MDA's Testimony at the September 28th FDA PDUFA VII Reauthorization Hearing

Good morning and good afternoon everyone, I am Paul Melmeyer with the Muscular Dystrophy Association, and we serve the over 300,000 Americans with rare neuromuscular diseases, most of whom do not have an FDA-approved treatment indicated for their disease, and even fewer of whom have a treatment that substantially alters the course of their disease.

We are here to offer strong support for the proposed PDUFA VII agreement, and will support the enactment of the agreement in Congress next year and the implementation thereafter.

There are several provisions included within the agreement that we believe will accelerate the development and approval of more and better therapies for the neuromuscular disease community.

To start where today's meeting started, we are pleased to see the influx of resource dedicated to CBER's gene and cell-based therapeutic review. MDA's number one priority for this PDUFA cycle, outlined in our August 2020 comments submitted to FDA and industry negotiators, was a surge in resources for reviewing gene therapies. Our community knows the impact these transformative therapies can have as Zolgensma, one of the first gene therapies approved by FDA, has been substantially improving the lives of children with Spinal muscular atrophy. Furthermore, with gene therapies for Duchenne muscular dystrophy, limb-girdle muscular dystrophy, Pompe disease, ALS, and more in the pipeline, any unnecessary delay in developing, reviewing, and hopefully approving these therapies must be avoided. We hope these additional resources will accomplish this goal.

Second, we called for the consistent use of regulatory flexibility across the FDA when reviewing rare neuromuscular disease therapies. We called for the expansion of Oncology Center of Excellence programs to outside of Oncology, including taking the "o" out of RTOR. We're very pleased to see the proposed creation of the split real time application review program, and see no reason why we couldn't similarly expand Project Facilitate outside of OCE as well. We hope further efforts to ensure consistent regulatory reviews across divisions are undertaken, either as part of this agreement by modernizing FDA's internal data systems, or otherwise, as a recent report highlighted the varying approaches to assessing and determining substantial evidence of effectiveness used across the Agency.

Third, we called for further innovation on rare disease clinical trials, and we are pleased to see the proposed creation of Rare Disease Endpoint Advancement Pilot program. Our community is no stranger to the use of antiquated endpoints with little connection to what is meaningful to the patient as evidenced by the continued use of the six-minute walk test, ALSFRS, impressive neuropathy scales, and more. We hope endpoints in neuromuscular disease trials will be included within the pilot, and hope that the lessons from the pilot will not be limited to the handful of development programs allowed under the pilot.

Fourth, with the advancements made in Patient-Focused Drug Development over the previous two agreements, we called for taking these efforts one step further by further facilitating the use of patient preference information and patient experience data in regulatory submissions. We are pleased to see the inclusion of guidances and public meetings aimed at this goal as with all the data collected in PFDD meetings and PFDD-instructed studies, such data can be most impactful when actually considered as part of a regulatory submission, perhaps even used as confirmatory evidence.

Fifth, and finally for today, we are very supportive of the innovations proposed for expedited review pathways, including the Breakthrough therapy pathway and the accelerated approval pathway. We were pleased to contribute to a proposal put forward by Friends of Cancer Research and other colleagues that called for certain reforms in our expedited approval pathways, including moving Beyond Breakthrough and better integrating expedited development programs with the needs of CMC in innovative treatments. We're pleased to see such considerations included in the agreement.

There is plenty more to say, including on complex innovative trial designs, real world evidence, decentralized clinical trials, and more, but to stick to our time allotment, we'll include our thoughts in our written comments. Thank you again for the time today.