

COST-EFFECTIVENESS OF VORETIGENE NEPARVOVEC FOR PRO29 VISION LOSS DUE TO BIALLELIC RPE65-MEDIATED INHERITED RETINAL DISEASE IN A DANISH SETTING

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Objectives

RPE65-mediated inherited retinal dystrophy is a rare, progressive disease, which until now has had no pharmacological treatment options. Patients experience decreasing functional vision often from childhood and half of all patients are legally blind by age 18. This study evaluates the cost-effectiveness of voretigene neparvovec, a gene therapy which is the first treatment option ever developed, in a Danish setting.

Methods

A multi-state Markov model with annual cycles was developed to describe the disease progression with and without treatment respectively. The model, which is focused on the healthcare sector perspective, follows patients from 15 years of age until death (using Danish mortality statistics), with the assumption that the drug works for 40 years. Discount rates in Denmark follow national guidance in which the rate is 4% in the beginning but lower over time. The average rate, weighted by survivors in the model, ended up at 3,5% in the base case. Inspired by earlier NICE guidance¹, sensitivity analyses were performed also on lower discount rates given the upfront nature of payments. Utility values are based on EQ-5D-5L, with sensitivity analyses on two other sets of utility values.

In the model (see figure 1), patients can transition through the different health states based on the worse of either Visual Acuity or Visual Field, with death as an option from all other states. Costs were based on list prices of drugs and either DRG costs or prices derived from a Danish eye care hospital clinic for cost of treatments. In line with Danish convention, the model focused on the perspective of a healthcare payer only and thus, social costs were not included. The expected willingness-to-pay is 1 million DKK due to this being an orphan disease.

Results

In the base case, the ICER lands at DKK 834,241 with a QALY gain of 6.27 (see figures 3 and 4). The results (see figure 2) are mostly affected by choice of discount rate and assumptions regarding the duration of treatment effect. It is particularly worth noting that a lower discount rate of 1.5% almost halves the ICER, to DKK 447,034 with a QALY gain of 11.7. The undiscounted QALY gain is 20.1.

Discussion

Healthcare costs in a municipal setting

It is worth noting that the cost-effectiveness model does not include costs for care and treatment in case of blindness itself. Once blind, costs would for the most part be limited to cost of care in a municipal setting and data on this part of the burden of blindness do not exist for a Danish setting. It could however be argued that being able to include such costs may have a positive impact on the ICER.

Societal costs

The Danish HTA authorities do not as a rule look at societal costs. This model has therefore not attempted to quantify any such costs. However, a recent study² of the cost of blindness did show that a majority of people with blindness or severe visual impairment (but no other handicaps) were more likely than not to

Figure 1: Markov Model Structure

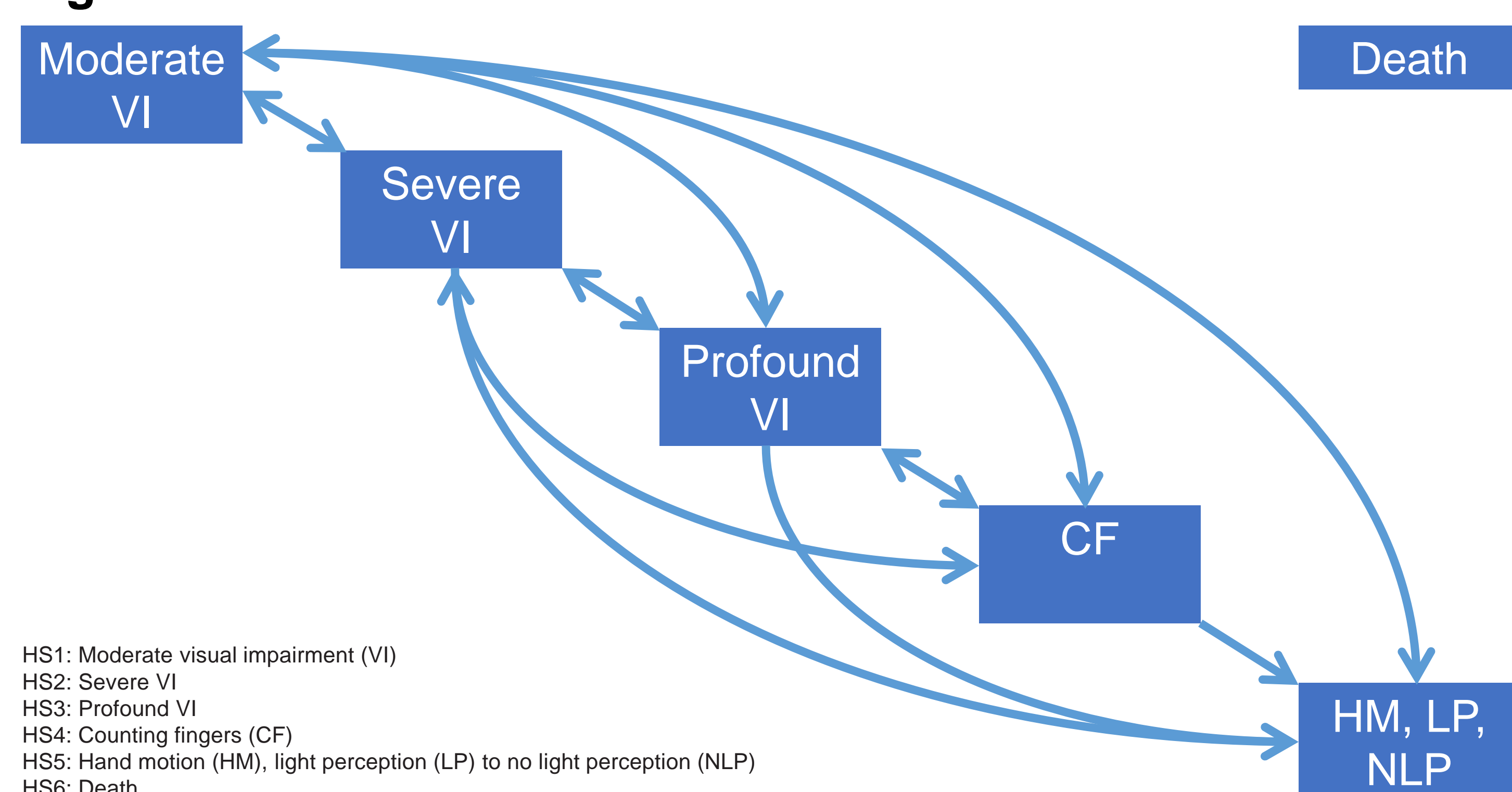


Figure 2: Sensitivity Analysis

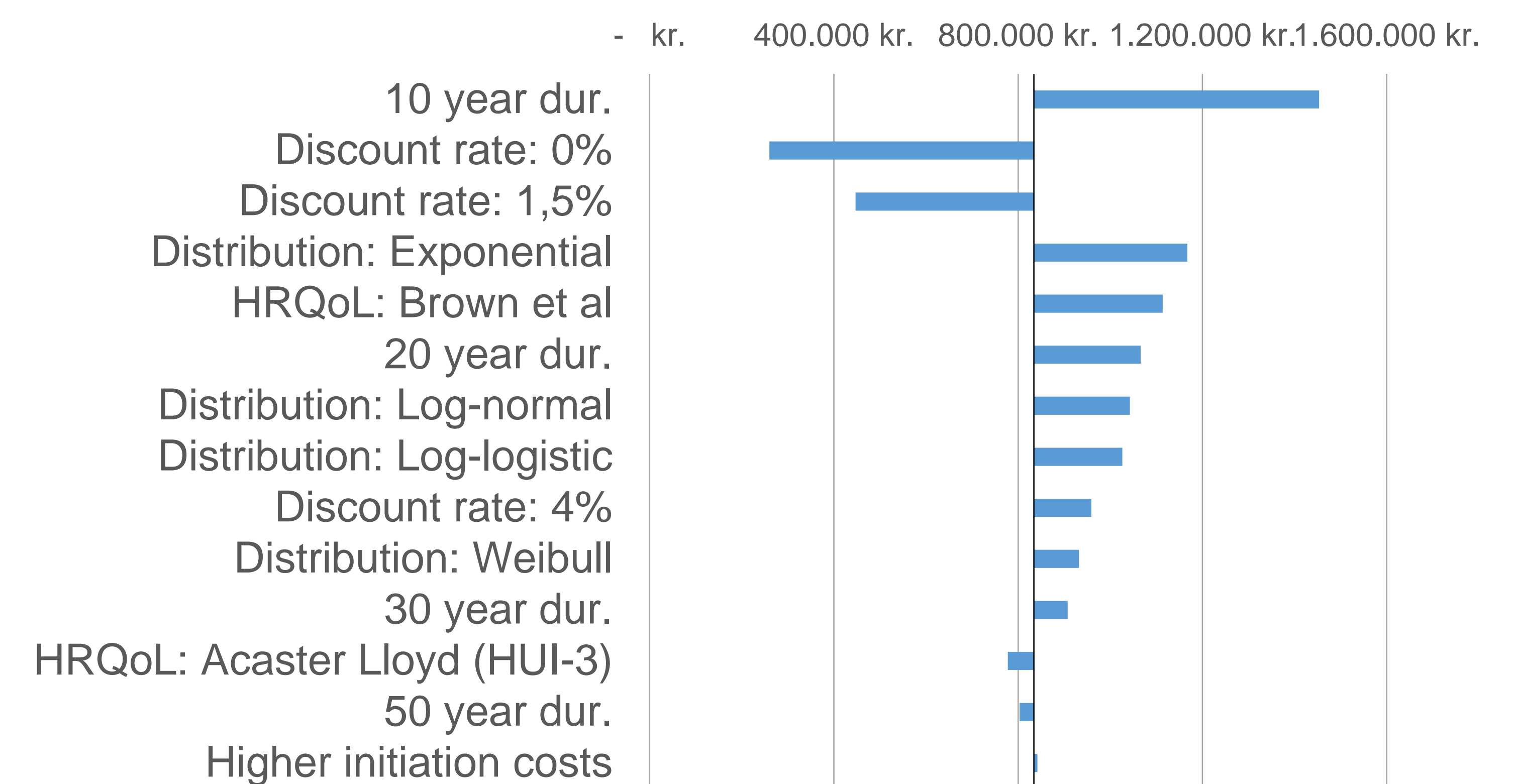


Figure 3: Cost-effectiveness plane

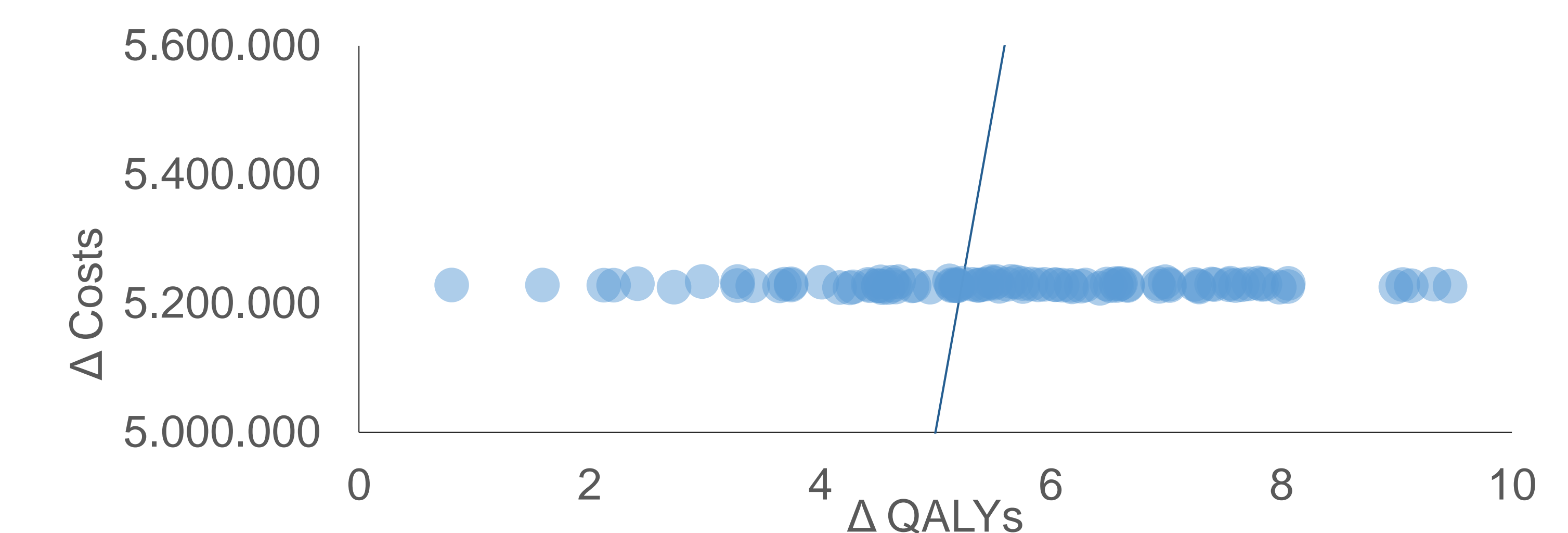
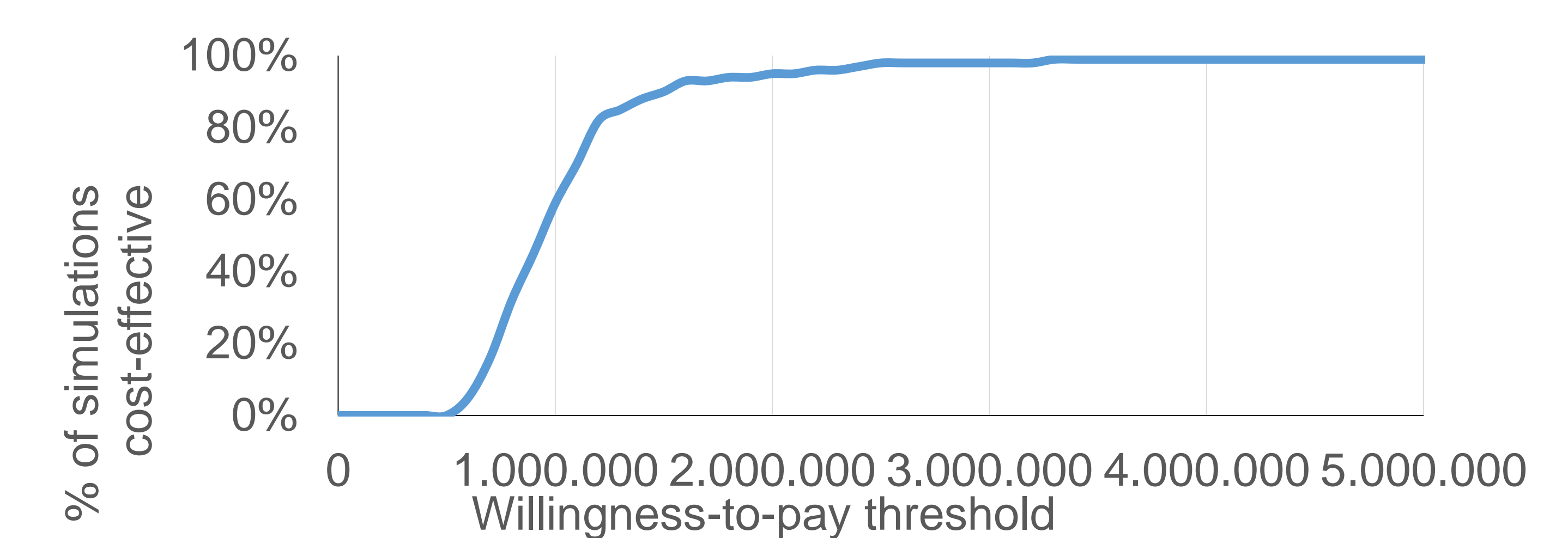


Figure 4: Cost-Effectiveness Acceptability Curve



be on transfer incomes whereas a similar cohort without any handicap at all would be in a paying job. In addition, likelihood of getting a university degree was lower in people who were blind or suffered from severe visual impairment.

Conclusion

With an assumed ICER threshold of DKK 1,000,000 for orphan indications, voretigene neparvovec is cost-effective in a Danish healthcare payer setting.

References

- NICE. *Discounting of health benefits in special circumstances*. July 2011.
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Disclosures

UJP is an employee of Novartis.

Poster presented at ISPOR Europe 2019 (Copenhagen, Denmark, November 4-6 2019)

This study was sponsored by Novartis Healthcare A/S, Copenhagen, Denmark