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PROJECT HERCULES: A SYSTEMATIC REVIEW OF THE CONTENT AND STRUCTURAL VALIDITY OF PROS USED TO ASSESS QUALITY OF LIFE IN DUCHENNE MUSCULAR DYSTROPHY (DMD)

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OBJECTIVES: Duchenne muscular dystrophy (DMD) is a rare genetic, progressive life-limiting paediatric neuromuscular disorder. Numerous patient-reported outcome measures (PROs) are administered to measure quality of life (QoL) in DMD, yet there has been no formal assessment of their validity. In this systematic review, we applied COSMIN criteria to evaluate the content and structural validity of PROs used to assess QoL in DMD.

METHODS: Systematic searches were conducted in Autumn/Winter 2018 across five academic databases (EMBASE, MEDLINE, CINAHL, PsycINFO, and Cochrane Library), supplemented by searches and citation tracking in Google Scholar. Full-text published articles containing evidence of content and/or structural validity of PROs assessing QoL in DMD, and/or articles on PRO development, were included. Evidence was synthesised and critically evaluated using established COSMIN criteria.

RESULTS: From an initial 1,752 records, reviewed at the title-abstract stage, 216 manuscripts were reviewed at full-text. Of these reviewed articles, 161 were excluded for not meeting the eligibility criteria (not a full-text published research article: n=87; not having at least a 75% DMD sample: n=26; not measuring QoL: n=21; not in English: n=16; not a multi-item PRO: n=5). An additional five articles were added from citation tracking, resulting in 60 eligible manuscripts. From these records, 40 PROs were identified, and 26 were evaluated using COSMIN. Evidence on content and/or structural validity was extracted from 41 articles (including 37 development papers). Most PROs demonstrated *low quality* evidence and *unsatisfactory* or *inconsistent* validity in DMD. The best performing PRO was the KIDSCREEN, with an *adequate* rating for PRO design and a *satisfactory* rating for content validity overall.

CONCLUSIONS: Evidence is lacking on content and structural validity of QoL PROs in DMD. In the absence of further work, to assess QoL in younger people with DMD, we advocate the use of the KIDSCREEN due to superior content validity.