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Título	Efficacy and safety of enzyme replacement therapy with alglucosidase alfa for the treatment of patients with infantile Pompe Disease: a systematic review and metanalysis
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Efficacy and safety of enzyme replacement therapy with alglucosidase alfa for the treatment of patients with infantile Pompe Disease: A Systematic Review and metanalysis.

Introduction: Pompe disease is a glycogen storage disorder caused by deficient activity of acid alpha-glucosidase (GAA). We sought to review the latest available evidence on the safety and efficacy of recombinant human GAA enzyme replacement therapy for early-onset Pompe disease (EOPD). Methods: We systematically searched the MEDLINE (via PubMed), Embase, and Cochrane databases for prospective clinical studies evaluating enzyme replacement therapy for EOPD on pre-specified outcomes. Results: Of 1722 articles identified, 15 were included. Studies were heterogeneous and with very low certainty of evidence for most outcomes. Moderate/high risk of bias were present for outcome evaluated according to ROBINS-I. The following outcomes showed improvements associated with GAA alglucosidase alfa, over natural history of PD/placebo, for a mean follow-up of 48.3 months: cardiomyopathy [(mean change 131.3 g/m² ((95% confidence interval [[CI]] 81.02, 181.59)))], time to start ventilation (TSV) [(HR 0.21 (95% CI 0.12, 0.36))] and survival ([HR 0.10 (95% CI 0.05, 0.19)]). There were no differences between the pre- and post- ERT period for myocardial function and neuropsychomotor development. Conclusion: Our data suggest that alglucosidase alfa GAA enzyme replacement therapy potentially improves cardiomyopathy, TSV, and survival in EOPD patients.