

Uncertainty appraisal in economic opinions of the French National Authority for Health

Sambuc C¹, Segquier C¹, Tehard B¹, Chevalier J¹, Midy F¹, Roze S¹

¹VYOO AGENCY, 10 rue Yvonne, 69100 Villeurbanne/34 rue du Faubourg Saint Honoré, 75008 Paris

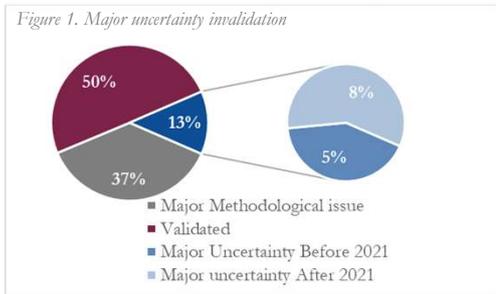
Context

The French Commission for Economic and Public Health Evaluation (CEESP) appraises the manufacturers' incremental cost-effectiveness ratio (ICER) validity. The ICERs are valid if there is no mention of major methodological reservation or uncertainty. Although ICERs have been invalidated for the latter reason since 2014, it wasn't until 2021 that the CEESP doctrine clarified the reasons that could lead

Results

Over the period 01/2014 to 07/2024, the CEESP published 229 economic opinions related to 237 indications (151 before 2021 and 86 after 2021).

Eighty-eight indications were excluded due to methodological issues. 31 indications were invalidated solely due to high uncertainty (13%).



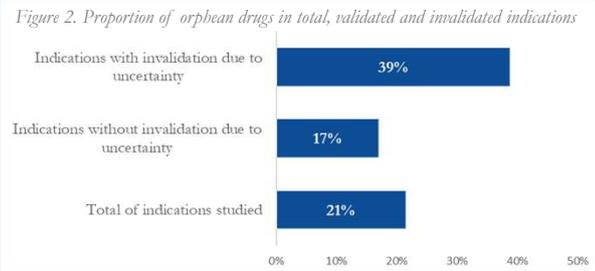
Objective & Methods

The aim is to quantify and analyze invalidations exclusively due to uncertainty in CEESP opinions.

Economic assessments invalidated by the CEESP exclusively on the grounds of uncertainty over the period 01/2014 to 03/2024 were analysed.

Focus on orphan drugs

Orphan drugs account for 21% of indications evaluated (with no major reservations) and account for 39% of assessments invalidated due to uncertainty, whereas they account for 17% of assessments without major uncertainty.



Relationship between the number of important methodological issues and invalidation due to uncertainty

After 2021, the average of important reservations is higher in assessment invalidated due to major uncertainty (6.2 with vs 3.3 without) and the proportion of opinions with at least 5 important reservations was also higher (77% vs 26%).

However, the CEESP only explicitly cited the accumulation of reservations to justify the major uncertainty in 3 opinions. In the CEESP conclusion, the main argument is the uncertainty in estimating certain key parameters (7/13, 54%). In 39% (5/13) of cases, the CEESP concluded that the uncertainty could not be assessed. These different reasons aren't mutually exclusive.

Figure 3. Analysis of the reasons of global major uncertainty

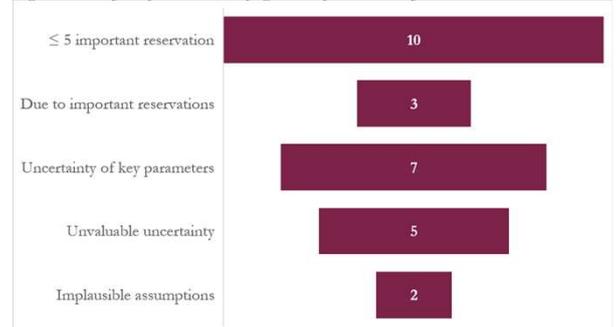
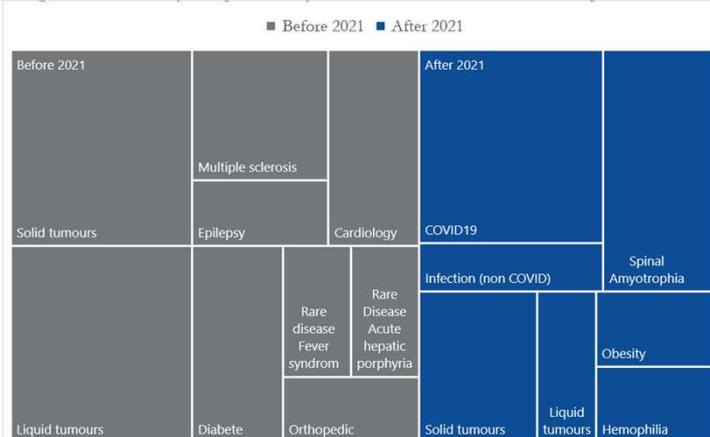


Figure 3. Breakdown of therapeutic areas for invalidated assessments due to uncertainty



Clinical area description

Prior to 2021, the main therapeutic areas affected by an invalidation due to uncertainty were onco-haematology (22%) and oncology (22%). This observation is consistent with the over-representation of these areas in submissions.

After 2021, a lower proportion is observed in oncology (15%) and haemato-oncology (8%) assessments.

The onset of the pandemic has had an impact: COVID-19 assessments account for 31% of invalidations due to uncertainty. The problem lies in the modelling of a single epidemic wave, specific to an epidemiological context and timeframe. The major uncertainty reflects the difficulty of producing appropriate clinical data in a context of evolving variants.

Conclusion

The definition of major uncertainty in the CEESP doctrine seems to have led to more invalidations on the grounds of uncertainty, with an increase from 8% to 21% of the CEESP opinions by indication. This trend should be confirmed over time.