

Accounting for caregiver quality of life in cost-effectiveness analyses of rare genetic disease of obesity – a case study in the Swedish context

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“It mostly affected me emotionally because I constantly felt like I was failing. It was like a no-win situation because if I did not give her extra food, then I felt terrible for denying her that when I knew she felt like she was starving, and if I did give it to her, I felt like I was slowly killing her and causing her health problems. So yes, it mostly affected me emotionally because no matter what, I felt like I was failing.”
(RGDO caregiver)

Background

- In a recent report by the Swedish health technology assessment agency (TLV)[1], the possibility of including caregiver’s quality of life (QoL) in health economic evaluations was discussed
- The following arguments were given for the inclusion of caregiver utilities
 - A patient’s condition has a substantial impact on the caregiver’s daily life
 - There is reliable evidence showing that treatment leads to QoL improvements for the caregiver.
- TLV also noted that it may be particularly relevant for chronic conditions for which the burden on informal caregivers is highest .

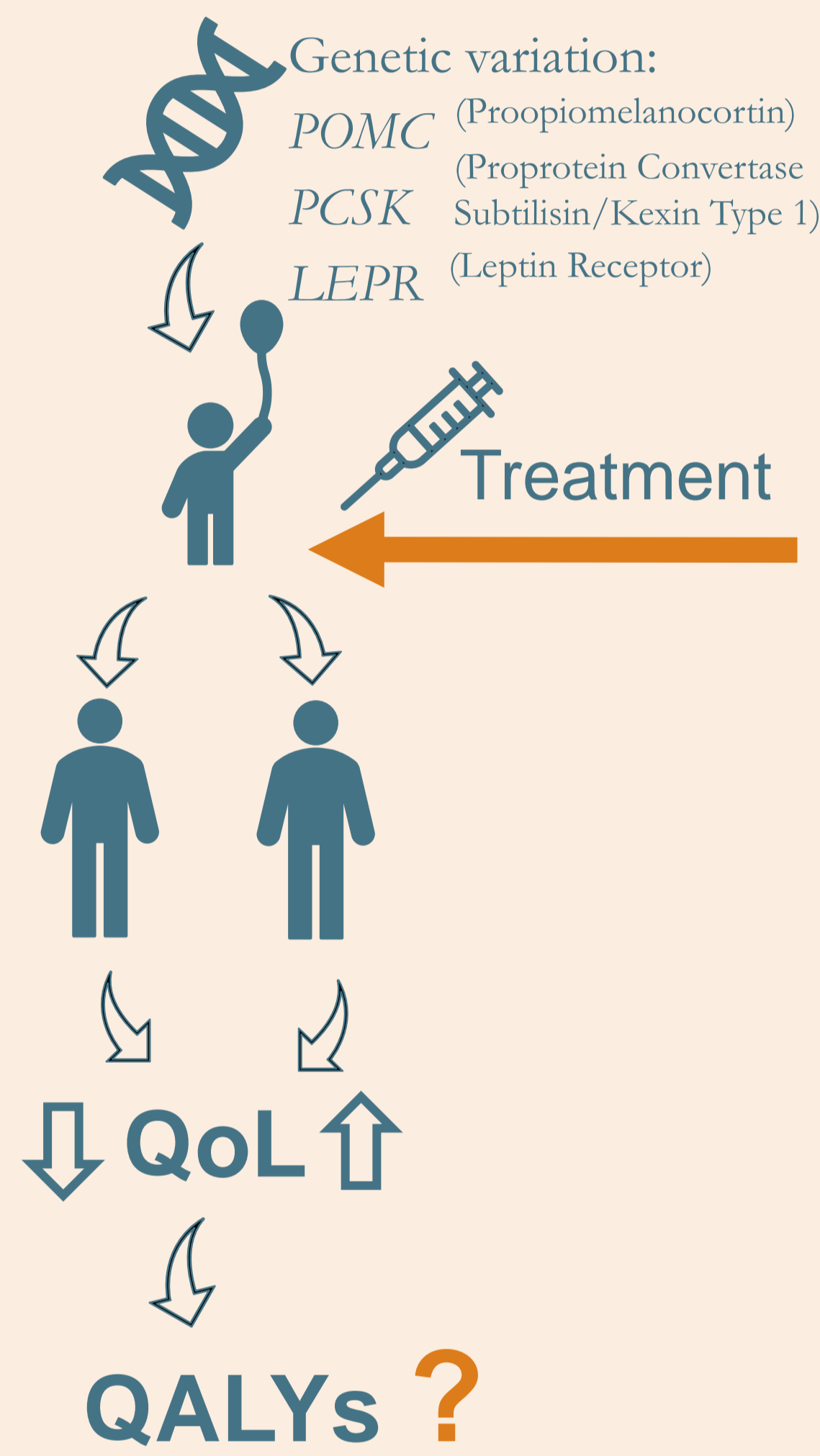
Rare genetic diseases of obesity (RGDOs)

Symptom burden in children: pathological insatiable hunger (hyperphagia) and early-onset obesity

Burden on caregivers: need for food monitoring, and consequences on family dynamics and psychological well-being

Degree of burden on caregivers may affect their QoL

Caregivers QoL may affect the incremental cost-effectiveness ratio (ICER) of treatment



Aim of study: To demonstrate the impact of caregiver QoL on the cost effectiveness of a treatment for children with rare genetic diseases of obesity (RGDOs) in a Swedish context

Methods

- A Markov model was developed to assess the cost-effectiveness of setmelanotide, a selective MC4R agonist, with model health states were based on BMI/BMI-Z-score for adult and paediatric patients.
- Costs and utilities associated with obesity and obesity-related comorbidities were included.
- Utilities associated with severity of hyperphagia reflected the direct impact of treatment on hyperphagia and subsequent QoL. Utility decrements were not allowed to go beyond 0.
- Caregiver QoL decrements were related to hyperphagia severity and based on literature [2]. It was assumed that while on treatment, caregivers would not experience any decrease in QoL, and thus utility decrements were applied to patients not responding to treatment and to standard of care.

Formula 1: Total QALY=Max(QoL_{patient}-QoL caregiver decrement;0)

- The methods used to implement QoL decrement in the model were aligned with TLV’s discussed approaches and previous NICE submissions [1,2,3].
- Costs and utility values were adjusted to reflect the Swedish health care
- A scenario analysis was conducted to take into account that for the conditions included in this analysis, more than one caregiver may be affected. This has also been discussed by HTA agencies previously [1,4].

Acknowledgements

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Results

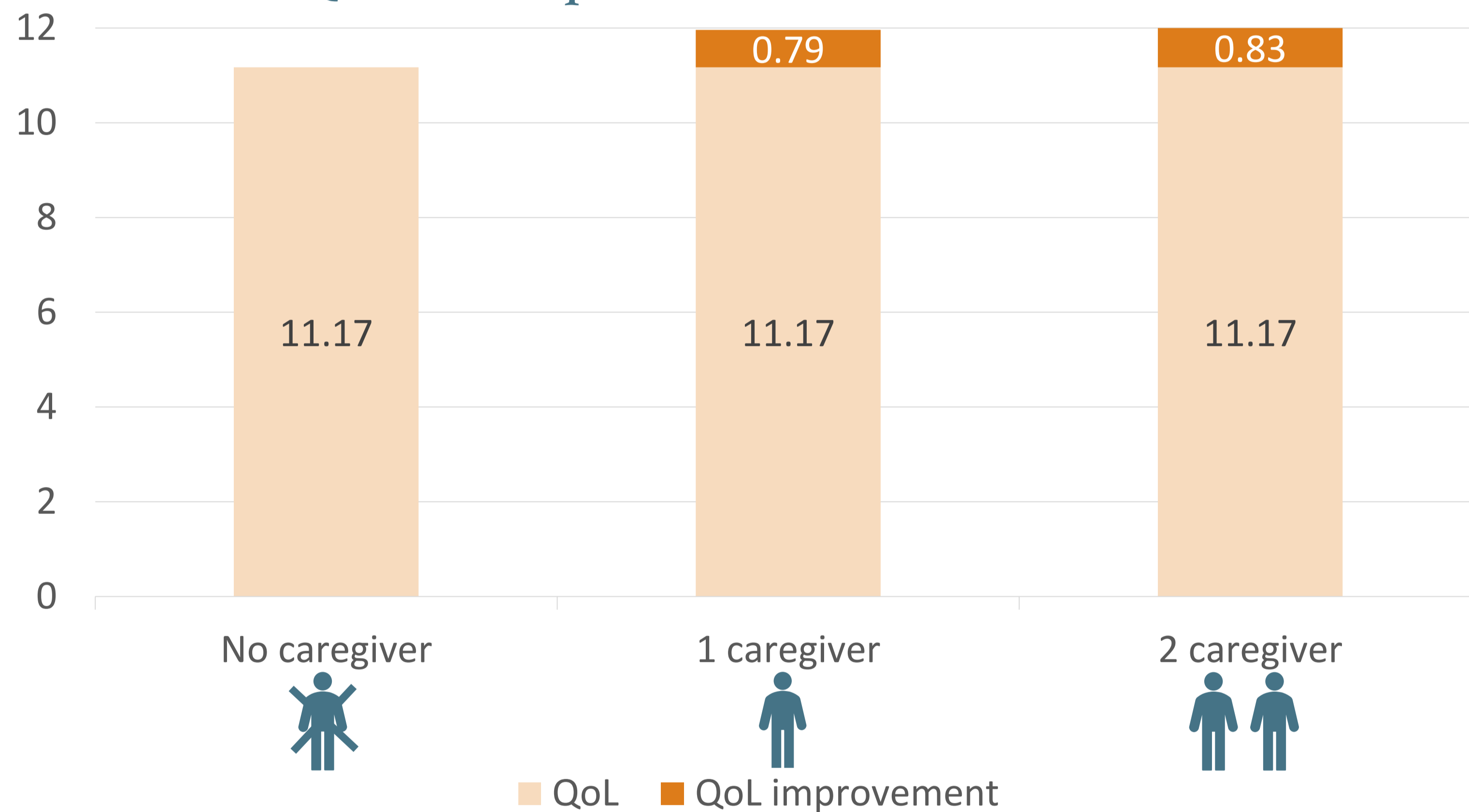
Impact of including caregiver burden on quality-adjusted life years and cost-effectiveness

	Total QALYs - treatment	Total QALYs – comparator	Incremental QALYs	Effect on ICER
Base case (no caregiver)	12.66	1.49	11.17	–
1 caregiver	12.00	0.04	11.96 ↑	- 6.6% ↓
2 caregivers	12.00	0.00	12.00 ↑	-6.9% ↓

ICER, incremental cost-effectiveness ratio

- In the base case, treatment increased total QALYs eight-fold.
- The improvement in incremental QALYs associated with treatment was greater when a caregiver’s QoL was considered vs for a patient where caregiver burden was not considered.
- A corresponding decrease in the ICER was observed.
- The impact of adding two caregivers resulted in a smaller additional increase in incremental QALYs and a corresponding smaller reduction in ICER vs a scenario with a single caregiver.

Incremental QALYs compared to base case



- The total number of QALYs was restricted in the calculations to not go below zero. This led to a difference in incremental QALYs between one and two caregivers to be minimal.

Conclusion

- This case study demonstrates the importance of considering caregiver utility in economic evaluations, especially in conditions like RGDOs that manifest in early childhood and impose substantial caregiver burden.
- Including caregivers QoL in ICER estimations increased total QALYs, leading to a decrease of the ICER.
- Previously, the appropriate number of caregivers has been debated. In this analysis, the number of caregivers did not have a large impact on the results. This may have been due to the restriction that QALYs cannot be lower than 0. Allowing for negative total QALYs would lead to an additional decrease in ICER.
- HTA agencies should continue to develop guidance to facilitate methodological rigour in the area of impact of caregiver QoL on cost-effectiveness of medications.

References

1. Swedish Dental and Pharmaceutical Benefits Agency. DN: 01868/2021. 2022.; 2. National Institute for Health and Care Excellence. HST14. 2021.; 3. Ara and Brazier. Value Health. 2010;13(5):509-18; 4. National Institute for Health and Care Excellence. TA808. 2022