HTA136

Impact on Scottish Medicines Consortium reimbursement decisions when assessed under the orphan drug process

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Background

Orphan and ultra-orphan drugs are used in the diagnosis, treatment, or prevention of orphan diseases.[1] Orphan diseases are life-threatening or rare conditions which affect a very small percentage of the population. As per the World Health Organization (WHO), orphan diseases affect < 6 out of 10,000 persons. Ultra-orphan drugs are those used to treat chronic and severely disabling, rare, or orphan diseases with a prevalence of ≤ 1/50,000 persons in Scotland.[2] Pharmaceutical companies tend to show less interest in medicine development for the treatment of orphan diseases due to their small patient populations[3,4] and, therefore, less potential for company earnings and shareholder investment returns. Due to the unprofitable nature of orphan medicine development, there is a need for government and regulatory authorities' attention.

Objective

The objective of this observational study is to evaluate the impact of orphan drug criteria on Scottish Medicines Consortium (SMC) reimbursement decisions for health technology assessments (HTAs).

Methods

SMC reimbursement decisions between 2012 and 2022 for oncological conditions containing orphan drug criteria comprised the data set. HTAs with positive and negative decisions were included while HTAs with no decision were excluded. A total of 301 HTAs were included: 184 were recommended and 117 were not recommended.

Results

Among the 301 HTAs, 41.86% (n = 126) were assessed under the orphan drug process whereas 58.14% (n = 175) were not studied **(see Table 2 and Figure 1)**. Among the HTAs with a positive decision, 24.25% (n = 73) were assessed under the orphan drug process, while 36.88% (n = 111) were not studied **(see Table 1 and 2, and Figure 2)**. Similarly, among the HTAs with a negative decision, 17.61% (n = 53) were assessed under the orphan drug process while 21.26% (n = 64) were not studied **(see Table 1 and 2, and Figure 2)**.

Table 1: Percentage of Recommend and Do not recommend decisions based on orphan criteria

Orphan Criteria	Recommended (%)	Do not recommend (%)
YES	24.25	17.61
NO	36.88	21.26
Total	100	

Table 2: Total number of HTAs based on orphan criteria

Orphan Criteria	Recommended	Do not recommend
YES	73	53
NO	111	64
Total	301	

Figure 1: Orphan criteria

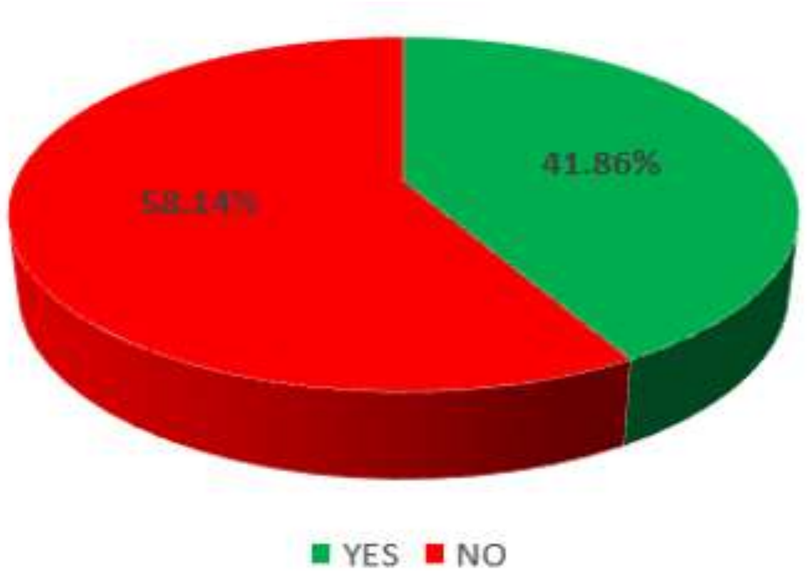
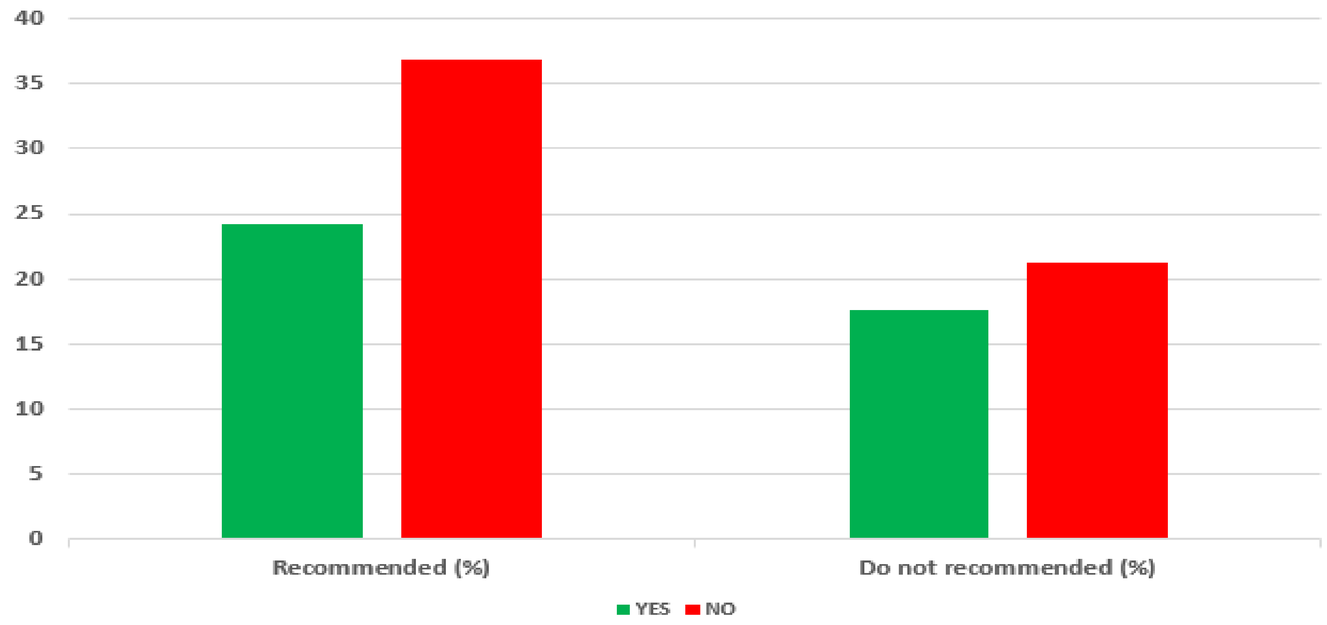


Figure 2: Orphan criteria based on reimbursement decision



Conclusion

Based on observations of this data, it can be deduced that most of the oncology HTAs with a positive reimbursement decision were not assessed through the orphan drug process. Therefore, it can be concluded that reimbursement decisions made by SMC for oncology HTAs are not impacted by the orphan drug criteria.

References

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