HTA168

"THE MOUNTAIN LABOURED AND GAVE BIRTH TO A MOUSE": A SURVEY OF CURRENT AND FORMER EUROPEAN PAYERS ON THE FIRST EU JOINT CLINICAL ASSESSMENT REPORT

CONCLUSIONS

Experienced EU healthcare decision-makers/payers regarded the first JCA report as largely methodologically rigorous but of limited practical utility for local HTA, tempering early expectations. Whether this reflects an atypical rare-disease index case or more fundamental underlying issues, warrants monitoring as further JCA reports are published. It also remains to be seen whether the JCA process/evidentiary requirements process will be modified for orphan drugs in the future.

AUTHORS

James Horscroft,[1] Sylwia Lach[1], Helena Emich[1], Rachel Beckerman[1]

AFFILIATIONS

1. Maple Health Group, LLC. New York, USA

REFERENCES

1. European Commission, (2026) JCA for medicinal products. Available at: https://health.ec.europa.eu/health-technology-assessment/implementation-regulation-health-technology-assessment/joint-clinical-assessments-medicinal-products_en
2. European Commission, (2026) JCA report on tovorafenib (OJEMDA). Available at: https://health.ec.europa.eu/publications/joint-clinical-assessment-report-tovorafenib-ojemda_en

MAPLE HEALTH GROUP

INTRODUCTION

- Joint Clinical Assessment (JCA) is a comparative analysis of the clinical evidence for a new health technology at the European Union (EU) level, evaluating relative effectiveness and safety against existing treatment alternatives. From January 2025, new medicines for the treatment of cancer, as well as advanced therapy medicinal products are subject to JCA with its scope expanding to orphan medicines from January 2028 and to all new medicines from January 2030.[1]
- For each product, a JCA report is published at the end of the assessment process. National health technology assessment (HTA) bodies across the EU Member States must give the assessment report "due consideration" for their local HTA and decision making.[1]
- The first JCA report was published in June 2026, assessing tovorafenib for the treatment of paediatric low-grade glioma. In this first JCA, comparative clinical evidence was only provided for one of eight specified PICOs, with the manufacturer citing insufficient comparative data as a reason for not addressing the remaining seven.[2]

STUDY OBJECTIVE & METHODOLOGY

- We surveyed experienced EU healthcare decision-makers/payers to characterise perceptions of the inaugural JCA report and its anticipated impact on local HTA.
- A structured online survey was administered to current and former healthcare decision-makers/payers across EU Member States. Respondents were asked to rate six statements on a five-point Likert scale (from "strongly disagree" to "strongly agree") focusing on PICO relevance, evidentiary thoroughness, local HTA efficiencies, attainability of JCA standards in rare diseases, and overall optimism. In addition, respondents had the opportunity to provide any relevant comments via an open free-text field. Responses were summarised descriptively.

RESULTS

- Eleven healthcare decision-makers/payers from Germany (n=4), Spain (n=3), France (n=2), and Italy (n=2) responded; three were currently active (two AMNOG arbitration board members and one hospital pharmacist) and eight formerly held relevant positions (two Transparency Committee members, one AIFA member, one Commissione Unica del Farmaco member, one AMNOG arbitration board member, one CatSalut member, one German Association of Private Health Insurance member and one Spanish Ministry of Health member).
- Overall, most respondents (6/11) agreed or strongly agreed that the eight scoped PICOs reflected relevant populations and comparators in their market (**Fig. 1**). Similarly, 6 of 11 respondents agreed or strongly agreed (and only 2 disagreed or strongly disagreed, with 3 expressing a neutral opinion) that the tovorafenib JCA was exhaustive and thoroughly reviewed all available comparative evidence, despite only 1 of 8 PICOs being addressed in the submitted dossier (**Fig. 1**).
- In terms of future outlook and implications on local HTA, 4 of 11 agreed or strongly agreed and 3 were neutral that the high evidentiary standards set by the JCA will raise standards for local HTA across the EU (**Fig. 1**). However, at the same time, the majority (6/11) agreed or strongly agreed that the specified evidentiary standards may be unattainable in rare diseases (**Fig. 1**).
- Crucially, most respondents (6/11) disagreed or strongly disagreed that the tovorafenib JCA report would save local HTA review time, and the same proportion were not optimistic about the JCA's overall broader benefit to local HTA (**Fig. 1**).
- Free-text themes described a voluminous yet largely "non-assessment", concern over a single manufacturer-conducted matching adjusted indirect comparison (MAIC) that potentially introduces biased results and conclusions as well as minimal patient/clinical input. One of the respondents highlighted the potential benefit of JCA on local HTA when the assessment is done based on a strong dossier with several randomized controlled trials (RCTs). In addition, there was an expectation that national bodies will set their own (additional) PICOs regardless of the pan-European assessment scope, rendering the published JCA report inutile.

Figure 1. Responses to each question in the survey

