June 10, 2019

Institute for Clinical and Economic Review (ICER)
Two Liberty Square
Ninth Floor
Boston, MA 02109

Submitted electronically to publiccomments@icer-review.org

Re: 2020 Update to ICER's Value Assessment Framework

To whom it may concern,

The Muscular Dystrophy Association (MDA) appreciates this opportunity to provide input to the Institute for Clinical and Economic Review (ICER) on its work to update the Value Assessment Framework, specifically regarding suggestions for improvement on the methods that the Institute uses to communicate with stakeholders in the patient community. As an organization with a mission of transforming the lives of individuals affected by neuromuscular diseases through innovations in science and innovations in care, MDA is committed to funding groundbreaking research; accelerating the development of treatments and cures; promoting early identification, diagnosis and treatment; and improving health outcomes. For more than 65 years, MDA has been on the frontlines of research for amyotrophic lateral sclerosis (ALS), spinal muscular atrophy (SMA), Duchenne muscular dystrophy (DMD), and other neuromuscular diseases.

MDA has also previously provided input to ICER on therapy reviews and believes that this exercise to implement suggestions, especially from patients and patient advocacy organizations, to improve the overall review process is an important undertaking. MDA also appreciates the supplementary publication of "*A Patient's Guide to Open Input"* offered on this topic as it signals ICER's commitment to ensuring that the voices of patients and their advocates are reflected in the incoming input that you receive in this process. Patients are the experts in the diseases that they live with in many ways, and heeding the input of patients and patient advocacy organizations is critical.

Paramount for ICER's consideration, and which goes across the three enumerated aspects of the request for information, is that ICER should work diligently at the outset, and through the review process, to understand the information pertaining to patient preference, risk/benefit frameworks, and other activities that will provide a more complete perspective of the patient experience with regard to any disorder, to the extent such materials and resources exist.

Specifically, MDA recommends that ICER undertake a process at the outset of each review whereby you work with the appropriate stakeholders including patient advocacy organizations to conduct a cursory review of information that is already available to inform ICER of the current needs and challenges that confront patients. We suggest that you look to umbrella organizations

like MDA and the National Organization for Rare Disorders (NORD) that can help ICER identify all relevant single disease patient advocacy organizations that may be able to offer valuable insights as you define the scope of your reviews. MDA also recommends that, whenever possible, you closely review all applicable materials that have been generated through the Food and Drug Administration's (FDA) Patient Focused Drug Development (PFDD) program. This program, led by the FDA's Center for Drug Evaluation and Research, captures meaningful data on patient experience and priorities. The reports, surveys, and web recordings from PFDD efforts are publicly available and contain valuable information shared directly from patients which would meaningfully inform ICER in its reviews. Through this process, ICER will likely learn of multiple disease registries that also contain insights and, in some cases, data sets that can also yield important findings. For example, MDA and many other organizations maintain robust patient registries. MDA's registry, the MOVR (neuroMuscular ObserVational Research) Data Hub, is one such example. We encourage ICER to build enough lead time into your review process in order to engage with patient advocacy organizations that maintain registries so that, whenever possible, information gathered by them can be utilized to inform your work.

With regard to QALYs and other ways to measure impact and value, as ICER acknowledges in the Value Assessment Framework, there are myriad ethical issues that must be considered when assessing and opining on value. MDA encourages you to also include patients, their advocates, and clinicians in the public meeting voting process in order to meaningfully take their perspective and experience into account.

The value of a therapy that saves the lives of patients with a deadly rare disease is more complex than simple economics and cannot be measured without meaningfully engaging the patient community. We strongly urge you to ensure that the voices of those living with the disorder you are evaluating treatment for are considered at the outset of your work, from the initial scoping process to the publication of your final reviews. MDA appreciates this opportunity to provide input on ICER's Value Assessment Framework and we thank you for your consideration of the comment we have offered. If you have any questions about the information provided herein, please contact me at advocacy@mdausa.org.

Sincerely,

Brittany Johnson Hernandez
Senior Director of Policy and Advocacy
Muscular Dystrophy Association