

# FOCUS ON TWO YEARS OF ECONOMIC OPINIONS ON ORPHAN DRUG - WHAT ARE THE SPECIFIC FEATURES?

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## Introduction

- In France, the Commission for Economic and Public Health Evaluation (CEESP) provides an economic opinion of the incremental cost-effectiveness ratio (ICER) validity. The CEESP uses the same methodological framework to evaluate economic analyses relating to very different clinical contexts.
- To approach the challenges specific to the economic evaluation of treatments for rare diseases according to a discriminating criterion, our study focused on drugs with orphan drug designation.
- Orphan drugs occupy a special place in terms of technological innovation and are often awaited by patients who have reached a therapeutic impasse. At the same time, the availability of data to document the analyses associated with these treatments represents a real challenge.
- The aim is to analyze the CEESP opinions in orphan drug appraised over the last two years (2021-2022) to discuss how the CEESP stands in relation to this type of challenge.

## Methods

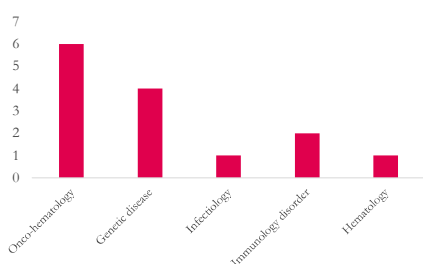
- Since the publication of the first efficiency opinions on the HAS website, Vyoo Agency has built up a structured database on the key elements constituting these opinions. Using Vyoo Agency efficiency database, all CEESP opinions on orphan drug between January 1, 2021, and December 31, 2022, were reviewed.
- We consider an opinion valid if there are no mention of major uncertainty or objection.

## Results

### Orphan drugs opinions

- Between 01/01/2021 and 31/12/2022, fourteen CEESP opinions relative to an orphan drug were issued.
- These opinions were related to twelve different diseases and classify through three therapeutics areas.

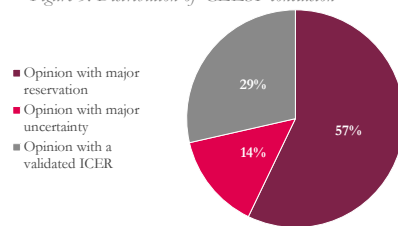
Figure 2. Numbers of opinions therapeutic area



### CEESP assessment

- The conclusions of 8 of these 14 analyses were invalidated by the CEESP regarding a major reservation. Two analyses were rejected regarding major uncertainty regarding the lack of robust data.

Figure 3. Distribution of CEESP conclusion

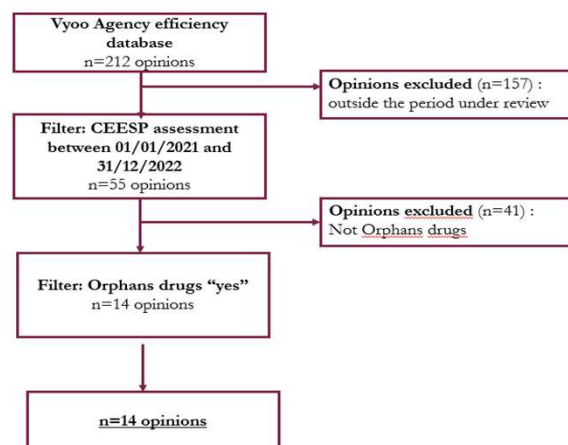


- A total of 12 major reservations were raised. These reservations mainly concern the estimation of utility scores (5 major reservations) and relative effectiveness (4 major reservations relating to the indirect comparison method).

## Steps of data extraction

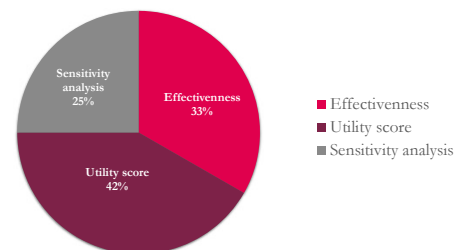
Filter : Orphan Drug restriction, time filter 01/01/2021\_31/12/2022

Figure 1. Flowchart orphan drug (2021-2022) (May 25, 2023)



Fourteen CEESP assessments on orphan drug referenced in Vyoo Agency database between 01/01/2021 and 31/12/2022

Figure 4. Distribution of major reservations



### ICER

- All analyses considered, the average ICER is €496,167/QALY. Only three ICER submitted are under €150,000/QALY and one invalidated analysis concluded that the intervention evaluated was dominant.
- Among validated ICER, the average ICER is €777,878/QALY and ranges from €7,392/QALY to €2,429,397/QALY. Three out of four ICER are above €250,000/QALY.

## Conclusion

- Over the two years considered for the analysis, CEESP has delivered 55 economic opinions, including 21 with a validated ICER (38%). Opinions on orphan drugs (N=14) comprised 25% of all opinions appraised and is characterized by a lower proportion of validated economic opinions (29%), especially in other therapeutic areas than onco-hematology (13%).
- Regarding the distribution of major reservations, the way relative effectiveness and utility are estimated significantly weakens the acceptability of economic analyses of orphan drugs.
- The availability of robust data that satisfy the requirements of the CEESP is a real challenge in the context of diseases involving very small target populations - between 100 and 3,000 patients in France.
- Considering the low number of validated evaluations of orphan drugs, it seems important to consider how to improve the ability of these evaluations to produce relevant economic information.

## References

- Vyoo Agency efficiency database. Online subscription only : <https://efficiency.vyoo-agency.com/>
- Choices in methods for economic evaluation - HAS, 2020. [https://www.has-sante.fr/upload/docs/application/pdf/2020-11/methodological\\_guidance\\_2020\\_choices\\_in\\_methods\\_for\\_economic\\_evaluation.pdf](https://www.has-sante.fr/upload/docs/application/pdf/2020-11/methodological_guidance_2020_choices_in_methods_for_economic_evaluation.pdf)
- CEESP Doctrine. [https://www.has-sante.fr/upload/docs/application/pdf/2021-09/doctrine\\_de\\_la\\_ceesp.pdf](https://www.has-sante.fr/upload/docs/application/pdf/2021-09/doctrine_de_la_ceesp.pdf)

Figure 5. Acceptability of ICER of orphan drug by therapeutic area

