The use of vignette studies to estimate health-state preference in Sweden and Norway

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Background

When performing a cost utility analysis (CUA) in the context of health technology assessment (HTA), it is necessary to capture both the costs associated with the introduction of an intervention and its utility in health gains ('QALY-weights'). The accepted methodology relies on standardized questionnaires like the EQ-5D instruments.

With certain diseases, direct data from patients are unavailable, therefore alternative methods such as vignette studies are used to inform the utilities. The objective of this poster is to investigate the use of vignette studies in the HTA practice followed by the Swedish and Norwegian authorities (TLV & NoMA).

A vignette is a description related to a specific health state that is valued by patients through a preference elicitation task used to generate a vignette-based utility. A vignette describes symptoms of the condition and in general the impact on health-related quality of life (HRQL) [1]. According to TLV guidelines, QALY-weightings should be based on direct measurements with the above-mentioned methods or through indirect measurements such as the EQ-5D instrument [2]. However, there is no clear guidance if these data are not available, and the use of alternative methods is required. NoMA guidelines state that the EQ-5D instrument is preferred when possible. While more details are provided, none involve specifically vignette studies [3].

Methods

Both the TLV and NoMA webpages were searched to identify recent assessment of new interventions using vignette-based utilities in the CUA.. These were identified through a search within the TLV and Nye Metoder decision database, for assessed decisions that contained "livskvalitetsvikter & vinjett" related key words. All the drugs were granted Orphan status by EMA and are used to treat rare disease, with labels including pediatric populations for all but one drug. They are used to treat diseases which were either classified as high in severity or in case of heterogenous development, with variable degree, including very high severity. However, TLV to date assessed more applications containing vignette studies than NoMA. The validity of the vignette approach was measured against the criteria proposed by Matza et al in Table 1, which identified situations in which the use of vignette-based utilities may be motivated.

Table 1. Situations when vignette-based methods are deemed advantageous [1]

Criteria	Description
I	**Patients who are difficult to access due to the rarity of the disease or their age**
II	**Isolating the utility impact of specific attributes, such as AEs, symptom, or treatment attribute**
III	**Treatment process utilities, such as mode of administration, dose frequency, medical device attributes, and treatment convenience**
IV	**Acute and temporary health states, such as exacerbations**
V	**Health states that change over time**

Results

The drugs analyzed in the past 5 years for Norway and Sweden are presented in Table 2.

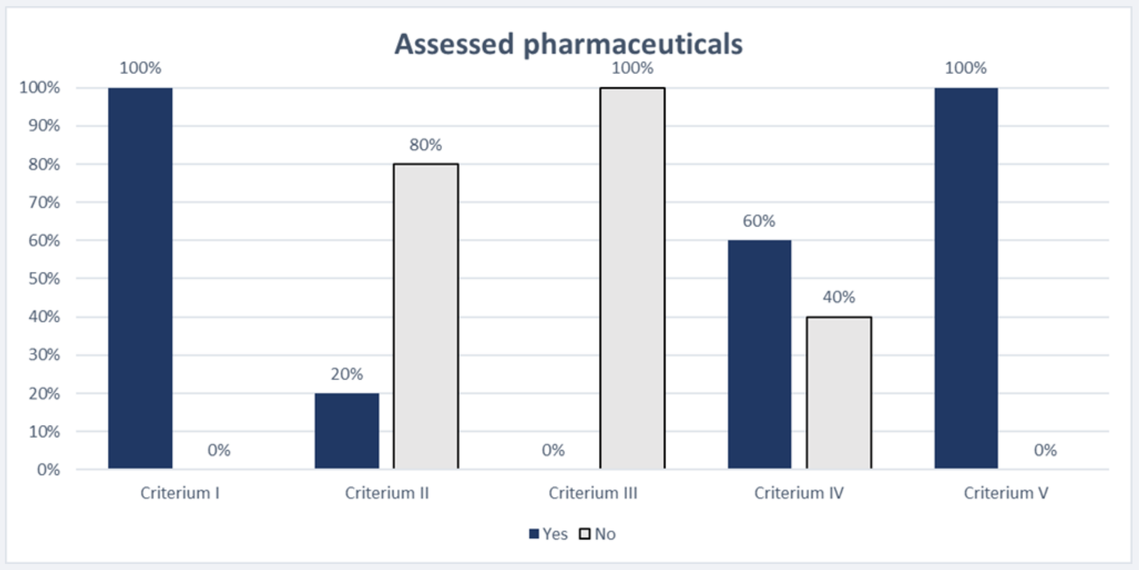
Table 2. Summary of characteristics of assessed pharmaceuticals

Drug	EQ 5D study in the decision	Reimbursed/ Recommended (SWE/NO)	Severity	Hospital drugs
Koselugo	No	No / n.a.[4]	n.a.[2]	No
Strensiq	Yes	No / No[5]	n.a.[3]	Yes[1]
Crysvita	Yes	Yes / Yes	High	Yes
Waylivra	Yes	No / n.a.	High	Yes
Epidyolex	No	No / Yes	Very High	No

Notes: 1: can be administered at home; 2:NF1 PN is a disease of great heterogeneity, with manifestations from a variety of organ systems and where the severity depends both on the tumor's size and exact location; 3: The specific symptoms can show great individual variations and the different forms of the disease vary in severity. Even within the same form the severity can vary greatly; 4: Currently in the process of being assessed in Norway - Sykehusinnkjøp HF is responsible for carrying out price negotiations with the company and work is now underway to prepare a price note; 5: In Norway, the company did not submit the required material, therefore a negative decision was finalized.

All the drugs fulfil one or more criteria presented by Matza et al, as shown in Figure 1. However, only one of the five assessed drugs was recommended/reimbursed for use in Sweden, while for Norway two out of three got a positive decision and one is still being processed with a simplified assessment that will not make use of the vignette study utility, in line with the Norwegian simplified assessment guidelines [3].

Figure 1. Assessed pharmaceuticals criteria fulfillment



Discussion and conclusion

Overall, TLV considered that applications informed with vignette-base utilities were associated with such a high degree of uncertainty as to question their use for decision making. This despite the challenges associated with directly measured generic preference-instruments in these patient populations and conditions motivating the use of such methodology according to ISPOR guidelines [1]. NoMA presented a similar positioning with respect of uncertainties but diverged in terms of final outcomes compared to Sweden. Moreover, the Norwegian policy allows a more flexible assessment in case of particular pharmaceuticals' assessments, which profiles do not allow for an in depth HTA such as orphan drugs with a few patients and lacking clinical efficacy data [3]. While it is not possible to isolate the impact of vignette studies on the overall decision process, it is clear that these are seen as an uncertain approach by the TLV, while NoMA seems to be more open and flexible in terms of applying those utilities in their decision processes. Therefore, to increase transparency and reduce uncertainty in decision making, technical guidance on the use of vignettes in the health economic assessments performed by TLV would be a welcome addition to the TLV guidelines.

References

[1]Matza, L.S., et al., *Vignette-Based Utilities: Usefulness, Limitations, and Methodological Recommendations.* Value in Health, 2021. 24(6): p. 812-821.
[2]TLV, *General guidelines for economic evaluations from the Pharmaceutical Benefits Board* 2003.
[3] Nye Metoder, *Guidelines for the submission of documentation for single technology assessment (STA) of pharmaceuticals*, 2018