How is carer quality of life included in health technology assessment globally? A review of guidance, with case studies and recommendations

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Objectives

- Including health outcomes for carers – as well as patients – in economic evaluations can change the findings of the health economic analysis.
- Whilst in many disease areas there can be clear justification for including carers' health-related quality of life (HRQL), this is not consistently done across economic evaluations
- Previous research demonstrated that including carers' HRQL is relatively uncommon in health technology assessments (HTA) in England (Pennington, 2020).
- Our aim was to review guidance and case studies from international HTA bodies, to understand how carers' HRQL is considered in economic evaluations globally.
- Given the rarity and inconsistencies in the inclusion of carers in HTA, we also aimed to develop recommendations to improve transparency and methods

Methods

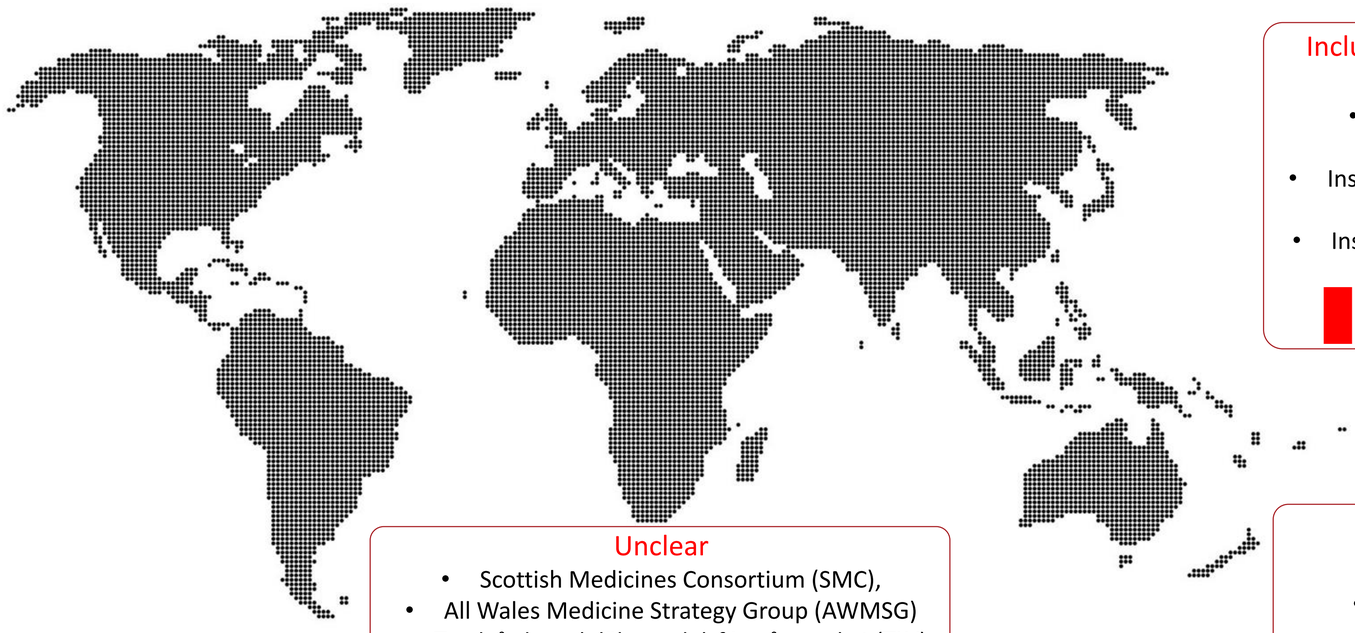
We reviewed methods guides, from 13 HTA bodies for direction regarding carers' HRQL

We selected five interventions as case studies:

- Elosulfase alfa for mucopolysaccharidosis type IV
- Ocrelizumab for (relapsing remitting) multiple sclerosis
- Nusinersen for spinal muscular atrophy
- Patisiran for hereditary transthyretin amyloidosis
- Voretigene neparvovec for retinal disease

For each case study, we extracted information on whether carers' HRQL was included by the manufacturers and/or assessors. We reviewed the methods used, and the impact on the results (where publicly available).

Figure 1: Global guidance



Include carer outcomes in reference case (where relevant)
- National Institute for Health and Care Excellence (NICE)
- Haute Autorité de Santé (HAS)

Inclusion of carer outcomes depends on other factors
- Canadian Agency for Drugs and Technologies in Health (CADTH)
- Institute for Clinical and Economic Reviews (ICER)
- Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen (IQWIG)

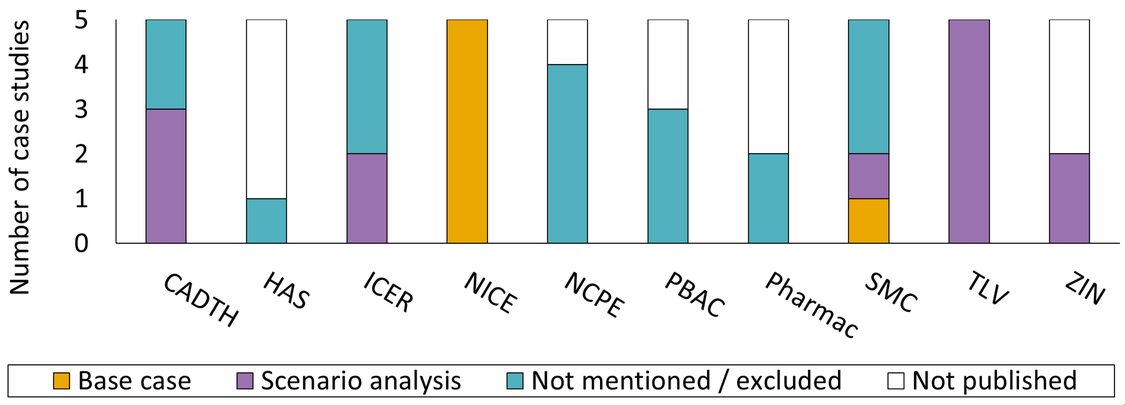
Include outcomes for all individuals in reference case
- Health Information and Quality Authority (HIQA)
- Zorginstituut Nederlands (ZiN)

Unclear
- Scottish Medicines Consortium (SMC),
- All Wales Medicine Strategy Group (AWMSG)
- Tandvårds- och läkemedelsförmånsverket (TLV)
- Agenzia Italiana Del Farmaco (AIFA)

Exclude carer outcomes in reference case
- Pharmaceutical Benefits Advisory Committee (PBAC)
- Pharmac

Inclusion of carer quality of life in case studies

Figure 2: Number of case studies where carers' HRQL was included in analysis



Number of carers

The number of carers included varied between 1 and 3 across interventions, HTA bodies, disease stage and patient population. For example, in the assessment of voretigene neparvovec , the company included 1 carer for children and 0.5 for adults in their NICE submission, but the Evidence Review Group (ERG) preferred to assume 1.78 carers for children and 0 for adults.

Sources of carers' HRQL values: Limited sources applied over many diseases

Gani et al, 2008:
Economic model included carer disutilities by Multiple Sclerosis patient health states
Refers to Neumann et al, 1999

Kuhlthau et al, 2010:
US study reporting EQ-5D for parents of children with and without activity limitations

Al-Janabi et al, 2016:
UK study reporting EQ-5D for family members of meningitis survivors exposed to and not exposed to after-effects

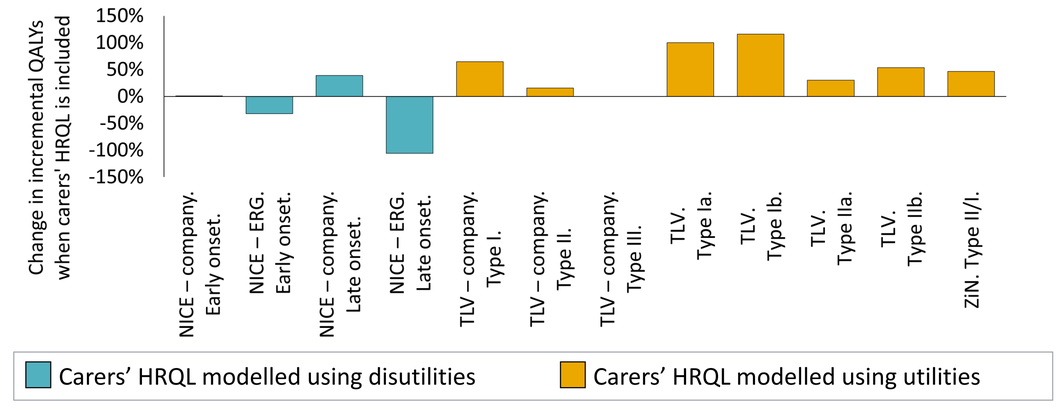
Lopez-Bastida et al, 2017:
Spanish study reporting utility for carers of patients with SMA (overall, and for type II)

- Ocrelizumab ← Gani et al, 2008; Kuhlthau et al, 2010; Lopez-Bastida et al, 2017
- Patisiran ← Gani et al, 2008; Kuhlthau et al, 2010; Lopez-Bastida et al, 2017
- Elosulfase alfa ← Gani et al, 2008; Kuhlthau et al, 2010; Lopez-Bastida et al, 2017
- Voretigene neparvovec ← Gani et al, 2008; Kuhlthau et al, 2010; Al-Janabi et al, 2016; Lopez-Bastida et al, 2017
- Nusinersen ← Lopez-Bastida et al, 2017

Impact of including carers on incremental QALYs

- Including carer QALYs in addition to patient QALYs increased the incremental QALY gain in all of the analyses for elosulfase alfa, patisiran and voretigene neparvovec, by up to 22%. The nusinersen case study is shown in Figure 3.
- The variation in magnitude and direction impacted by the modelling methodology.
- Some case studies included a carer "disutility", akin to modelling an adverse event which has a negative impact on the patient's QALYs
  - This disutility reduces when the patient's health status improves, thus increasing the total QALYs for the intervention arm.
  - However, this disutility may also reduce when the patient dies, leading to an reduction in incremental QALYs where an intervention improves survival. This is the case for the nusinersen examples where including carers decreased the incremental QALY gain.
    - Including a disutility for bereavement (as in the NICE company model) can counteract this.
- The alternative modelling method was to include carer utilities, for as long as the patient was alive.
  - In this case, increasing the patients' health status increases the total number of QALYs (as the carers' HRQL also improves). Extending the patients' life includes carer impacts for longer, and thus a increases the total number of QALYs

Figure 3: Percentage change in incremental QALYs when carers are included as well as patients – nusinersen case studies



Recommendations:

1. Clearly justify why carers' HRQL is included
2. Use HRQL data from the population under comparison (where possible)
3. Clearly justify the use of data from another disease area(s) or country(ies) (and address transferability/applicability)
4. Acknowledge limitations of cross-sectional data (a widely used source)
5. Be explicit about the assumptions and implications of the modelling approach
6. Present disaggregated and aggregated results for patients and carers.

References

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