What role does caregiver burden play in health technology assessment bodies' appraisals of highly specialized technologies?

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Background and Objectives

Caregiver burden (CB) describes the impact on an individual's daily life of providing care for a friend, spouse, parent, offspring or other relative with a chronic disease (including rare and ultra-rare)[1] and, while significant, it may be overlooked when considering the total burden of such diseases[2]. CB is highly varied and can have emotional, social, health and financial implications[3,4]. Despite its significance, CB in rare diseases is researched less often than direct patient burden[4].

There are numerous methods of assessing CB, ranging from validated instruments to measuring costs through resource and service use[5]. However, the nature of CB can vary substantially based on the disease and the extent of caregiving[2]. There is also limited guidance on suitable methods to measure types of CB such as utilities in health technology assessments (HTAs)[2]. Furthermore, CB is assessed differently across HTA bodies. NICE allows for inclusion of caregivers' health-related quality of life and utility in economic evaluations for HTAs and highly specialized technology (HST) assessments[6], although utility values can be rejected if not robustly estimated[7,8]. PBAC and SMC HTAs only include caregiver utility as supplementary information in scenario analyses.

This study aimed to review whether and how CB has been considered within HTAs of drugs in ultra-rare indications.

Methods

A review was undertaken of all NICE (England and Wales) HST assessments for ultra-rare diseases, and the corresponding CADTH (Canada), G-BA (Germany), HAS (France), PBAC (Australia) and SMC (Scotland) assessments, initiated up until June 2020. Incomplete assessments that had sufficient evidence published to date were also reviewed.

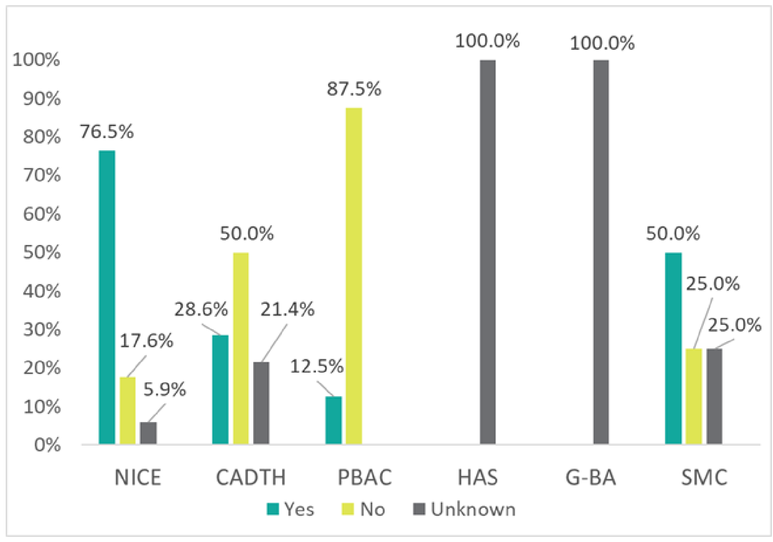
The review examined:

- whether CB was mentioned in the HTA documents;
- whether CB data were included in the manufacturer's submission;
- the type of evidence included in the manufacturer's submission;
- whether it was accepted by the HTA body; and
- whether it was a key consideration in decision-making.

Results

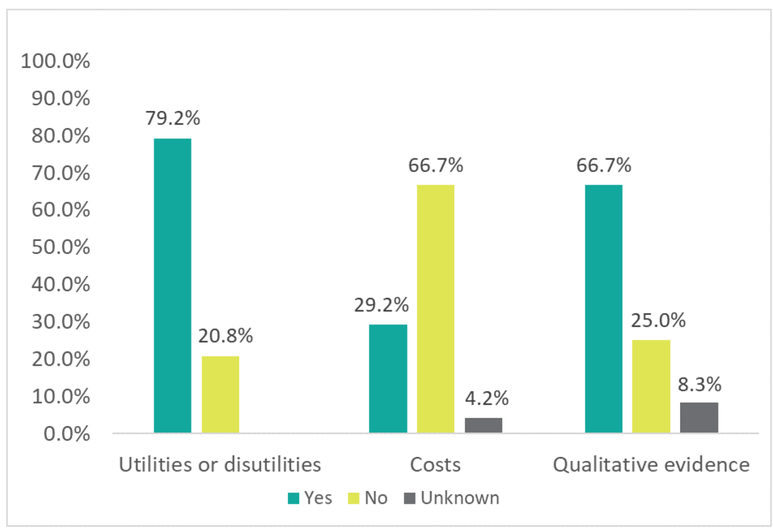
81 HTAs were identified across 6 HTA bodies, covering 21 treatments for ultra-rare diseases[note]. Across all 6 HTA bodies, CB was included in the manufacturer submission in 30% of HTAs. As demonstrated in Figure 1, CB data is more commonly included in manufacturers' submissions in NICE HTAs, while very few manufacturers' submissions in PBAC HTAs include CB data.

Figure 1: The proportion of HTAs that included CB evidence in manufacturers' submissions, compared by HTA body



Where CB evidence was included in manufacturers' submissions, caregiver utilities or disutilities from primary and secondary research were included in 79% of HTAs. Qualitative evidence from cross-sectional surveys and burden studies was included in 67% of HTAs and captured impacts on mental health, finances and/or productivity loss. Caregiver costs were submitted as evidence in 29% of submissions and captured as productivity losses, transportation for hospital visits and home adaptations. Both costs and utilities were included in manufacturers' economic models.

Figure 2: Comparison of the different types of CB evidence submitted in manufacturers' submissions



When included in manufacturers' submissions, CB was a key consideration in HTA bodies' decision-making process (92% of submissions), indicated by a critique of inclusion/exclusion of CB evidence or highlighting CB in their decision-making frameworks.

CB evidence was considered 'rejected' if it was challenged by the HTA body, and was 'accepted' if it was acknowledged in the HTA without critique. Submitted CB evidence was accepted in 75% and rejected in 25% of assessments.

Discussion and Conclusions

CB was included in about half of ultra-rare disease HTAs, most commonly as utility and disutility values. Our review included 17 NICE HST assessments and identified twice as many submissions evaluating caregiver disutilities than a 2018 review of 13 HST assessments[9] despite adopting similar methods.

Quantitative evidence is evaluated differently by HTA bodies in ultra-rare indications, due to the low patient numbers. Further reviews on this topic could comment on the role of CB in rare disease HTAs by different bodies. A 2020 review of NICE submissions reported that 50% of HST assessments and 3% of HTAs included caregiver health-related quality of life in a cost-utility analysis[10]. A review of NICE HTA submissions published in 2018 also reported that CB disutility values were more commonly included in economic evaluations in HST submissions than HTAs[11].

This review also highlights a lack of HTAs that quantify CB through costs. Some literature highlights the difficulty of valuing the cost of time spent caring[2]. Furthermore, this review indicates that qualitative evidence is also commonly submitted to HTA bodies; NICE considers qualitative evidence about the caregiver experience when quantitative data cannot be robustly estimated[12,13].

Where included, CB appears to be a key consideration in HTA bodies' decision-making process. However, this was not explicitly stated in HTA decisions and is therefore subject to interpretation.

This review was limited by not having access to full manufacturers' submissions for the SMC and HAS, meaning it could not be ascertained whether CB was included. Similarly, inclusion of CB in full G-BA submissions was assessed using basic language translations, as full submissions were not available in English. It is possible that elements of CB discussions were missed during the translation process.

Notes

A comprehensive list of the assessments of treatments for ultra-rare diseases is available upon request.

References

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