Assessing the Value of Real-World Evidence for HTA-Decision Making

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OBJECTIVE

This study aims to **assess the value of Real-World Evidence (RWE) for Health Technology Assessment (HTA)** using the UK National Institute for Health and Care Excellence (NICE) and French Haute Autorité de Santé (HAS) as case studies.

METHODOLOGY

1. **Data Collection**: We retrieved HTA reports published between May 2023 and May 2024 from the websites of the NICE and the HAS. The selection included NICE Single Technology Appraisals (STAs), Highly Specialised Technologies (HSTs), and HAS Commission de la Transparence (CT) reports.
2. **Automated Retrieval**: An R-based web scraper, adapted from Polak et al. (2020, 2023), was employed to automatically identify and download HTA reports containing predefined keywords related to RWE in both English and French.
3. **Data Extraction via HTAi Application**: The collected reports were processed using the HTAi application, a Large Language Model (LLM)-based tool. This application extracted key information based on predefined variables and prompts, compiling the data into a structured table. Human reviewers conducted manual validation to ensure data accuracy before exporting the final dataset to Excel.
   - ***Data Extraction Variables***: Submission details, product details, as well as submitted clinical evidence, RWE studies, and economic evidence.
4. **Analysis of RWE Utilization**: We analysed trends in the use and impact of RWE within the HTA reports. Each report was categorized based on the role of RWE as a primary source of evidence, supportive evidence, or insufficient for HTA decision-making.

RESULTS

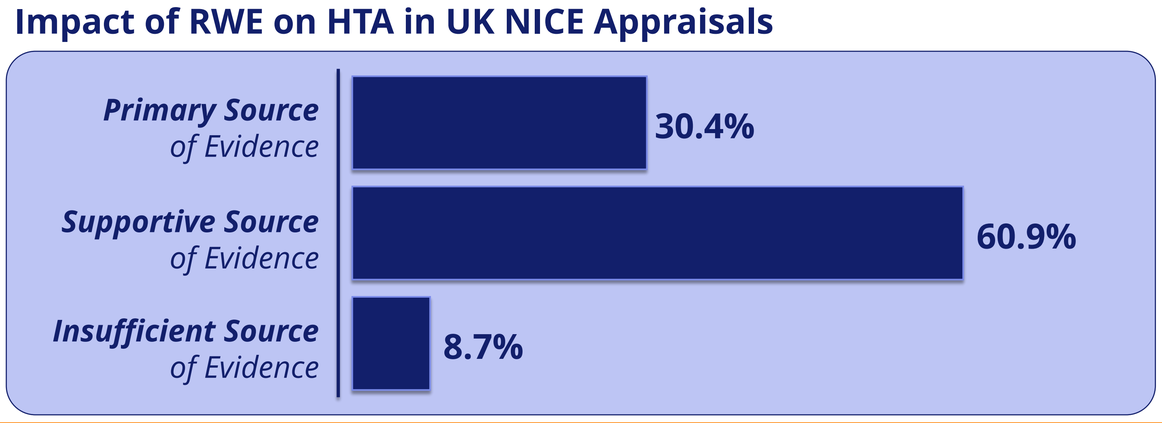
Use of RWE for HTA in UK NICE Appraisals

The analysis of HTA reports submitted to NICE over the last year identified retrospective cohort studies and registry-based research as the most common types of RWE submitted.

> **Observational retrospective cohort studies accounted for 57%** of submitted RWE studies. These studies primarily aimed to compare the effectiveness of treatments in real-world settings, such as OpenSAFELY data comparing hospitalizations and mortality between treatments for COVID-19.

> **Registry-based research accounted for 35%** of submitted RWE studies. These studies focused on long-term, real-world insights into disease progression, treatment effectiveness, and safety. For example, a transition probabilities and survival study conducted with the RaDaR database for chronic kidney disease captured transitions between disease stages over time, providing natural history data that informs models on disease progression.

Impact of RWE on HTA in UK NICE Appraisals



RESULTS

Use of RWE for HTA in French HAS Appraisals

The analysis of HTA reports submitted to HAS over the last year indicate a growing role of RWE in HTA. Observational studies, registries, and analyses of national health databases are the most common RWE studies submitted.

> **Observational Studies made up 50%** of submitted RWE studies.

- **Retrospective cohort studies** were utilized to analyse existing data from patient records or databases, while **prospective cohort studies** collected data on effectiveness and safety over time in clinical settings, such as the EPI-PHARE study assessing the risk of meningioma with *nomegestrol acetate*.

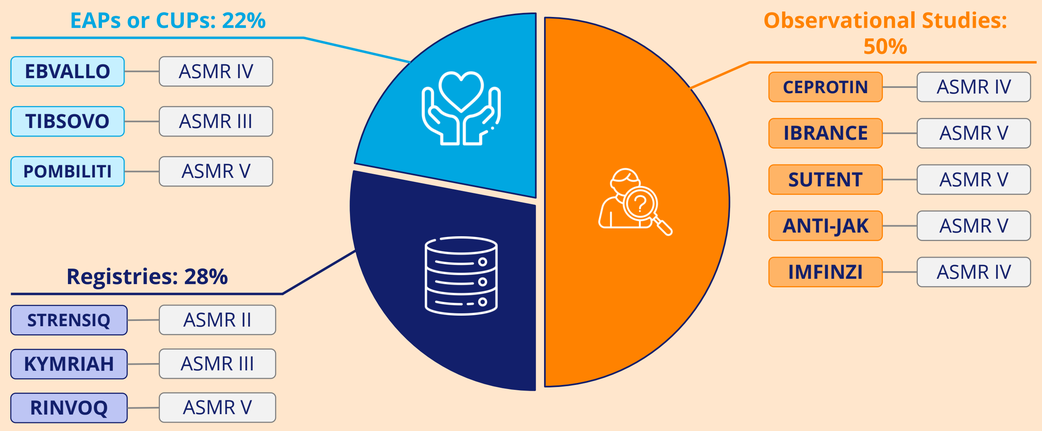
> **Registry-based research accounted for 28%** of submitted RWE studies.

- **Treatment registries** were used to demonstrate effectiveness and safety for receiving specific therapies such as the DESCAR-T registry for CAR-T cell therapies. **Disease registries** were also utilized to monitor long-term outcomes and rare adverse events such as the hypophosphatasia registry used in the evaluation of STRENSIQ which received an ASMR II.
- Due to comprehensive databases like the SNDS and policies favouring the use of nationally representative data, the **vast majority of RWD came from France** (37.5% French data sources, and 44.6% European Union-based data sources which also included the French population).

> Data collected during **Early Access or Compassionate Use Programs (EAP or CUP) accounted for 22%** of submitted RWE studies.

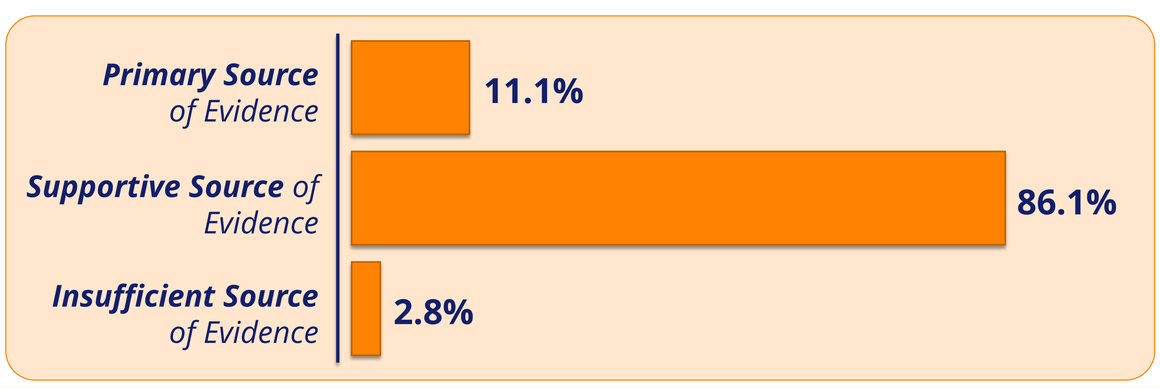
- The HAS recognizes the pivotal role of EAP/CUP in rare disease indications and advocates for the collection of RWD with committee guidance.

Types of RWE Submitted and ASMR Grading Outcomes



Impact of RWE on HTA in French HAS Appraisals

In the HAS, RWE primarily serves as supportive evidence to clinical trial data, particularly for rare diseases, long-term safety monitoring, and in scenarios where RCTs are not feasible.



CONCLUSION

> NICE considered a higher proportion of RWE submissions as primary evidence (30.4%) compared to HAS (11.1%), while HAS predominantly regarded RWE as supportive evidence (86.1%) versus NICE's 60.9%. Additionally, insufficient use of RWE was more pronounced in NICE (8.7%) than in HAS (2.8%).

> Although RWE frequently served a supportive role in validating and enhancing clinical trial data, certain submissions relied heavily on RWE as the primary evidence source, particularly for chronic and rare diseases where real-world insights are vital.

> While RWE offers unique advantages, some submissions faced limitations in follow-up duration, data completeness, and consistency, occasionally reducing their impact in HTA decision-making. These challenges underscore the importance of transparent methodologies and robust data protocols to improve the quality and reliability of RWE.

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