A review and evaluation of reimbursement outcomes for non-orphan and orphan drugs in Ireland in 2023

HTA5

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SUMMARY

OBJECTIVES

- This study aimed to analyse health technology appraisal (HTA) outcomes made by the National Centre of Pharmacoeconomics (NCPE) in 2023 comparing drugs with non-orphan drug status successfully achieving NCPE reimbursement to those with European Medicines Agency (EMA) orphan drug status.
- Identifying any impact that orphan status might have on reimbursement success in Ireland.

METHODS

- Rapid reviews and full HTAs published by NCPE in 2023 were reviewed, with data extracted regarding appraisal type and decision.
- Results were tabulated and descriptive statistics were compiled. Drugs were classified as orphan based on the list of authorised pharmaceuticals with orphan designations obtained from the EMA website.

FINDINGS

- Orphan drugs were reimbursed at a lower rate than non-orphan drugs regardless of reimbursement route.
- Notably, the Irish HTA process is not tailored to the reimbursement of orphan drugs. Patients suffering from rare diseases in Ireland may face setbacks as a result of the adoption of a single, non-tailored approach to assessing drugs.

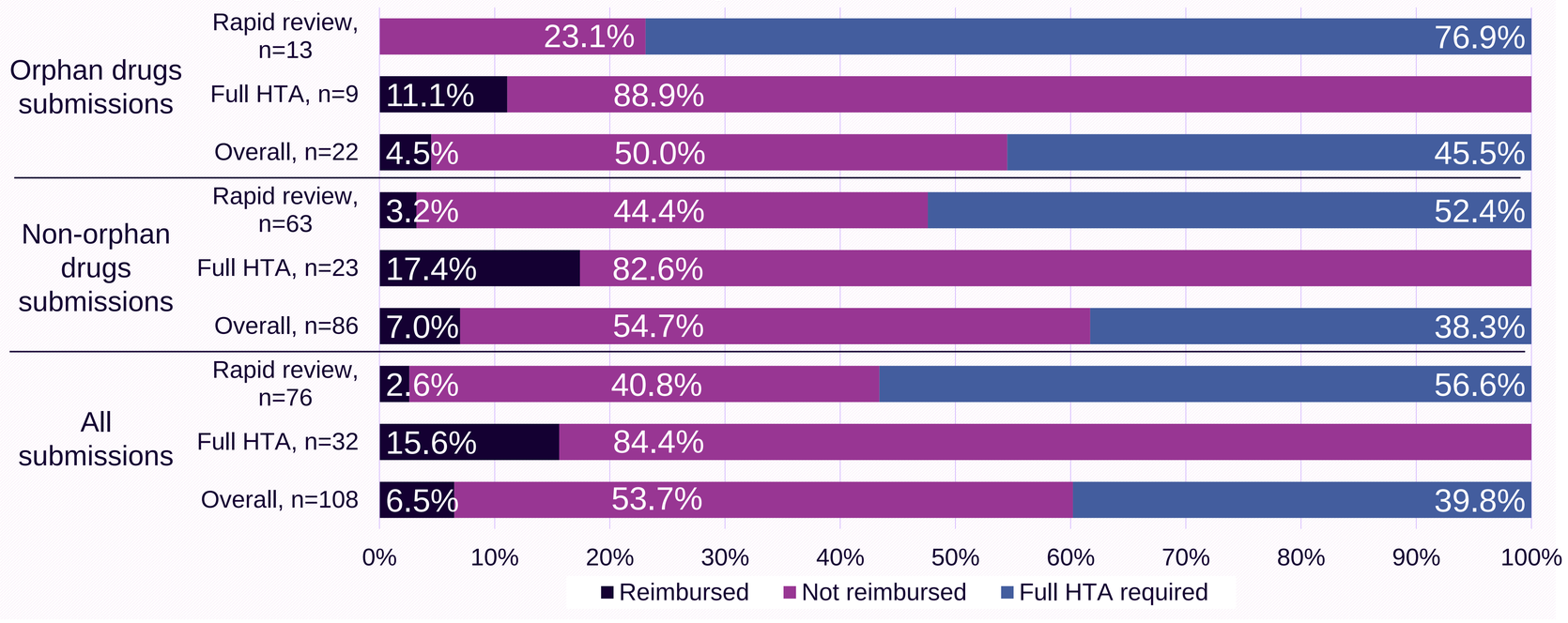
BACKGROUND & AIMS

- In Ireland, HTAs are conducted by the National Centre for Pharmacoeconomics (NCPE)[1].
- Since 2009, the NCPE has allowed recommendations to be made via Rapid Review (full HTA required or full HTA not required) in an effort to enable more appropriate resource prioritisation, although some manufacturers still gain reimbursement following price negotiations with Ireland's Health Service Executive (HSE).
- Orphan drugs typically face unique challenges in securing reimbursement, due to high-costs and evidence limitations. European Medicines Agency (EMA)[2] orphan designation is drugs that treat <5/10,000 people.
- The discrepancy between orphan and non-orphan EMA drug status in terms of reimbursement success is an important consideration.
- The aim of this study is to better understand the impact orphan drug status had on reimbursement outcome in Ireland, taking into consideration the reimbursement route (full HTA versus Rapid Review).
- This research builds on a similar analysis of the year 2022 conducted by the authors and presented at ISPOR US (HTA92).

METHODS

- Rapid Review and full HTA outcomes published by the NCPE were reviewed using data collected as part of Initiate's 2023 Reimbursement Radar[3], a database of global HTA outcomes published access 13 global HTA markets.
- HTA outcomes were reviewed based on reimbursement route, orphan status, and if "improve cost-effectiveness" was the reason given by the NCPE (if the reimbursement decision was negative).
- The list of authorised pharmaceuticals with orphan designations was obtained from the EMA website[2].
- Results were tabulated and descriptive statistics were compiled.

Figure 1. HTA outcomes by orphan-status and reimbursement route



RESULTS

- **Figure 1** summarises the HTA outcomes published in Ireland in 2023.
- A total of 108 submissions (86 non-orphan and 22 orphan) were made in 2023. Of these, 76 were Rapid Reviews and 32 were full HTAs.
- Of all treatments assessed, 5 (15.6%) that underwent a full HTA and 1 (2.6%) that underwent a Rapid Review were reimbursed.
- Of the 75 drugs which were not reimbursed at Rapid Review, 43 (57.3%) were recommended for a full HTA.
- 1 of the 22 (4.5%) orphan drugs reviewed in 2023 was successfully reimbursed after a full HTA, compared to 3 in 2022 (2 through the Rapid Review process).
- 8 (7%) non-orphan drugs were reimbursed in 2023: 2 via the Rapid Review process and 6 after full HTAs.
- **Table 1** summarises the number of HTA submissions not reimbursed and the decision restrictions for improved cost effectiveness.
- 4 orphan submissions compared to 10 non-orphan submissions were reimbursed with the condition of improved cost-effectiveness.
- In Ireland, cost-effectiveness restrictions apply to both reimbursed and not-reimbursed decisions, with 5 reimbursed decisions having a decision restriction for improved cost-effectiveness (1 orphan; 4 non-orphan).

Table 1. Summary of HTA submission outcome and the requirement for improved cost-effectiveness for orphan and non-orphan submissions

		n	Not reimbursed	Improve cost-effectiveness
Orphan submissions	Overall	22	11 (50%)	4
	Full HTA	9	8 (89%)	4
	Rapid review	13	3 (23%)	0
Non-orphan submissions	Overall	86	47 (55%)	10
	Full HTA	23	19 (83%)	8
	Rapid review	63	28 (44%)	2

CONCLUSIONS

- In Ireland in 2022, orphan drugs were approved for reimbursement at a lower rate than non-orphan drugs. This pattern has remained in 2023, regardless of reimbursement route.
- Very few submission were successfully reimbursed through the NCPE's Rapid Review process. There were no orphan drugs which received a positive reimbursement decision.
- While there are several plausible explanations for this, it should be noted that the Irish reimbursement framework does not contain a process specifically tailored to improve access to orphan drugs.
- As such, patients suffering from rare diseases in Ireland may experience setbacks as a consequence of the adoption of NCPE's single, non-tailored approach to assessing drugs.

References

1. *NCPE Website (www.ncpe.ie)*
2. *EMA Website (https://www.ema.europa.eu/en/human-regulatory/overview/orphan-designation-overview)*
3. *Initiate Consultancy, 2023, Reimbursement Radar*

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