Investigating the influence of therapy-related factors on reimbursement success: A multi-country analysis

HTA12

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SUMMARY

OBJECTIVES

- Successful health technology assessment (HTA) is fundamental in achieving patient access for new medicines in global markets. HTA outcomes can offer differ by country and are intrinsically related to product characteristics.
- The objectives of this study were to identify therapy-related factors that influence reimbursement success across seven countries with universal healthcare systems, and to examine any between-country differences.

METHODS

- HTA appraisals conducted between January and December 2023 in seven countries with universal healthcare systems were reviewed. Data specific to each HTA decision was collected.
- Univariable and multivariable logistic regression models were used to examine the relationship between therapeutic area, therapy type, advanced therapy medicinal products (ATMP) status, orphan designation, target patient age group and reimbursement decision.
- Subgroup analysis were conducted to determine whether relationships differed according to country.

FINDINGS

- Two of the examined therapeutic factors were significantly associated with reimbursement decision:
  - Indicated age-group, with the odds of reimbursement success being greater for products with paediatric indications.
  - Therapeutic area, with greater odds of reimbursement success in non-oncological treatments.
- Associations between significant therapy-related factors and reimbursement success varied according to country.
- Oncology products tend to be more frequently reimbursed in markets which consider the unique complexities of oncology as part of their HTA process.

BACKGROUND & AIMS

- Health technology assessments (HTAs) are a fundamental part of achieving patient access to new therapies. They require the presentation of evidence associated with the clinical and/or cost-effectiveness of a therapy for an indication.
- The criteria for recommendation of therapies differ among countries, owing to various factors, such as the healthcare budget, whether an orphan drug or end of life policy exists, the extent of the unmet need, and whether countries have a social health insurance policy or a tax-based healthcare system [1,2].
- Our objective was to explore the relationship between therapy-related factors on reimbursement outcomes in seven countries with universal healthcare systems, and to examine any differences between these countries.

METHODS

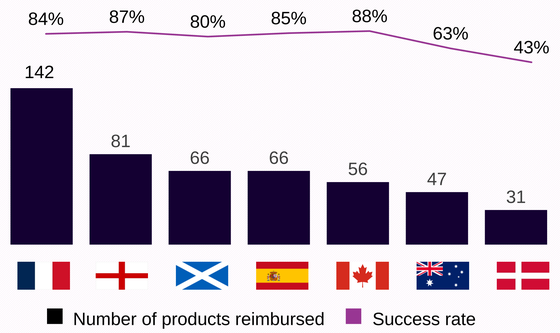
- HTA recommendations from seven countries with universal healthcare coverage were reviewed: England, Scotland, France, Denmark, Spain, Australia, and Canada (**Figure 1**).
- Data was collected on date of recommendation, type of HTA, reimbursement decision, indication, therapeutic area (oncology or other), therapy type (mono or combined therapy), advanced therapy medicinal product (ATMP) status (Yes or No), orphan drug designation (Yes or No) and target age group (any age, children, or adults).
- Univariable and multivariable logistic regression models were used to examine the relationship between each of the factors and reimbursement outcome. Country-specific analysis were also performed. Results were presented as odds ratios (OR) with corresponding 95% confidence intervals (CI).

Figure 1. Countries included in analysis

RESULTS

- In 2023, a total of 634 HTA appraisals were conducted across the seven countries, of which 489 (77%) resulted in a positive reimbursement recommendation.
- **Figure 2** shows the total number of products approved for reimbursement across each of the analysis countries and the reimbursement success rate. The country with the highest successful reimbursement rate was Canada (88%) and the country with the lowest was Denmark (43%).

Figure 2. Number of products approved for reimbursement across analysis countries in 2023.



- Univariable analyses demonstrated that two of the examined therapeutic factors assessed were significantly associated with reimbursement decision, including indicated age group, with the odds of reimbursement success being 5.65 times higher in paediatric indications (95% CI: 1.97, 23.82), and therapeutic area, with oncology products being less likely to be reimbursed than other disease areas (OR: 0.65 [95% CI: 0.44, 0.94]).
- These associations remained after adjusting for all other factors in the model (**Table 1**).
- When examining country-specific associations:
  - Rates of successful reimbursement were higher for non-oncological products in Denmark, England, France, and Spain, but lower in Canada.
  - Rates of successful reimbursement were higher for products with paediatric indications in all countries, bar Scotland and Spain.

Table 1. Results from univariable and multivariable regression analyses

Variable	Variable type	Reference category	Univariable		Multivariable	
			OR (95% CI)	p-value	OR (95% CI)	p-value
Therapeutic area	Binary (oncology or other)	Other disease area	0.65 (0.44, 0.94)	0.021*	0.64 (0.43, 0.95)	0.027*
Therapy type	Binary (mono or combo-therapy)	Monotherapy	0.94 (0.91, 1.47)	0.770	1.18 (0.74, 1.90)	0.496
ATMP status	Binary (Yes or No)	No	1.26 (0.50, 3.82)	0.652	1.75 (0.65, 5.59)	0.295
Orphan drug indication	Binary (Yes or No)	No	0.76 (0.49, 1.22)	0.248	0.71 (0.44, 1.15)	0.154
Target age group	Categorical (any, children, or adults)	Any age	Adults: 1.21 (0.82, 1.77) Children: 5.65 (1.97, 23.82)	0.334 0.005*	Adults: 1.24 (0.84 1.82) Children: 5.40 (1.87, 22.88)	0.281 0.006*

Abbreviations: ATMP, advanced therapy medicinal products; CI, confidence interval; OR odds ratio. * Significant at a level of p=0.05.

DISCUSSION

- Therapeutic area was identified as one of the therapy-related factors associated with reimbursement success: non-oncological products are more likely to be reimbursed.
- Often oncological patients have substantially reduced life expectancy versus the general population. Oncology trials predominantly focus on survival benefit, in which minor improvements in survival can have a large impact on patients.
- Most markets do not make special considerations for oncological products. Therefore, the benefit of incremental improvements in survival for people with oncological diseases may be underestimated, contributing to the divergence in HTA outcomes between oncological and non-oncological products.
- When examining the reimbursement success rates of oncological products across markets, it was found that markets which consider the unique complexities of oncology as part of their HTA process – i.e., offering separate assessment processes in some cases (Pan-Canadian Oncology Drug Review), increasing cost per QALY thresholds in others (severity modifier - England), or providing outcome-based managed entry agreements (Cancer Drugs Fund - England) – have a greater likelihood of success for oncological products (88%) when compared to markets that do not make any formal considerations (68%). In addition, these markets have a reduced timeframe for patients to achieve access to new products (339 days vs 397 days) [3].
- Improved reimbursements outcomes were also observed for products with paediatric indications. Historically, there has been a lack of innovation for medicines in the paediatric population [4]. However, since the introduction of the Paediatric Regulation (2006), the number of new medicines/indications receiving authorisation for use in children has increased substantially [4].
- Potential drivers of the higher reimbursement success rate for paediatric products include: (1) political influences, with increased lobbying and advocacy for childhood diseases, (2) economic benefits, due to additional considerations of caregiver disutilities and costs, (3) clinical benefits, with greater potential for the accrual of long-term benefits associated with treating conditions early, and (4) perceived higher unmet need, leading to greater tolerance for uncertainties.
- Further research is required to corroborate our findings.

References

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