Are there specificities for assessing quality of life and utilities in rare diseases for economic evaluation in France: a case study of published **CEESP** opinions

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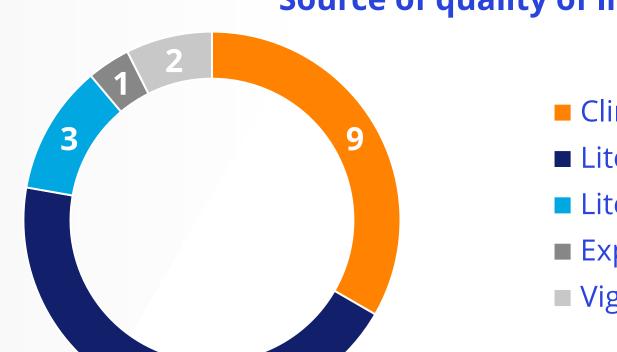
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OBIECTIVES

> Measuring quality of life (QoL) in rare diseases could be challenging: small samples or populations without cognitive ability to answer QoL questionnaires requiring a proxy (third person) to respond (including pediatric patients). These challenges are identified in the French HTA body guidelines for health economics, but no standard is proposed to tackle them.

> The repartition of data sources for utility of all opinions is presented below.



Source of quality of life data

- Clinical trial
- Literature
- Literature and clinical trial
- Expert opinion
- Vignettes

This study aims to assess the impact of the methodology used to estimate utility values and the conclusions of the French health-economic committee (CEESP), including ICER results, for orphan drugs.

METHODOLOGY

> A descriptive review of QoL measures used by manufacturers and their assessments including ICER and conclusions in CEESP opinions published since 2014 in rare diseases (orphan drugs).



Twenty-seven CEESP opinions on rare diseases were analyzed (target populations varied from 75 to 8 830 patients): 14 opinions included both pediatric and adult populations, one pediatric population only.

Challenge in utility assessment for orphan drugs

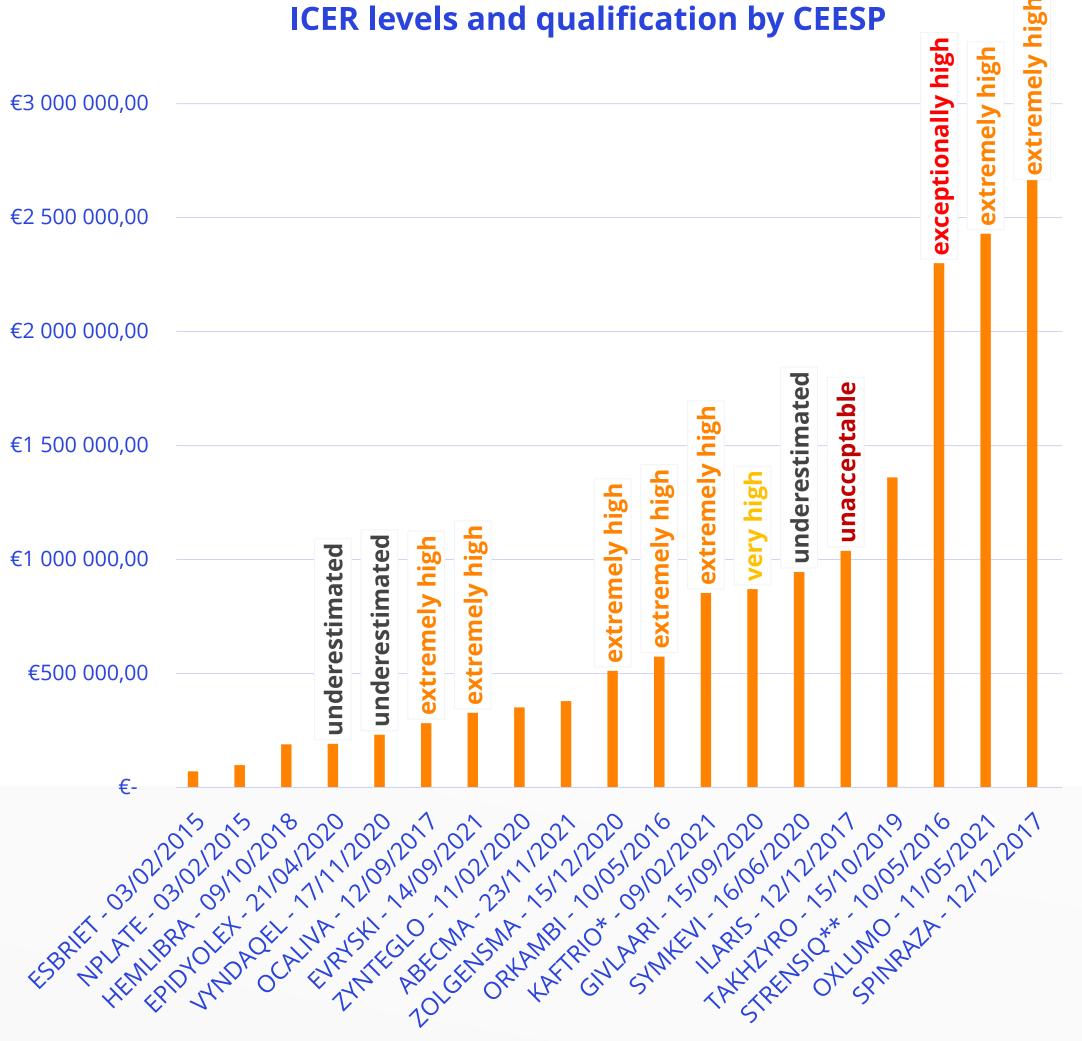
> Out of the 27 opinions analyzed, 52% (n=14) have at



- > For the opinion without major reservation, when the method was considered appropriate by CEESP, it was supported by robust data sources: literature (n=11), clinical trials (n=7) or both (n=3).
- No differences were noted between adult and pediatric populations and no proxy respondent (parent or carer) had to be asked when the data came from clinical trial.

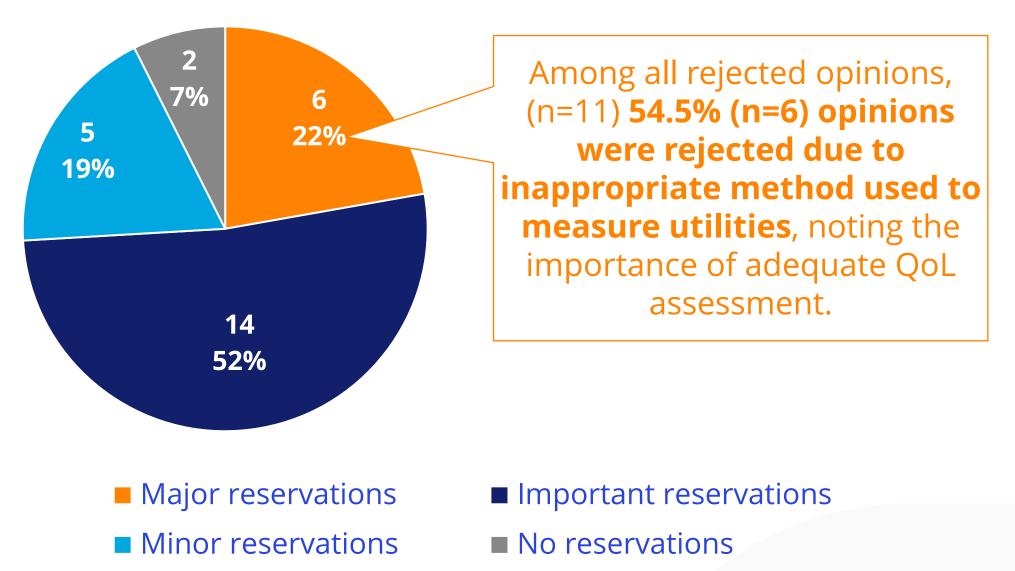
Methodology accepted by CEESP: the challenge of willingness to pay for orphan drugs

- > When the methodology was acceptable, CEESP could have assessed efficiency of the drugs, but **other issues** appear with important ICER levels.
- > CEESP concluded on efficiency for 19 opinions (70%) ICER was approximately and the average **€827,000/QALY** with a maximum at €2.7 million/QALY.



least one important methodological reservation regarding the QoL measure.

Maximum methodological reserve level associated with utility



Methodology rejected by CEESP: the role of data sources

- For 5 opinions with major reservation on utilities, >CEESP rejected the methodology considering **data source** were inappropriate:
 - Vignette study (n=2), rejected because they were \rightarrow not completed by patients themselves,
 - Expert opinion (n=1),

**Complementary analysis used by the HAS despite a major reservation on the utility* **ICER of the subpopulation for which there is no major reservation

- > In 50% of the cases, the CEESP considered these ICER levels to be extremely high and in 2 cases, the ICERs
- Disease-specific questionnaires (n=2). \rightarrow

"exceptionally qualified high" as were even or "unacceptable".



CONCLUSION

Despite methodological difficulties for assessing utilities in rare diseases, **most of the opinions have implemented CEESP guidelines without specific issues** related to QoL measure identified. Beyond the methodology used, when results can be estimated, they illustrate the debate in the academic literature questioning the relevance of higher thresholds for rare diseases.



REFERENCES

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