

KEY ISSUES WITH ICER'S REPORT ON DUCHENNE MUSCULAR DYSTROPHY



Duchenne Muscular Dystrophy (DMD) is a devastating rare, genetic disorder characterized by progressive muscle degeneration and ultimately premature death. It is essential that disease-altering treatments are developed and available to patients. The Institute for Clinical and Economic Review's (ICER) assessment of treatments for DMD falls far short of accurately capturing the value of these medicines that could offer patients treatment options.

Oversimplifies Diseases

DMD is a complex condition with a heterogeneous patient population and a spectrum of health states. ICER completely oversimplified the disease by representing it with essentially just two health states, ambulatory and non-ambulatory (they describe a model with four, but they only have transition probabilities between two). This type of dichotomization of a complex condition has been shown to lead to underestimation of outcome effects, which subsequently devalues interventions to address the condition. This faulty model has the very real implication of limiting access for DMD patients.

Relies on Flawed and Discriminatory QALYs

The use of the QALY to evaluate treatments for DMD is inappropriate. Not only is the metric discriminatory against people with disabilities, the QALY does not accurately reflect the growing body of literature concluding public preference for directing healthcare resources towards patients suffering more severe disease or for those for which there are currently few effective therapies. DMD falls into both of these categories.

Ignores Outcomes that Matter to Patients and Caregivers

ICER's decision to capture only two health states in their model explicitly omitting respiratory function and daily functional outcomes, despite patient advocate and experts recommending these outcomes be incorporated. Patients consistently engage with ICER through their public comment process to convey that their daily quality of life and ability to better complete simple daily tasks are of primary importance to them. Yet, ICER ignored this important perspective in favor of using inappropriately simplified health outcomes and the discriminatory QALY.

"ICER's assessment fails to capture the true impact of this complex and life-threatening condition and could have serious implications for patients' access to these novel treatments."

- Cure Duchenne

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