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

Poster EE90

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OBJECTIVES

> 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.

> **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).

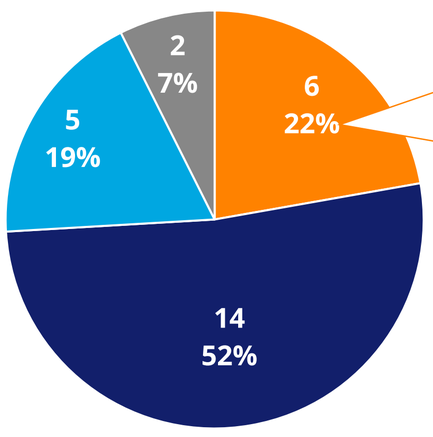
RESULTS

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 least one important methodological reservation regarding the QoL measure.**

Maximum methodological reserve level associated with utility



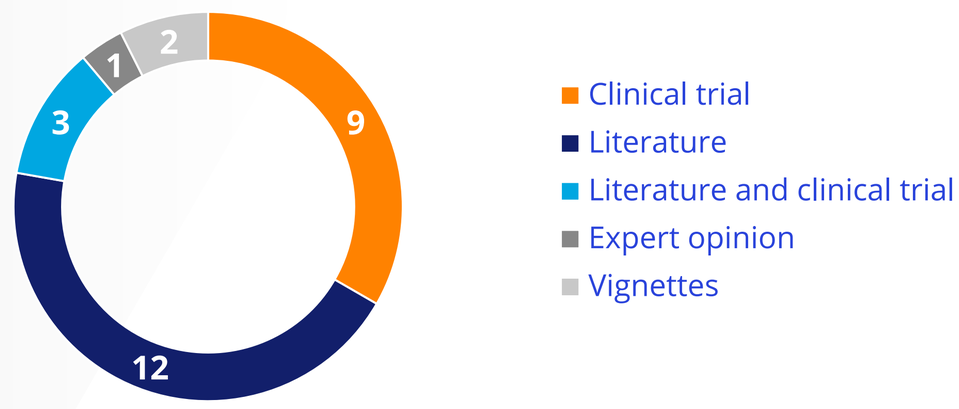
Among all rejected opinions, (n=11) **54.5% (n=6) opinions were rejected due to inappropriate method used to measure utilities**, noting the importance of adequate QoL assessment.

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 not completed by patients themselves,
  - → Expert opinion (n=1),
  - → Disease-specific questionnaires (n=2).

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

Source of quality of life data



> **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).

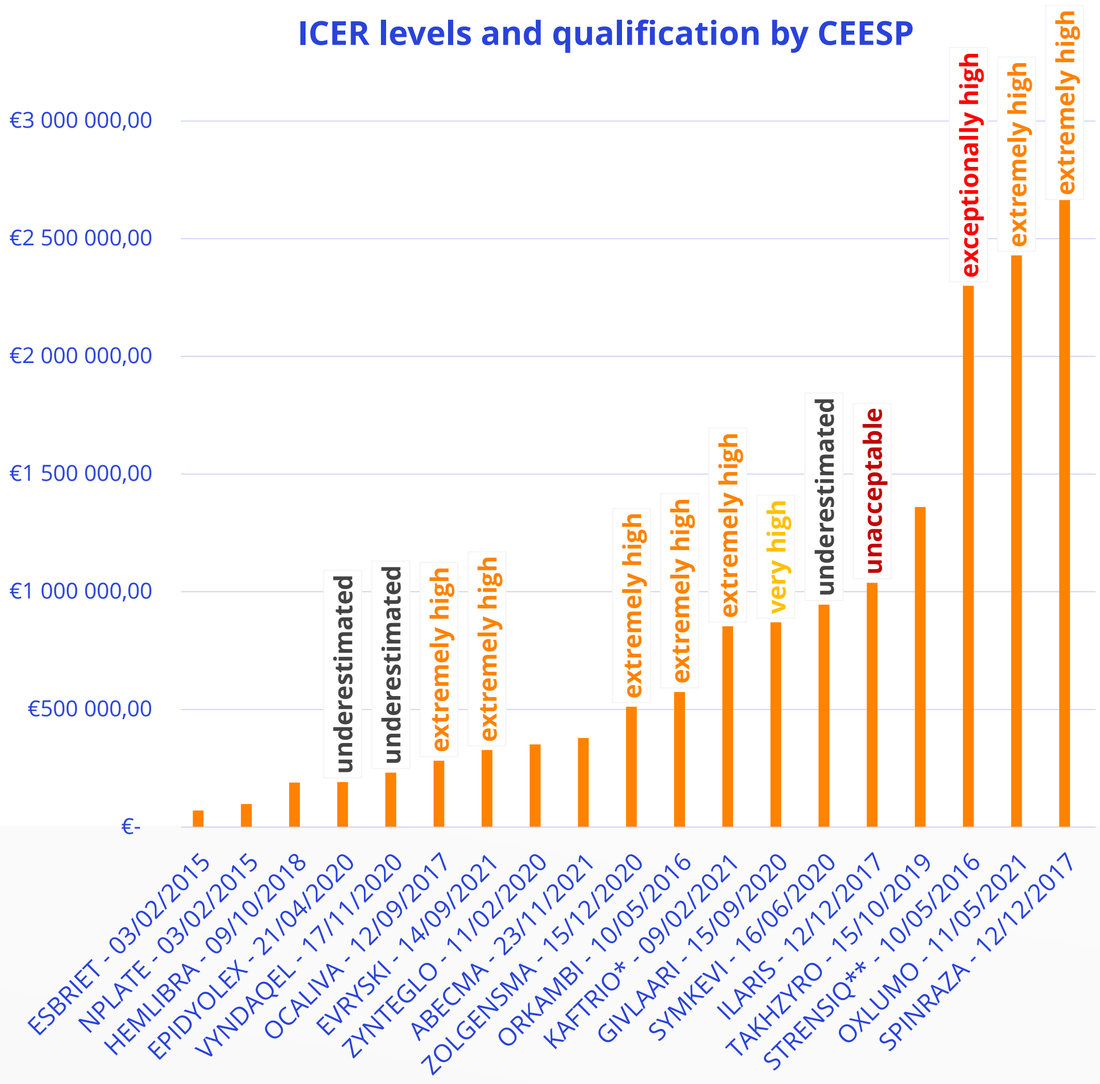
> **No differences were noted between adult and pediatric populations** and no proxy respondent (parent or carer) had to be asked when the data came from clinical trial.

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%) and the average ICER was approximately €827,000/QALY** with a maximum at €2.7 million/QALY.

ICER levels and qualification by CEESP



**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 were qualified as “exceptionally high” or even “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**.

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REFERENCES

> Data source : published CEESP opinions at https://www.has-sante.fr/jcms/p_3149875/fr/avis-economiques-rendus-par-la-commission-d-evaluation-economique-et-de-sante-publique-ceesp