A comparative analysis of reimbursement outcomes for orphan drugs between EU and non-EU countries.

HTA165

Sarah-Jane Lyttle[1], Tiyi Morris[1], Harry Lewis[1], Andrew Mumford[1,2], Rachael Piper
1. Initiate Consultancy, London, UK. 2. Initiate Consultancy, Zug, Switzerland.

SUMMARY

OBJECTIVES

- Orphan designation is granted in the European Union (EU) by the European Medicines Agency (EMA) and subsequently recognised by all EU member states and may be granted separately by non-EU countries.
- In this study, we examined the relationship between orphan drug status and reimbursement outcome and assessed any differences between the likelihood of reimbursement in EU and non-EU countries.

METHODS

- HTA recommendations published in 2023 for eight countries were reviewed to determine whether there were differences in the likelihood of successful reimbursement recommendations between EU and non-EU countries for orphan and non-orphan drugs.
- Chi-squared tests (χ^2) were used to examine the association between orphan drug status and successful reimbursement, and between EU membership decisions across the eight countries and successful reimbursement.

FINDINGS

- Our findings suggest the overall and non-orphan reimbursement rates are consistent regardless of EU-membership, and slightly higher for orphan drugs in non-EU countries.
- EMA orphan status had a statistically significant impact on reimbursement outcomes across all 8 countries in the study, in the EU cohort, and in the non-EU cohort.

BACKGROUND & AIMS

- Orphan drugs typically face unique challenges in securing reimbursement, due to high-costs and evidence limitations.
- European Medicines Agency (EMA)[1] grant orphan drug designation to drugs that treat <5/10,000 people. The product must also provide significant benefit to patients affected by the disease in order for orphan designation to be granted.
- EMA orphan drug designation is recognised by all EU countries, and some will provide alternative or modified processes to support reimbursement of orphan drugs.
- Some non-EU countries have their own orphan drug designation and reimbursement pathways. In Australia orphan drug designation is granted under similar criteria for products assessed in Australia. In England and Scotland have separate processes for orphan drugs. However. In Canda there is no such orphan designation available.
- In this study we examined the relationship between orphan drug status and reimbursement outcome, and looked at differences between the likelihood of reimbursement in EU and non-EU countries.

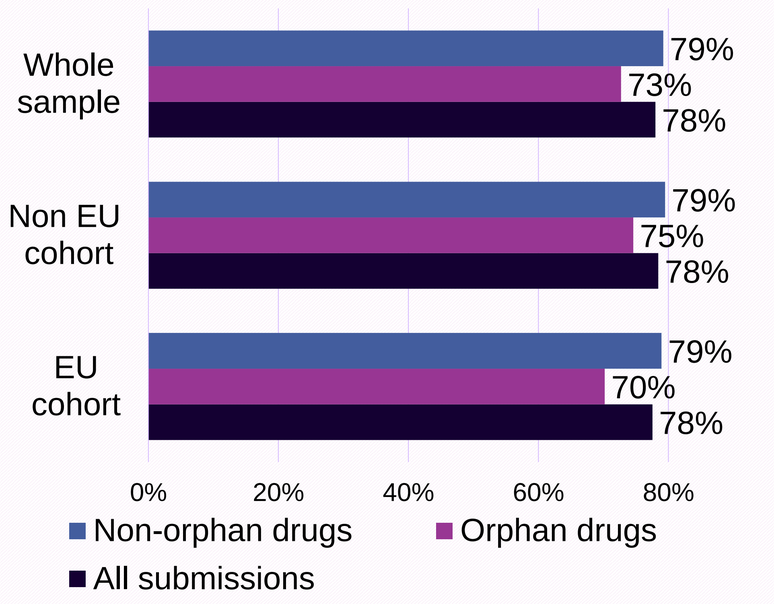
METHODS

- HTA decisions published in 2023 in 8 countries were reviewed, using data collected as part of Initiate's 2023 Reimbursement Radar[2]; a database of HTA and access outcomes across 13 global HTA markets.
- Decisions from standard HTAs and orphan drug-specific HTAs were included in the analysis.
- The countries in the analysis included EU countries: France, Ireland, Spain, and Sweden; and non-EU countries: England, Scotland, Australia, and Canada.
- Data was extracted regarding HTA appraisal type, reimbursement decision, and EMA orphan-designation status.
- The reimbursement success rate was calculated by EMA orphan drug status for the overall sample, EU and non-EU cohorts, and by countries.
- Chi-squared tests (χ^2) were used to examine the association between orphan drug status and successful reimbursement, and between EU membership decisions across the eight countries and successful reimbursement.

RESULTS

- **Figure 1** displays the reimbursement success rates across the whole sample, EU cohort, and non-EU countries.
- Overall, the likelihood of successful reimbursement for all 2023 submissions was the same for the whole sample, the EU cohort, and the non-EU cohort (78%).
- Similarly, the reimbursement success rate for non-orphan drugs was the same in the EU and non-EU cohorts (79%).
- The reimbursement success rate only differed between EU and non-EU countries for orphan drugs, which was lower in EU countries (70% versus 75% respectively).

Figure 1. Successful reimbursement rates



- However, Chi-squared testing revealed reimbursement outcomes for orphan and non-orphan drugs are likely to be independent of EU membership (χ^2=2.6; p=0.1).
- **Table 1** presents the reimbursement success rate by country for all submissions, orphan, and non-orphan submissions.
- Examining the reimbursement success rates across countries revealed that France had the highest likelihood of reimbursement and Ireland the lowest, regardless of orphan status.
- EMA orphan status had a significant impact on reimbursement outcome across all 8 countries, EU countries, and non-EU countries (χ^2 =15.6, 142.1, and 125.5, respectively; p<0.0001 for all groups).
- It is important to note orphan drug designation granted in Australia appears to align with EMA.
- EU membership did not have a statistically significant effect on reimbursement outcome across all 8 countries (χ^2 =0.002; p=0.97).

Table 1. Reimbursement success rate by orphan drug status

	All submissions		Orphan drug submissions		Non-orphan drug submissions	
	n	Reimbursement success rate	n	Reimbursement success rate	n	Reimbursement success rate
Whole sample	**591**	**78%**	**110**	**73%**	**481**	**79%**
Non-EU cohort	**297**	**78%**	**63**	**75%**	**234**	**79%**
England	93	87%	23	87%	70	87%
Scotland	65	75%	16	75%	49	76%
Australia	75	63%	10	20%	65	69%
Canada	64	88%	14	93%	50	86%
EU cohort	**294**	**78%**	**47**	**70%**	**247**	**79%**
France	129	90%	11	100%	118	89%
Ireland	32	16%	9	11%	23	17%
Spain	78	85%	19	89%	59	83%
Sweden	55	75%	8	50%	47	79%

CONCLUSIONS

- These data show a large discrepancy in HTA success rates across markets, particularly within the countries in the EU cohort.
- It is important to consider the unique HTA processes of certain markets and the modifications made for orphan drugs. This is likely to have a more significant impact on reimbursement outcomes than EU membership.

References

1. EMA, orphan designation: Overview, https://www.ema.europa.eu/en/human-regulatory/overview/orphan-designation-overview (accessed Nov 2024).
2. Initiate Consultancy, 2023, Reimbursement Radar.

Presented at ISPOR Europe, 17th-20th November 2024
initiateconsultancy.com hello@initiateconsultancy.com