HTA97

Exploring the Value of RWD Collected from Early Access Programs to Support HTA Decision-Making

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BACKGROUND & OBJECTIVES

> **Early Access Programs (EAP) present an opportunity for patients to access treatments** before receiving market authorization. **This is of particular significance to patients living with rare diseases**, who often suffer severe and life-threatening conditions with limited treatment options.

> **EAPs play a pivotal role in the healthcare landscape of both the UK and France**. While both countries have their unique approaches, program names, and governing regulations, the shared goal remains: to bridge the gap between clinical research and patient needs. We recognize the various early access schemes in both the UK and France, however, **to ensure clarity in this poster we will use 'EAP' as a collective term.**

> EAPs also serve as a **crucial channel for gathering real-world data (RWD) on the safety, efficacy, and resource use of treatments**. Unlike data collected in clinical trials, RWD from **EAPs captures treatment outcomes in routine settings, representing a diverse patient population**.

> This study aims **to assess the impact and value of RWD collected during EAPs for Health Technology Assessment (HTA), using the French National Authority for Health (HAS) and UK National Institute for Health and Care Excellence (NICE) as case studies**.

METHODOLOGY

> Technology Appraisals (TA) documents published on the NICE website and Transparency Committee (TC) reports published on the HAS **website between 1st January 2021 and 1st September 2023** were included in the study. **In this poster, all appraisal documents will be referred to as 'TA'.**

> **We utilized a computer script in R (i.e., web scraper) to identify and automatically download TAs** containing predefined keywords related to "EAP" in both English and French. Subsequently, **a manual review was conducted** to extract pertinent data on the use of EAP in TAs (Figure 1). The R code was adapted from research by Polak et al. (2020, 2023), where a comprehensive description of the web scraper is provided.

> While TAs by NICE encompass detailed assessments of both clinical effectiveness and economic benefits of new technologies, HAS publishes economic opinions separately through the Commission for Economic Evaluation and Public Health (CEESP). **The current study focused on TA published by the TC**.

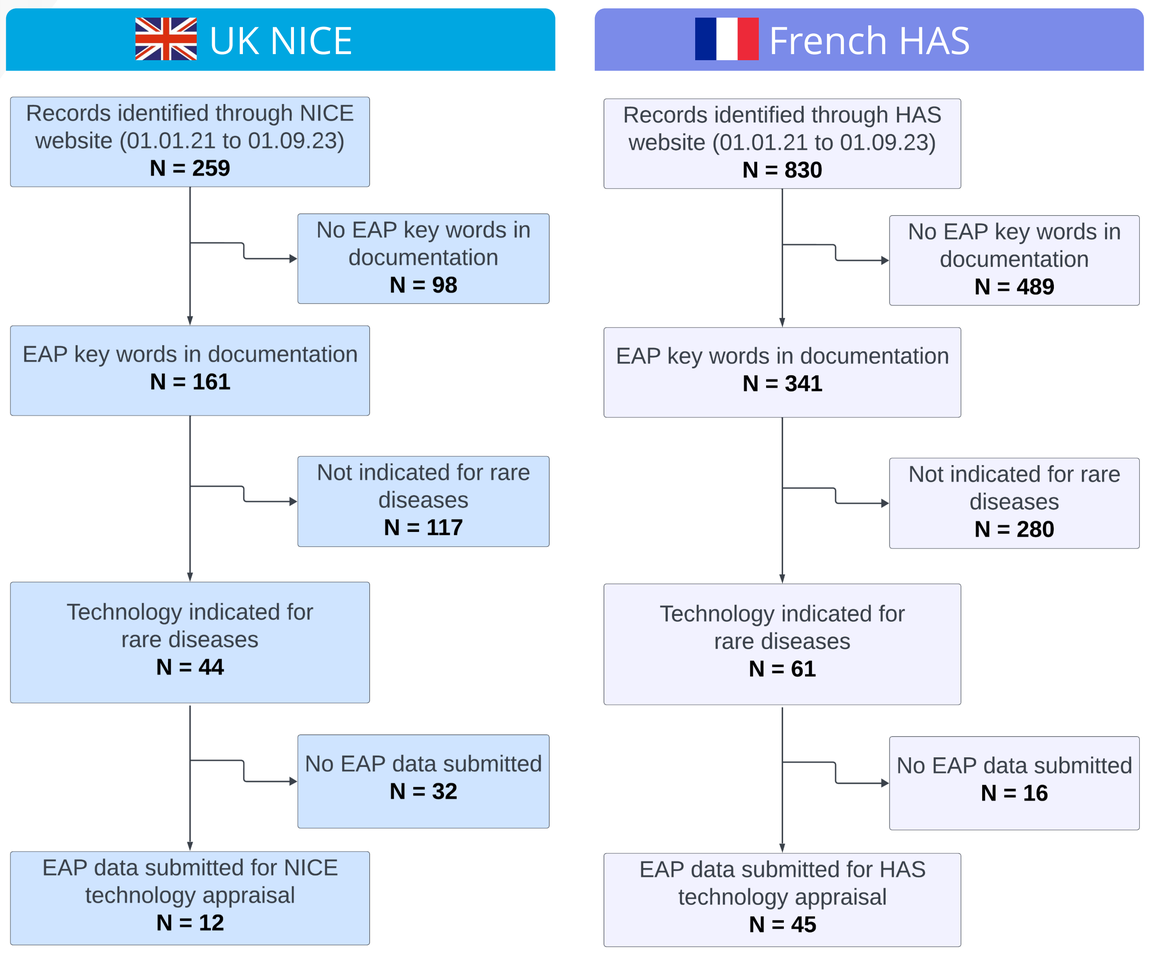


Figure 1: Flowchart of TAs reviewed for both NICE and HAS

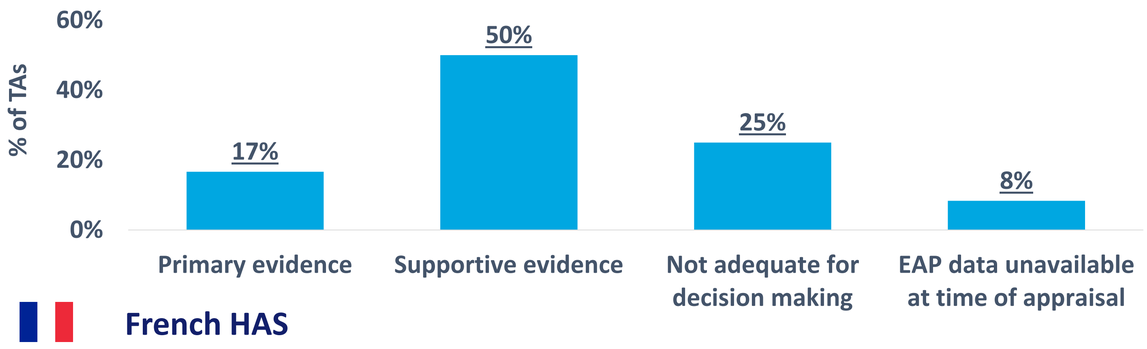
RESULTS

UK NICE

> **Overall, EAP studies** were used to demonstrate a **product´s clinical effectiveness and resource use, n=7** (Figure 4). Uniquely, for **asfotase alfa**, EAP data also covered **product safety** in addition to clinical effectiveness and resource use.

> **EAP data was predominantly utilized in cost-effectiveness evaluations and economic modelling**. Specifically, 9 out of 12 TAs incorporated EAP data to inform survival rates and disease progression. Furthermore, EAP data concerning cost factors, quality of life, and details on comparator products were employed to inform economic models.

Figure 2: TAs Submitted to NICE with EAP Data (n=12)

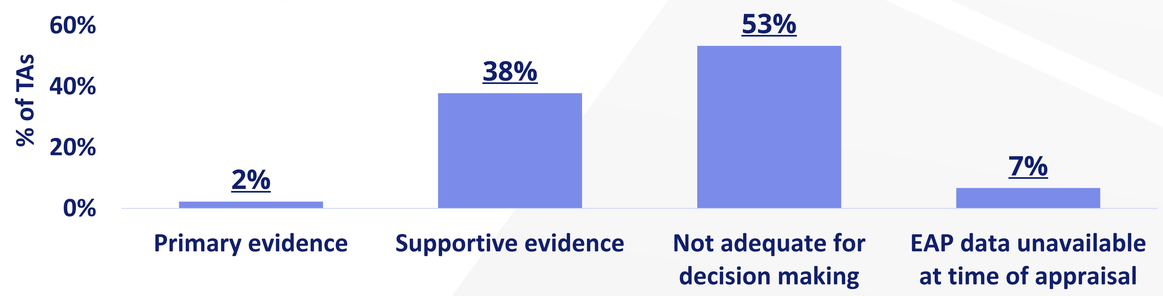


French HAS

> EAP studies were more frequently used to **demonstrate a product's safety in HAS TAs, n=20** (Figure 5).

> **No specific correlation was identified between the ASMR score and the influence of EAP data on the HAS decision**. Although, in general, it was observed that TAs with higher ASMR scores (III and IV) had a greater frequency of EAP data submitted as supportive evidence when compared to TAs with lower scores (i.e., ASMR V).

Figure 3: TAs Submitted to HAS with EAP Data (n=45)



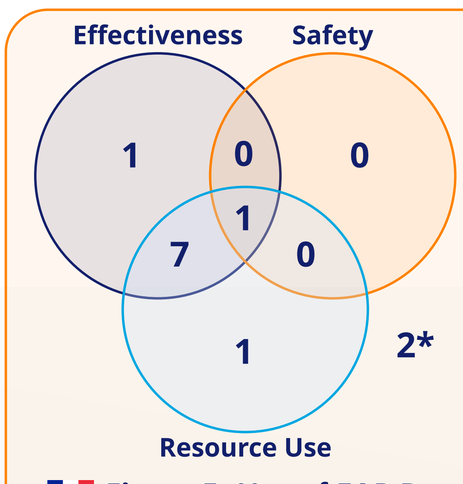


Figure 4: **Use of EAP Data in NICE TAs**
Venn-diagram illustrating the overlap of EAP data to demonstrate a product's effectiveness, safety, and resource use in NICE TAs.
**EAP data was unavailable at time of appraisal or did not inform any of the specified categories*

Figure 5: Use of EAP Data in HAS TAs
Venn-diagram illustrating the overlap of EAP data to demonstrate product effectiveness and safety in HAS TAs. Please note resource use was not evaluated for HAS TAs due to time constraints.

**EAP data was unavailable at time of appraisal or did not inform any of the specified categories.*

CONCLUSION

> Both the UK's NICE and France's HAS **recognize the pivotal role of EAPs, especially in rare disease indications**. Moreover, these HTA bodies advocate for the collection of RWD within these programmes. Such data not only provides **deeper insights into a product's real-world performance** but also offers companies an **avenue to showcase their product's superiority, potentially leading to favourable appraisals and beneficial reimbursement policies**.

> A substantial proportion of **EAP studies were deemed inadequate for decision-making** by both HAS (53% of TAs) and NICE (25% of TAs), **underscoring the importance of robust study design, relevant data collection for decision-making, and better alignment with HTA bodies regarding EAP data analysis**.

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