OP128 Uncertainties In The Cost-Effectiveness Analysis Of Onasemnogene Abeparvovec For Spinal Muscular Atrophy Type 1

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Introduction: Nusinersen and risdiplam are available in the Brazilian Unified Health System (SUS) for the treatment of spinal muscular atrophy (SMA) type 1. Onasemnogene abeparvovec, a promising gene therapy, was approved in 2020 in Brazil. Given the high cost of this therapy and its promise of a lifetime effect, the objective of this study was to evaluate the cost effectiveness of onasemnogene abeparvovec, compared with nusinersen and risdiplam, in the treatment of SMA type 1 from the perspective of SUS in different scenarios.
Methods: A Markov model was adapted from one originally developed for the USA that considers five states of health. Short-term data were obtained from pivotal clinical trials and long-term survival curves were extracted from published reports from the USA. Maintenance of motor function milestones achieved at the end of follow up in clinical trials was considered until death. Costs and quality-adjusted life-years (QALYs) were discounted at five percent per year over a baseline lifetime time horizon. Alternative scenarios were evaluated for horizons of five and ten years, with and without a discount.
Results: Onasemnogene abeparvovec resulted in an incremental cost of BRL742,890 (USD297,156) per QALY and an increase of 3.32 QALYs in relation to the alternatives over a lifetime time horizon. In the same time horizon, but without the discount, onasemnogene abeparvovec resulted in an incremental cost-effectiveness ratio (ICER) of BRL166,539 (USD66,615) per QALY. In a five-year time horizon, considering the discount rate, the therapy resulted in an ICER of BRL12,527,667 (USD5,011,066); in ten years the ICER was BRL3,384,793 (USD1,353,917).
Conclusions: Since the benefits of onasemnogene abeparvovec mainly occur in the long term, decision makers need to consider the uncertainty of assumptions of sustained effectiveness in view of the high initial cost of the technology.

OP129 A Cost-Utility Analysis Of Denosumab (Prolia®) For Treating Osteoporosis In Postmenopausal Women: A Swiss Healthcare Payer Perspective

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Introduction: Osteoporosis is characterized by decreased bone mass and density, increasing skeletal fragility, and the risk of fragility fracture. Fragility fractures are associated with a high economic burden. Denosumab (Prolia®) is a pharmacological therapy used to treat osteoporosis and reduce the risk of fragility fracture. This study aimed to assess the cost effectiveness of denosumab, compared with other pharmacological therapies (oral bisphosphonates, intravenous [IV] ibandronate, zoledronate, raloxifene, and bazedoxifene) and no treatment, for treating postmenopausal women with osteoporosis.
Methods: A discrete event simulation model was developed using a lifetime time horizon. A Swiss healthcare payer perspective was adopted. Time-to-fracture distributions were derived from Swiss-specific Fracture Risk Assessment Tool (FRAX®) probabilities. Reductions in the risk of vertebral and nonvertebral fractures due to treatment were informed by a Bayesian network meta-analysis. Cost-effectiveness frontier analysis was utilized. Pairwise incremental cost-effectiveness ratios (ICERs) between denosumab and each comparator were also estimated. Sensitivity analyses were conducted to identify key drivers and explore the overall certainty of findings.
Results: At a hypothetical willingness-to-pay (WTP) threshold of CHF100,000 (EUR101,630), IV ibandronate was the most cost-effective therapy in women aged 60 years who had a very high risk of fracture, and in women aged 70 or 80 years of any risk level. In women aged 60 years with a lower risk level, zoledronate was the most cost-effective option. Nevertheless, ICERs from pairwise comparisons between denosumab and some comparators (no treatment, bazedoxifene, raloxifene, and/or zoledronate depending on the cohort's age and risk profile) were below the hypothetical WTP threshold. Higher intervention costs, smaller reductions in the risk of hip fracture, and shorter duration of residual benefit associated with denosumab contributed to the high ICER values seen in pairwise comparisons with oral bisphosphonates (as a class) and IV ibandronate.
Conclusions: The present evaluation supported the cost effectiveness of denosumab against some, but not all, comparators. Nevertheless, these results should be interpreted cautiously in light of uncertainty in the true effect of treatments on fracture risk.

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