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Enzyme replacement therapy for late-onset Pompe disease: a systematic review

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Abstract:
Pompe disease (PD) is characterized by deficiency of acid alpha-glucosidase, leading to progressive glycogen accumulation in tissues. Previous systematic review (SR) on enzyme replacement therapy (ERT) for late-onset PD (LOPD) haven't evaluated important endpoints as quality of life (QOL) and safety, creating the need for reassessing clinical outcomes. **Objective:** To evaluate evidence available on the efficacy and safety of ERT for LOPD. **Methods:** We systematically searched PubMed and Embase for prospective clinical studies published until May, 2017 evaluating ERT for LOPD. Outcomes of interest were defined a priori. Assessment of quality of evidence (QOE) was performed according to the GRADE criteria. **Results:** A total of 1172 articles were identified, 185 studies were eligible for abstract and full text reading and subsequently, 25 articles were included in our analysis. Only 4/10 endpoints analyzed had moderate or high GRADE scores. QOL was evaluated in 6/25 studies and 5/6 used the SF-36 questionnaire. Despite heterogeneous results across studies, GRADE was moderate and favors ERT. Patients under ERT showed improvement in all 12 studies evaluating 6MWT, with high QOE. Muscle strength (MS) was evaluated in 8/25 included studies and only 2/8 were unable to show improvement after ERT, also with high QOE. In 4/5 studies antibody formation was analyzed and all patients developed antibodies, without correlation with severe adverse events (AEs) and infusion-associated reactions (IARs) or treatment efficacy. Most IARs were mild to moderate in severity. **Discussion:** Our results corroborate previously published SR on ERT impact on 6MWT and show positive effect of ERT on QOL and MS. Our findings also suggest that ERT is safe in LOPD, once most AEs were mild to moderate and antibody formation did not seem to interfere with any outcome evaluated.