Real-World Evidence in Health Technology Asessment – A Fad or the Future?

Learnings for real-world evidence derived from early-access pathways

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INTRODUCTION

- Real-world evidence (RWE) is gathering importance across EU markets, with payers leveraging this data more to support their decision-making
- Early-access pathways (EAPs) allow for RWE generation, offering manufacturers the opportunity to support trial reproducibility claims in real-world practice, and even help payers understand current disease burden, standards of care, and remaining unmet needs
- This research evaluates how EAP-derived RWE are leveraged in health-technology assessments (HTAs) in France and the UK, offering an insight on their potential impact in access-decision making

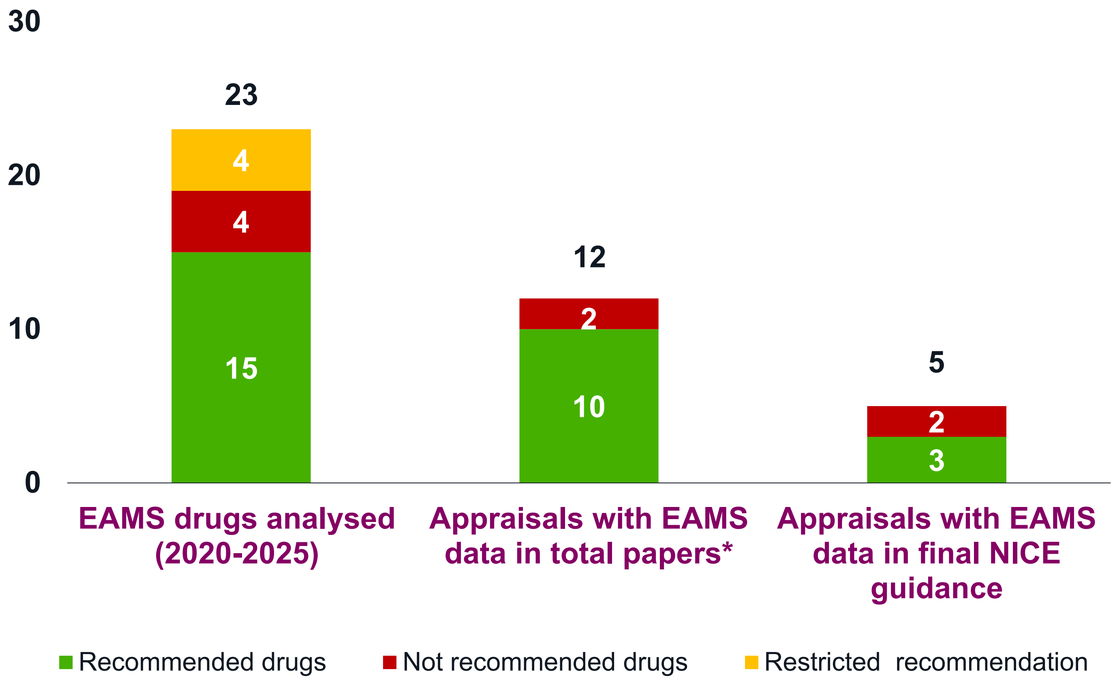
METHODS

We conducted secondary research on France and the UK to identify recently-assessed (i.e., 2020-2025) drug appraisals that entered the AAP and EAMS schemes respectively, looking at:

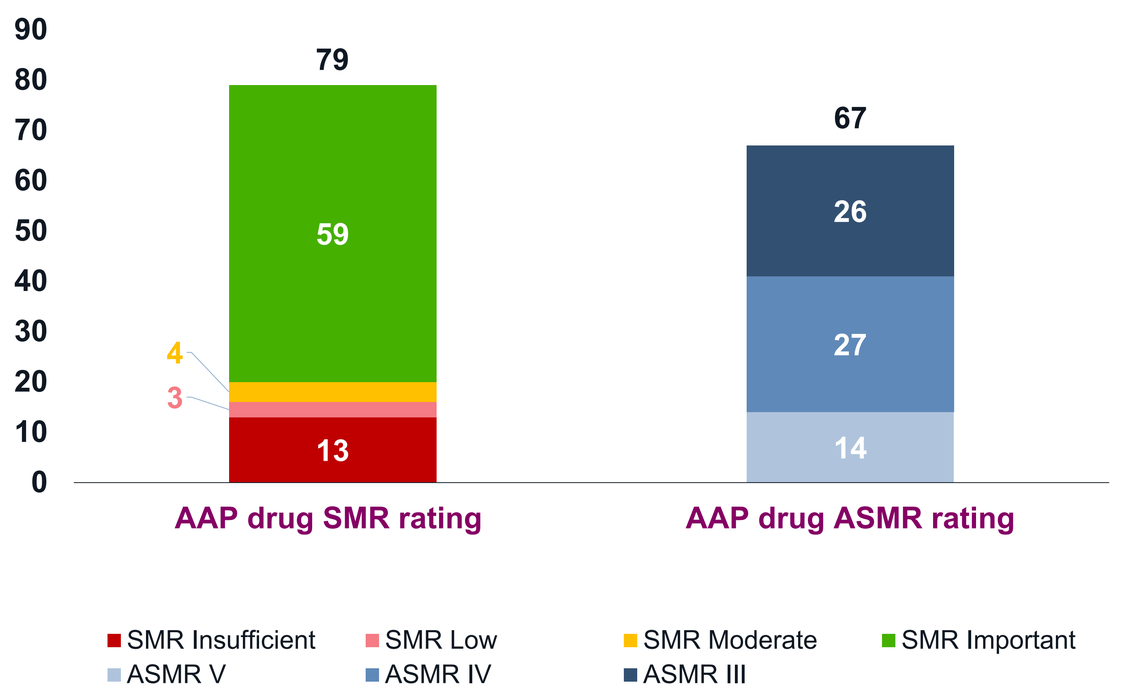
- **Reimbursement outcomes**
- **RWE utilization from AAP/EAMS schemes**
- **Types of RWE utilized in drug appraisals**

RESULTS

Graph 1. Reimbursement outcome analysis of EAMS drugs (2020-2025)

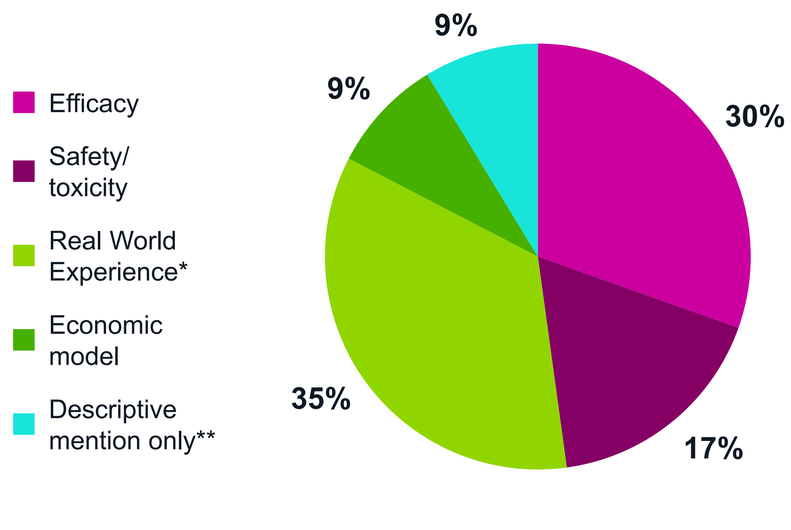


Graph 2. Reimbursement outcome analysis of AAP drugs (2021-2025)

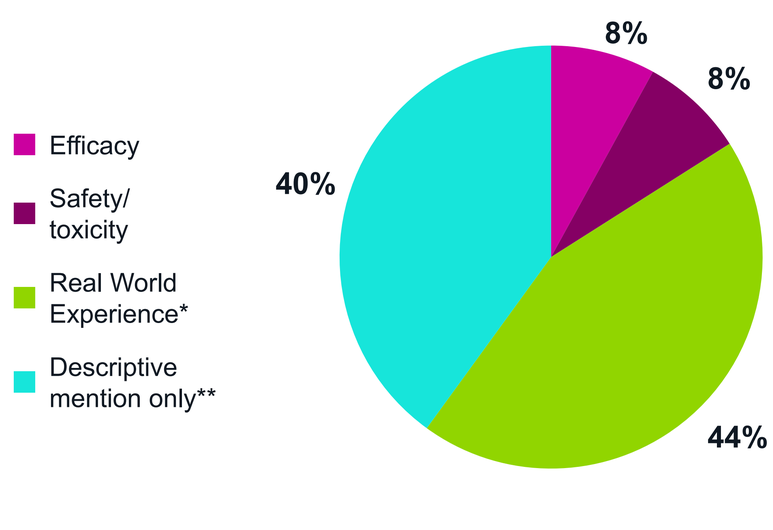


- Analysis of reimbursement outcomes of EAMS drugs reveals that ~83% of EAMS drugs received a NICE recommendation (full or restricted), with ~52% of appraisals referencing EAMS data, but only ~22% of them making it to final NICE guidance, suggesting a modest overall impact in decision-making
- In France, ~75% of fully reimbursed AAP drugs received an SMR Important, while no drugs received SMR Insufficient for the full population, suggesting a potentially positive correlation between AAP scheme enrollment and more favourable HTA outcomes

Graph 3. Proportion of EAMS data category mentions



Graph 4. Proportion of AAP data category mentions



Further analysis of the data categories of EAP evidence highlights the differences in RWE utilization between France and the UK. While NICE appraisals incorporate efficacy and safety data, respective HAS utilization is limited (47% vs 16%). Both agencies leverage real world experience from EAPs (35% and 44%), but HAS is far more likely to limit to a descriptive mention of EAP use as opposed to NICE (40% vs. 9%) respectively.

*Refers to clinician and patient/ caregiver input on experience with EAP drug: **Refers to EAMS only mentioned as an access pathway utilized with no further details and/or data provided

SUMMARY & CONCLUSIONS

- Whilst UK and FR EAP schemes are frequently cited in HTA submissions, these are typically limited to descriptive mentions or supporting advocacy
- Utilization of real-world effectiveness or safety data is much less common and even where these are used, they infrequently make it into the final appraisal document that summarizes the key decision and decision-drivers, indicating relatively low impact of such data
- Use of real-world effectiveness data from EAP schemes in HTA submissions may be hampered by challenges in data collection, EAP schemes being often for very unwell patients who have no other options who may response less well to therapeutic interventions, and the relatively short time these schemes are in operation for

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