorizations, and consistently supported by patients, their loved ones, the organizations that

serve them, their clinicians, and their elected officials. Thank you for the opportunity to testify today."