

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 ALS, 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 drug. 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. We are grateful the FDA mentioned exercising appropriate regulatory flexibility this morning and I encourage this Committee to remember the following three key points when evaluating this and all other neuromuscular therapies.

First, we encourage FDA and the Advisory Committee to consider all the ways of demonstrating substantial evidence of effectiveness, including through the use of one adequate and well controlled clinical investigation plus confirmatory evidence. As outlined in its December 2019 guidance, FDA states that the Agency, “will consider a number of factors when determining whether reliance on a single adequate and well-controlled clinical investigation plus confirmatory evidence is appropriate, including the seriousness of the disease, particularly where there is an unmet medical need; the size of the patient population; and whether it is ethical and practicable to conduct more than one adequate and well-controlled clinical investigation.”

Second, we remind the FDA and the Advisory Committee of flexibilities outlined in the ALS Developing Drugs for Treatment Guidance, including that the “FDA will consider patient tolerance for risk and the serious and life-threatening nature of the condition in the context of statutory requirements for safety and efficacy”, and, “FDA has long stressed the appropriateness of exercising regulatory flexibility in applying the statutory standards to drugs for serious diseases with unmet medical needs, while preserving appropriate assurance of safety and effectiveness.”

Finally, the FDA has a well-established record of approving treatments for serious and life-threatening rare diseases without the standard level of proof of effectiveness required in more common or less serious diseases. Analyses have shown that at least two-thirds of rare disease drug approvals are done so by the Agency flexibly considering whether the effectiveness evidence is adequate. These flexibilities have been reiterated by FDASIA, FDARA, and consistently supported by patients, their loved ones, the organizations that serve them, their clinicians, and their elected officials.

Developing treatments for rare neuromuscular diseases presents unique challenges that must be addressed with the previous mentioned flexibilities. Today, we are asking the FDA reviewers and this Advisory Committee to remember these flexible approaches already put forward by the Agency when evaluating this and all new potential treatments for ALS and rare neuromuscular diseases. Thank you.