# A comparative analysis of the relationship between orphan drug status and reimbursement status in Ireland, Sweden, France, and Canada in 2022

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## Background and objectives

It is estimated that 300 million people worldwide are affected by rare diseases.[1] A drug is defined as 'orphan' by the European Medicines Agency (EMA) if the condition it treats is life-threatening or severely debilitating, the patient prevalence is not more than 5 in 10,000, and the medicine is of significant benefit to those with the condition. Adopting orphan drug policies which optimise orphan drug licensing, pricing, research and development, and reimbursement processes can address the unmet medical need of patients with rare diseases. Some health technology assessment (HTA) organisations, however, opt not to use these frameworks. In this study, the relationship between orphan drug status and its receipt of successful reimbursement in Ireland, Sweden, France, and Canada is examined. The study also looks into whether a country having an orphan drug policy (such as those implemented in Sweden and France) affects access and decision-making regarding reimbursement.

## Methods

A list of EMA-approved medications with current orphan designations was compiled from the EMA website.[2] Information about reimbursement status was taken from the websites of national reimbursement or HTA agencies, along with information regarding the orphan drug policy associated with each national reimbursement framework.

## Results

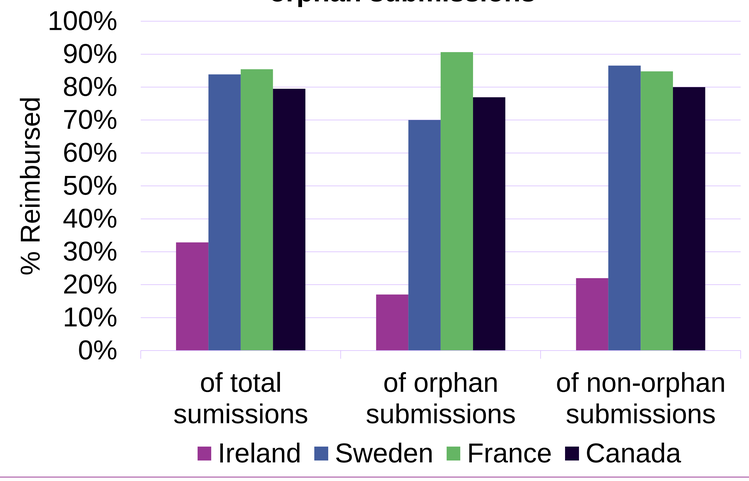
In Sweden, only 62 reimbursement decisions were made in 2022, 10 of which were for orphan drugs (16%) and 52 for non-orphan drugs (84%). In total, nine orphan drugs (90%) achieved either full or limited reimbursement, compared to 43 (83%) of non-orphan drugs.

France, on the other hand, published 230 reimbursement decisions in 2022. Excluding early access submissions, 12 of 15 submissions made for orphan drugs (80%) were successful, compared to 154 of 215 (81%) for non-orphan drugs. When early access programmes were included, this figure reached 19 (86%) for orphan drugs and 171 (82%) for non-orphan drugs.

In total, 107 submissions were made in Ireland in 2022: five of 28 (17%) orphan drugs and 17 of 78 (22%) non-orphan drugs were successful in gaining reimbursement at either Rapid Review or full HTA. In Canada, 83 decisions were made, with 10 of 13 (77%) orphan drugs and 56 of 70 (80%) non-orphan drugs receiving a positive decision.

France and Sweden both have orphan drug policies in contrast with Ireland and Canada. Across France and Sweden combined, 86% of orphan drugs were successfully reimbursed, compared with 36% in Ireland and Canada.

**Successful reimbursement rate of all submissions, orphan submission, and non-orphan submissions**




## Conclusion

Orphan drug reimbursement success rates were higher in countries with an orphan drug policy integrated into their HTA frameworks across the countries considered. Implementation of specific orphan drug policies could help avoid negative consequences for innovation, development, and access to orphan drugs.

Abbreviations: EMA: European Medicines Agency, HTA: health technology appraisal.

References: 1. Nguengang Wakap et al. 2020. 'Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database', European Journal of Human Genetics, 28: 165-73. 2. EMA Orphan Designation. https://www.ema.europa.eu/en/human-regulatory/overview/orphan-designation-overview

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