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Quality of life in late-onset Pompe Disease: partial results from a systematic review.

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Introduction: Pompe Disease (PD) is a deficiency of acid alpha-glucosidase, with progressive glycogen accumulation in tissues. Previous systematic reviews (SR) on late-onset PD (LOPD) haven't evaluated important endpoints for enzyme replacement therapy (ERT), thus creating the need for reassessing clinical outcomes. **Objective:** To evaluate efficacy of alpha-alglucosidase on Quality of Life (QOL) for LOPD patients. **Methods:** We systematically searched PubMed and Embase for prospective clinical studies that included 5 or more patients, published until August 2020, evaluating ERT for LOPD. Outcomes of interest were defined *a priori* including QOL. We present partial results of meta-analysis done only for QOL so far, in accordance with PRISMA guidelines. **Results:** In LOPD, a total of 1543 articles were identified and only 25 were included that evaluated outcomes of interest. QOL was evaluated in 6 studies and 5/6 used the SF-36 questionnaire. There were no significant differences in overall QOL [mean change 7.05 (95% CI 8.54, 20.78)] and in the mental component [mean change 5.37 (95% CI -4.04, 14.78)]. However, there was a significant difference in the physical component [mean change 1.96 (95% CI 0.33, 3.59)]. **Discussion:** Our results add information over previously published meta-analysis on ERT, once inclusion of observational prospective studies made it possible to evaluate through meta-analysis the impact of ERT on QOL. Our study indicates a beneficial effect of ERT on the physical component and this improvement in endurance has positive aspects in making patients less dependent on caregivers.