HTA Agencies' Expectations for Caregiver Quality of Life Considerations in CEA: A Focus on Pediatric Indications

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INTRODUCTION

- A caregiver is a patient's close relative – such as a parent – who willingly provides needed assistance. Being a caregiver entails time, financial and emotional burdens. Numerous studies have shown a decline in Health- Related Quality of Life (HRQoL) for caregivers, especially when the patient is a child.

- Given the focus on pediatric diseases, there is a clear rationale to support the inclusion of caregiver HRQoL in Health Technology Assessments (HTA).

- However, HTA agencies have differing opinions on this inclusion in their Cost Effectiveness Analyses (CEAs): some are in favor, some are against, and some remain unclear. NICE seems to discuss and considers the most caregivers' QoL.[1]

- Despite these discussions, there is a lack of methodological guidance to consider caregiver HRQoL into CEAs.

- This study highlights the methodological expectations of HTA agencies with the objective of providing guidance for potential future submission, based on pediatric case studies.

METHODS

- A systematic review of NICE appraisals for pediatric indications that included caregiver utility in their CEA between January 2019 and May 2023 was performed.

- European (HAS, ZiN, TLV), American (CADTH, ICER) and Australia (PBAC) HTA appraisals for the same indication were then identified and analyzed.

- The data collected from these assessments were intended to answer the following questions :
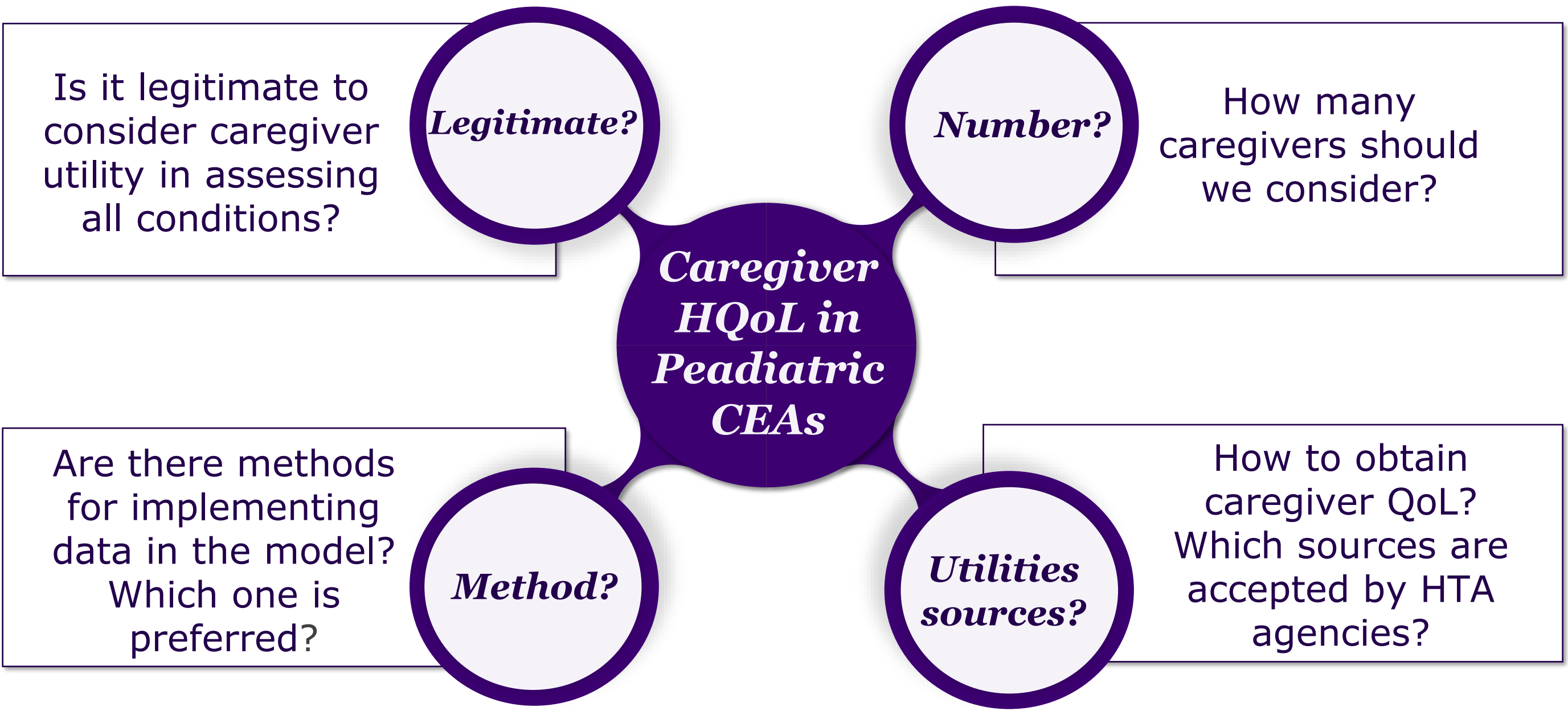
RESULTS

IDENTIFICATION OF NICE AND OTHER HTA APPRAISALS FOR PEDIATRIC INDICATIONS THAT CONSIDERED CAREGIVER HRQOL

- Between January 2019 and May 2023, caregiver consideration was found in **16 of 36 NICE appraisals for pediatric indications**.

- Of the 16 indications assessed by NICE, **7** have also been assessed by at least one HTA agency with caregiver utility data considered or discussed. The 7 indications identified are detailed in **Table 1.**

COMPARISON BETWEEN NICE AND OTHER HTA APPRAISALS

- A comparison of the **assumption regarding caregiver consideration** in the CEA between NICE and other HTAs is materialized thanks to a color coding in **Table 1**.

- While NICE's assessment considered caregivers, other agencies do not systematically consider them.

Table 1. A comparison of the assumption regarding the caregiver consideration in the CEA

Indication	Name	HAS	ZiN	TLV	CADTH	ICER	PBAC
Dravet Syndrome	EPIDIOLEX®	🟢	🔵	🔵	-	-	🟠
Lennox-Gastaut Syndrome	EPIDIOLEX®	🟢	🔵	-	-	-	🟠
Batten disease	BRINEURA®	-	-	-	🟠	-	🔵
Malabsorption syndrome	REVESTIVE®	-	🔵	-	🟠	-	🟠
Spinal Muscular Atrophy	SPINRAZA®	🔵	🟠	🔴	🔴	*Disscussion*	
	ZOLGENSMA®	🔵	🔵	-	-		-
	EVRYSDI®	🔵	🔵	⭕	🔴	-	🟠

🟢 Caregiver utility added in the base case - Similar assumption to NICE
⭕ Caregiver utility added in the base case – Assumption not described
🟠 Caregiver utility added in a scenario - Similar assumption to NICE
🔴 Caregiver utility added in a scenario – Different assumption to NICE
🔵 No caregiver part of the assessment
- No HTA assessment available

HTA AGENCIES' EXPECTATIONS FOR CAREGIVER HRQoL CONSIDERATION IN CEA FOR PEDIATRIC INDICATIONS

- If explicitly stated, HTA agencies will follow their position on the consideration – or not - of caregiver utility in CEA – base case or scenario.

- ZiN, TLV, CADTH, and PBAC opinions on caregiver assumption **lack transparency**, while NICE, HAS, and ICER are more detailed and assertive.

- There is **no clear consistency** among these assessments. The majority consider caregivers only in scenarios. However, regarding the methodological assumptions, there is a tendency to align with NICE.

- The figure below shows key data from 16 NICE and 14 other HTA appraisals:
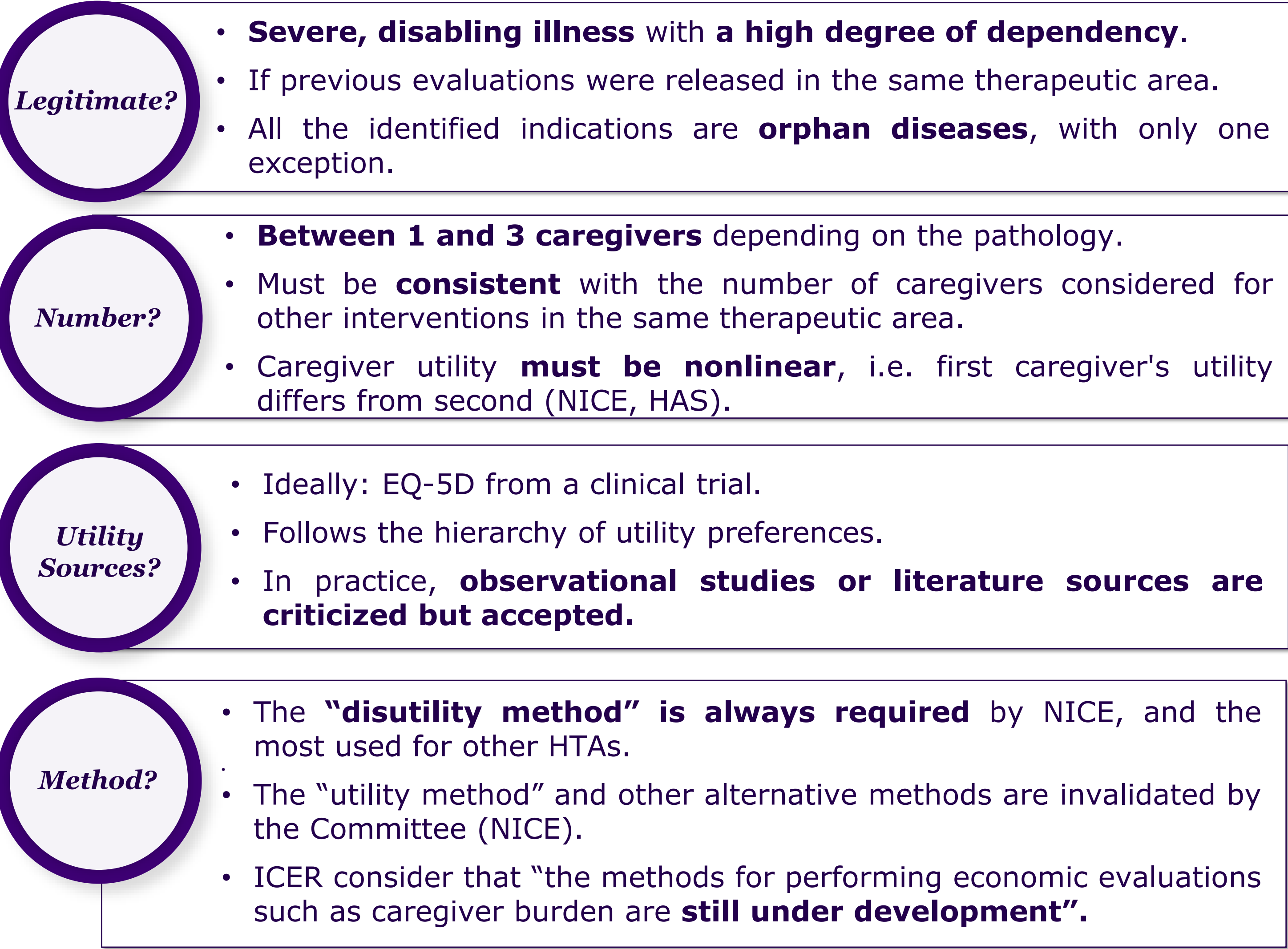
DISCUSSION

- The HTA agencies selected in this research were chosen to best represent the diversity of opinions regarding caregiver HRQoL.

- Further studies on adult or elderly indications are needed to know whether HTA expectations identify for pediatric indications are similar.

- The assumption regarding the choice of method for implementing caregiver utility in the model has the heaviest impact on the results and raises the most ethical issues.

- In fact, for fatal diseases, the "disutility" method produces a counterintuitive effect - a "**Caregiver QALY trap**"[2].

- **Consensus is needed** on which method – "utility","disutility" or an alternative one - to use. Even though the "disutility" methods are always requested, NICE doesn't deny limitations.

- In addition, bereavement issues have been identified in some appraisals.

CONCLUSION

- This analysis led to draw 3 recommendations on the implementation of caregivers' HRQoL in a CEA for pediatric indications:
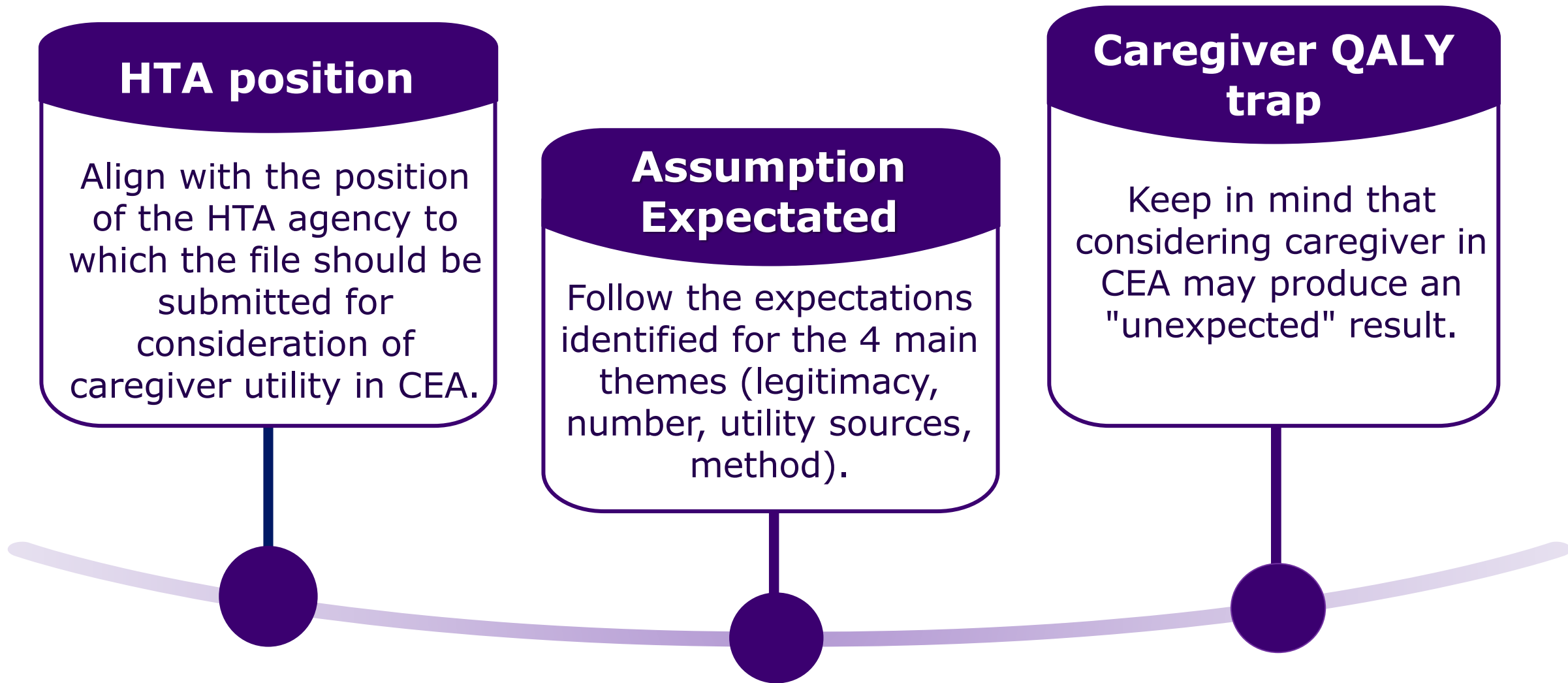
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2. Mott DJ, Schirrmacher H, Al-Janabi H, Guest S, Pennington B, Scheuer N, Shah KK, Skedgel C. Modelling Spillover Effects on Informal Carers: The Carer QALY Trap. Pharmacoeconomics. 2023 Dec;41(12):1557-1561. doi: 10.1007/s40273-023-01316-0. Epub 2023 Sep 2. PMID: 37659032; PMCID: PMC10635951.