Publication

Cost-effectiveness of voretigene neparvovec in the treatment of patients with inherited retinal disease with RPE65 mutation in Switzerland

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We aimed to evaluate the cost-effectiveness of voretigene neparvovec (VN) compared with standard of care (SoC) for patients with inherited retinal disease (IRD) caused by a biallelic RPE65-mutation. VN is a live, non-replicating adeno-associated virus serotype 2 (AAV2). SoC is best supportive care provided to patients with visual impairment. Patients under SoC may experience progressive vision loss leading to complete blindness., We adapted a previously published Markov cohort model for IRD. An annual cycle length, life-long time horizon, discount rate of 3% for cost and health outcomes, and Swiss health system perspective were used. Data from a randomised controlled phase III trial of VN versus SoC (ClinicalTrials.gov: NCT00999609) were used to estimate transitions between health states in the first year, after which VN patients were assumed to remain for 39 subsequent years in the health state they were in at the end of the first year. After the 40th year for VN patients and 1st year for SoC patients, visual decline was modelled based on observational data on the natural progression of the disease. Quality-adjusted life years (QALYs) were calculated based on an external study which elicited clinicians' EQ-5D-5L-based utility estimates for IRD patients with a RPE65-mutation. Costs (Swiss Francs (CHF), year 2018-2019) included drug acquisition/ administration, adverse events, testing for sufficient viable retinal cells, and healthcare-related costs of blindness. Societal costs of blindness were added in a complementary analysis. Robustness of the model results were tested in sensitivity and scenario analyses., For the base-case, VN resulted in incremental costs per patient of CHF 764'402 (VN: CHF 901'654, SoC: CHF 137'252), incremental blindness-free years of 7.67 (VN: 28.32, SoC: 20.65) and incremental QALYs of 6.73 (VN: 18.35, SoC: 11.62), leading to an incremental cost-effectiveness ratio of CHF 113'526 per QALY gained. In probabilistic sensitivity analysis, the cost-effectiveness of VN was better than CHF 100,000 per QALY gained in 41% of iterations. For the scenario analysis in which a societal perspective was adopted and for which a 50% work-related productivity loss from blindness was assumed, incremental costs of CHF 423,837 and an ICER of CHF 62'947 per QALY gained were produced. The scenario assuming VN treatment effect lasts for 20 years produced an ICER of CHF 156'171 per QALY gained, whereas assuming a life-long VN treatment effect resulted in an ICER of CHF 96'384 per QALY gained., The incremental cost-effectiveness ratio of VN compared to the SoC was estimated to be CHF 113'526 and CHF 62'947 per QALY gained, respectively, from a Swiss healthcare system, and societal perspective assuming a 50% productivity loss.

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