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Real-World Evidence (RWE) and its impact on Scottish Medicines Consortium (SMC) decisions: A retrospective review

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Introduction

SMC advises NHSScotland on the value of newly licensed medicines. Recognising that randomised controlled trials (RCTs) are not always feasible, particularly for rare/ultra-rare or rapidly evolving therapeutic areas, SMC acknowledges that RWE can complement early-phase or single arm studies. This project examined how RWE has been used in SMC submissions, and its influence on SMC decision-making.

Methods

A retrospective review (2018-2025) of all company submissions containing RWE was conducted. Eligible submissions specified use of RWE (control cohort studies, patient's registry, observational studies). Submissions were excluded if SMC advice was unpublished, deferred or withdrawn. Submission characteristics including therapeutic area, prevalence of disease, source and purpose of RWE were collected from SMC's internal database. A descriptive analysis was conducted to explore a correlation between the use of RWE and SMC's final decision.

Results

Of 675 submissions, 55 contained RWE; 39 with published SMC advice were analysed. RWE use in submissions peaked in 2022 (n= 10) as shown in Figure 1. The majority (79%) of submissions were for orphan (n= 23) or ultra-orphan (n= 8) conditions as shown in Figure 2; oncology (n= 14) and rare genetic conditions (n= 13) were the most common therapeutic areas (Figure 3). Patients' registries and observational cohort studies were the most common sources of RWE used to support the evidence. Base-case analyses were comprised of phase I/II studies (n= 19), patients' registry (n= 6), indirect comparisons (n= 12) or RCT (n= 7). SMC Committee accepted for use or restricted use 26 (67%) of the 39 submissions.

Figure 1: Trends in inclusion of RWE in submissions (2018 to 2024)

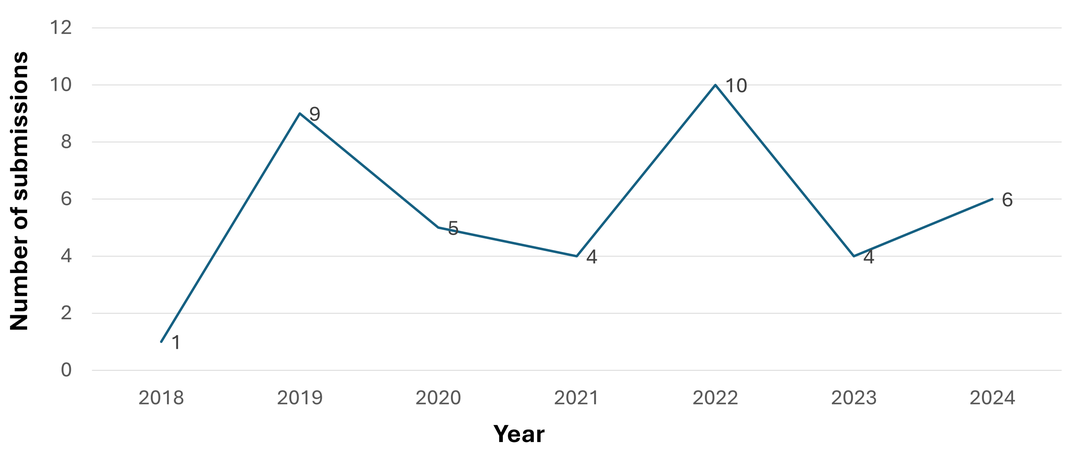


Figure 2: SMC process category in submissions with RWE (n=39)

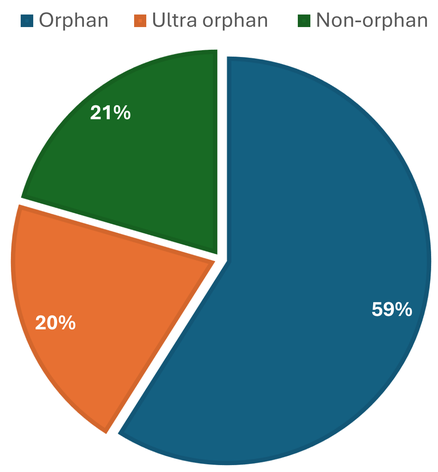
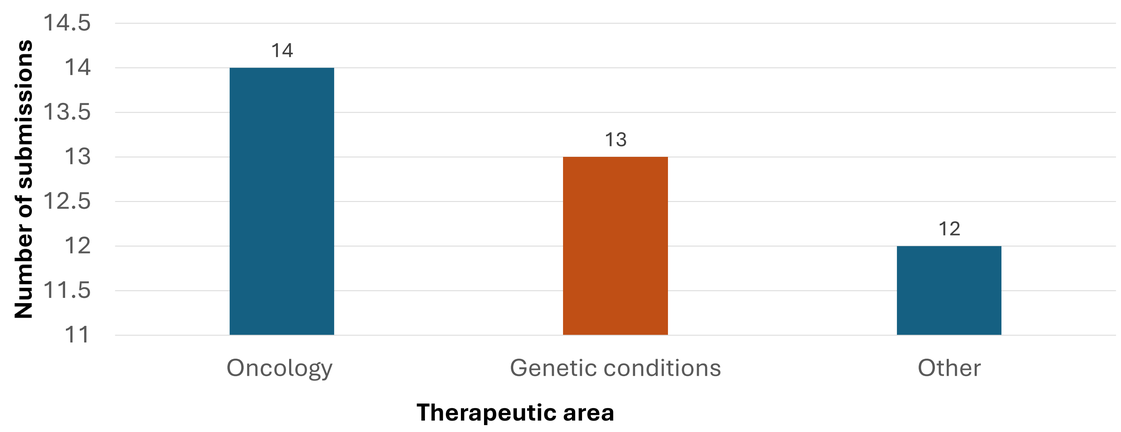


Figure 3: Therapeutic areas in submissions with RWE



Conclusion

- Use of RWE in SMC submissions has increased since 2018, providing additional evidence in areas where clinical trial data have limitations, especially for conditions with high unmet need such as rare diseases and oncology.
- SMC recognises the value of RWE to inform the health technology assessment of medicines and has recently published a position statement on its use.

References:

1. SMC Real World Evidence Position Statement

NHS SCOTLAND