HTA246

# Improving Access to Innovative Therapies for Rare Bleeding Disorders: Evidence From High Income Countries

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## Background

- Fragmented HTA criteria and regional funding limits create reimbursement barriers that restrict access to critical therapies, particularly innovative treatments for rare bleeding disorders (RBDs).
- RBDs account for approximately 5% of hereditary bleeding disorders, though undiagnosed cases likely increase this figure.[1]
- In high-income countries, access to innovative therapies varies, with wealthier regions—such as certain Canadian provinces and Germany—offering broader coverage than lower-budget areas like Eastern Europe.[2,3]

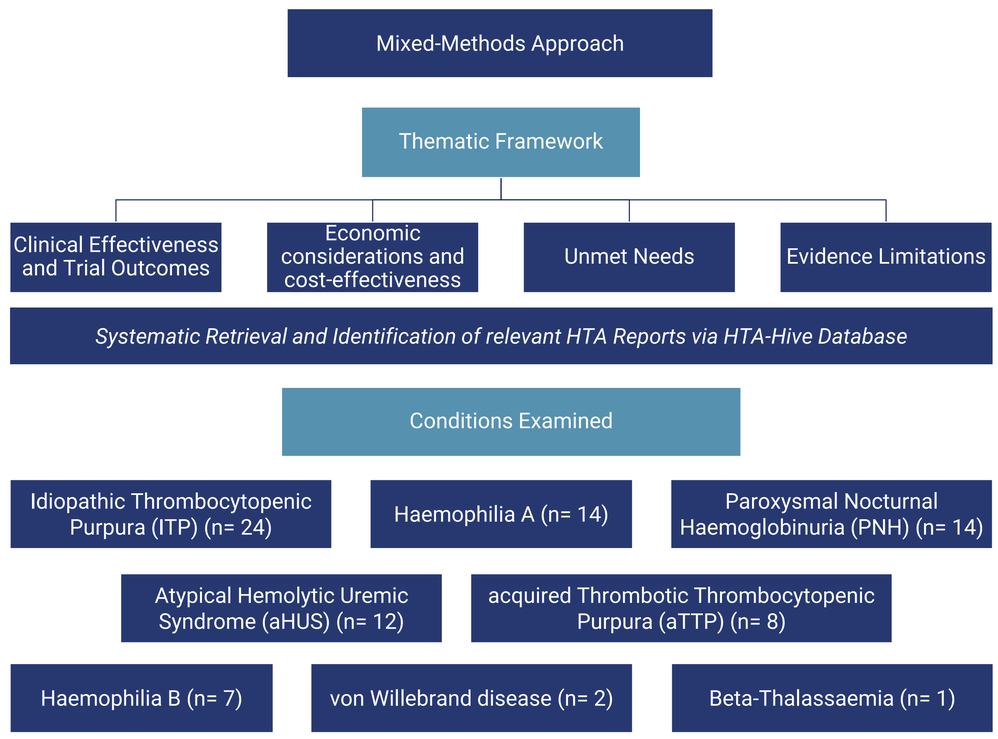
## Objectives

- This study investigates factors influencing reimbursement for innovative therapies in RBDs.
- It explores the roles of clinical development, regulatory approval, HTAs and payer perspectives.
- The goal is to understand how these elements interact to impact reimbursement decisions.

## Methods

- A mixed-methods approach using a thematic framework was applied to systematically review 83 HTA reports from England, Scotland, France, Germany, and Canada, identifying 8 RBDs (Figure 1).
- Key variables extracted included HTA outcomes, added benefit, main decision rationales, clinical and economic restrictions, comparative effectiveness evidence (e.g., clinical trials, ITCs, RWE), unmet medical needs and evidence uncertainties.
- Conditions like polycythaemia vera, beta-thalassaemia-associated anaemia, and anticoagulant reversal, along with minor HTA submissions (e.g., new dosage forms, abbreviated submissions), were excluded to ensure a targeted, focused analysis.

**Figure 1. Study Methodology**

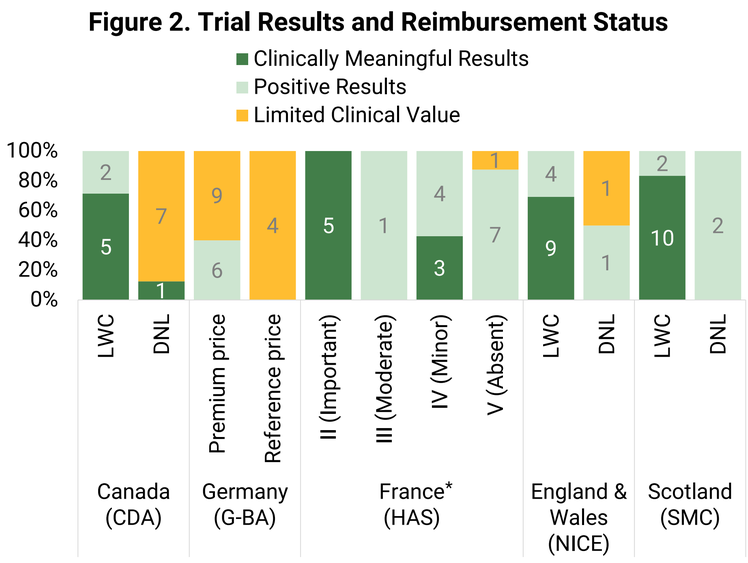



## RESULTS

Preliminary findings identified several factors influencing reimbursement outcomes:

- Positive trial outcomes, along with a preference for clinical endpoints over surrogate endpoints, are crucial for reimbursement success. (Figures 2 and 3)
- Weak study design strategies, unsuitable comparators, and limited robustness of ITCs and RWE were linked to negative outcomes. (Figure 4)
- High treatment costs necessitated substantial price reductions to secure approvals; however, PASs, MAAs, and cost-minimization strategies positively influenced decisions.
- Addressing specific patient needs, such as less frequent dosing, reduced transfusion requirements, and options for oral administration, improved HTA and reimbursement outcomes. (Figure 5)

**Figure 2. Trial Results and Reimbursement Status**




*ASMR levels

**Figure 3. Distribution of Endpoints and Associated Reimbursement Status in Agency Decision Rationales**

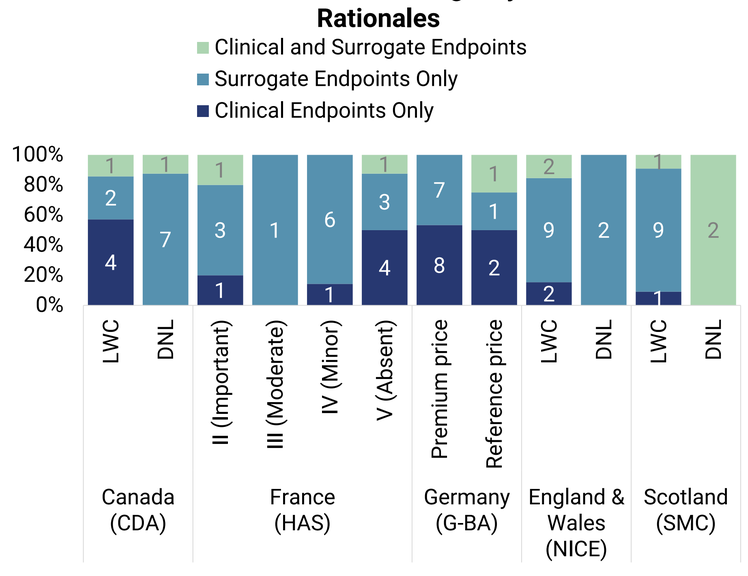



**Figure 4. Reimbursement Barriers Across HTA Agencies**

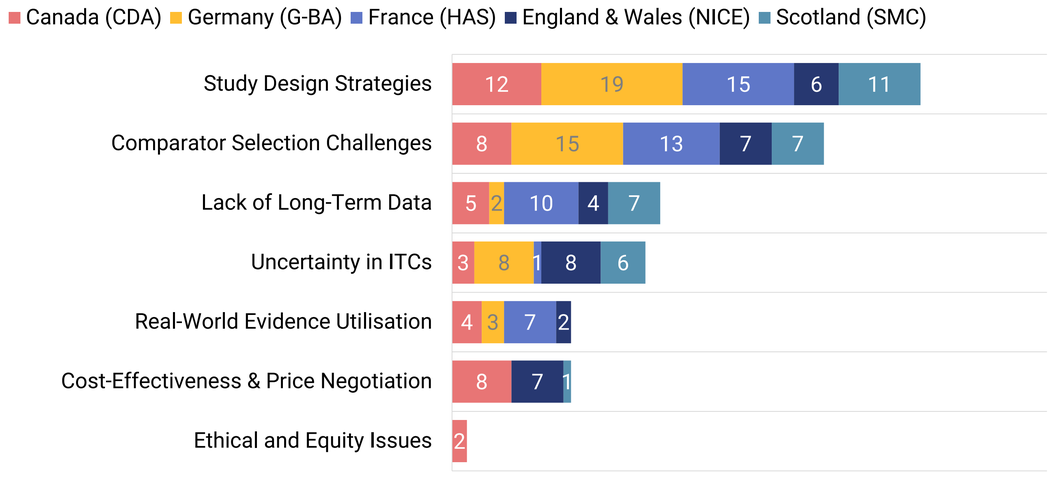
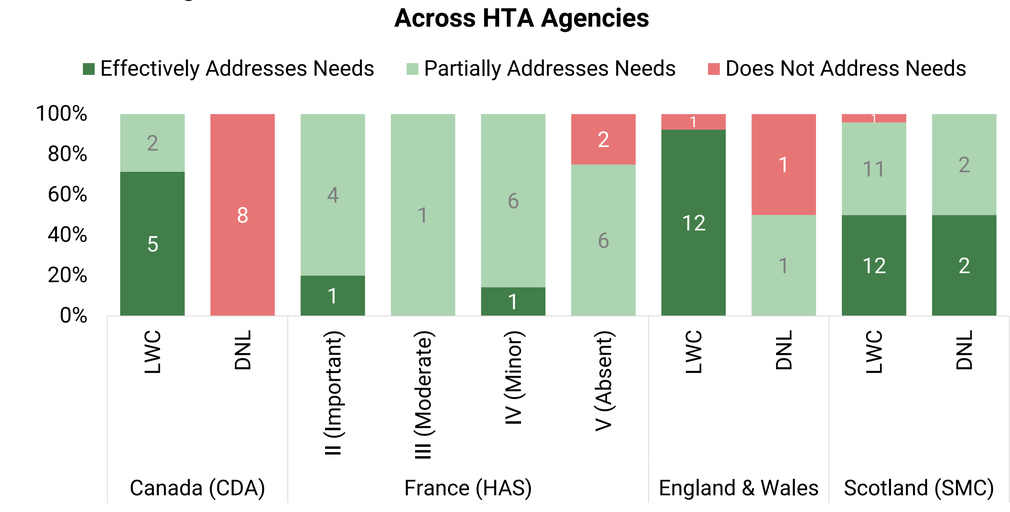



**Figure 5. Unmet Needs Fulfillment and Reimbursement Status Across HTA Agencies**




## Conclusions

- Robust study designs focused on clinical endpoints significantly enhance the likelihood of positive reimbursement outcomes.
- Gene therapies present unique logistical challenges, which may raise ethical and equity issues that impact accessibility and fair distribution among patients.
- High treatment costs remain a barrier; however, PASs and cost-minimization strategies can positively impact decision-making.
- Advances in HTA methods, such as the CDA's "Time-Limited Reimbursement Recommendations" offer promising pathways to improve accessibility and affordability.[4]
- These advancements are essential for ensuring timely and effective treatment for patients with RBDs.

## References

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2. Stevenson, H. (2023). Specific, fair, and transparent: A Canadian process for funding drugs for rare diseases. Healthcarepapers, 21(1), 28-33.
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4. Canada's Drug Agency. (2023). Overview of health technology assessment processes for time-limited recommendations. CDA-AMC

## Abbreviations

Amélioration du Service Médical Rendu (Improvement of the Medical Benefit), ASMR; Indirect Treatment Comparisons, ITCs; Canada's Drug Agency, CDA; Gemeinsamer Bundesausschuss (Federal Joint Committee), G-BA; Haute Autorité de Santé (French National Authority for Health), HAS; Listed with Criteria, LWC; Do Not List, DNL; Managed access agreements, MAAs; National Institute for Health and Care Excellence, NICE; Patient access schemes, PASs; Real-world Evidence, RWE; Scottish Medicines Consortium, SMC

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