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# Health Technology Assessment Recommendations for Pharmaceutical Drugs Submitted in Blood Disorders in CDA and INESSS: Focus on Rare Diseases

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## INTRODUCTION

- To improve patient equity and access to drugs for rare diseases, the 'National Strategy for Drugs for Rare Diseases (DRDs)' initiative was implemented by the Government of Canada, which is expected to enhance access to existing drugs and new emerging treatments in rare diseases.[1]
- As part of the initiatives, CDA has established a non-sponsored review pathway to provide public drug programs with advice on older therapies for rare conditions that were not filed for review by the manufacturer.
- While access to screening and treatments for blood disorders like sickle cell disease have improved, barriers in accessing novel treatments for rare diseases have been a challenge in Canada; in particular, traditional health technology assessment (HTA) processes are often challenged by limitations in clinical evidence and cost-effectiveness associated with DRDs due to small patient populations.[2]

## OBJECTIVES

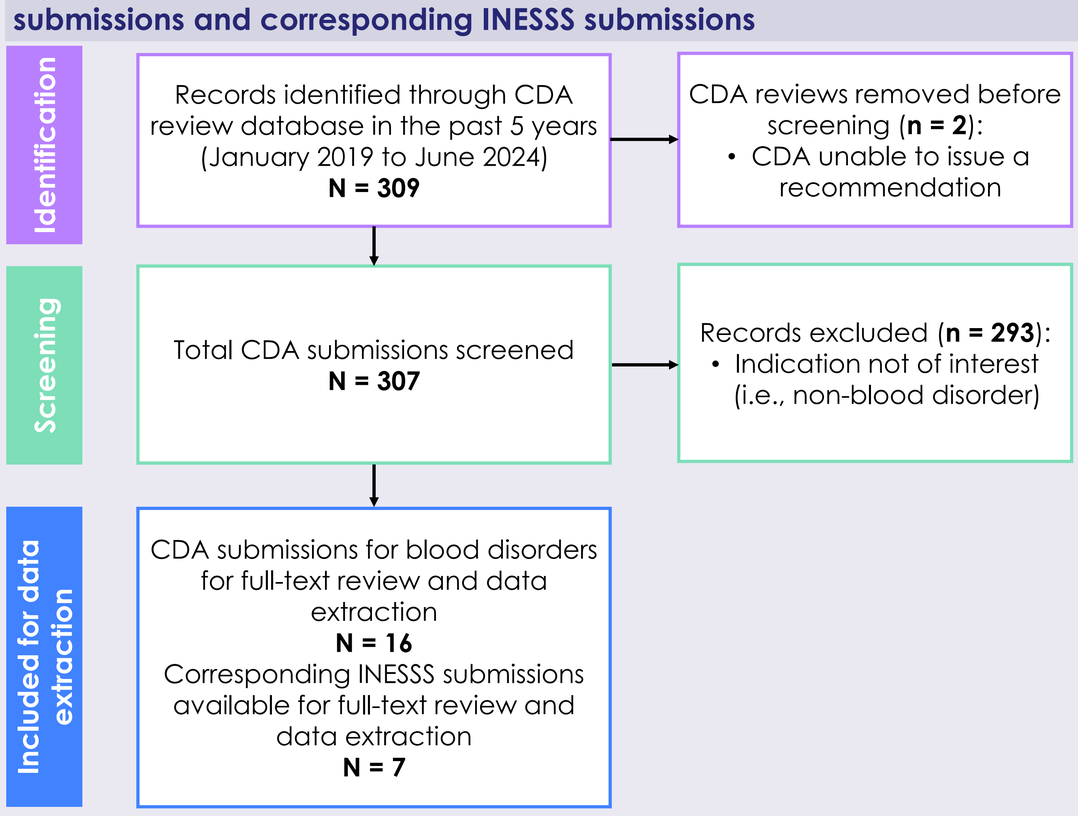
- To gain an understanding of reimbursement decisions in treatments for blood disorders, we reviewed and characterized Canadian HTA submissions with a focus on rare diseases.

## METHODS

- Submissions for blood disorder drugs (i.e., non-oncological indications such as bleeding and blood cell disorders), with final recommendations published Jan. 2019 to June 2024 were retrieved from the CDA website.[3] CDA final recommendation reports were reviewed by two independent investigators to extract information on:
  - Drug under review (e.g., brand and generic name, indication),
  - Submission details (e.g., submission status, final reimbursement decision),
  - Clinical evidence deliberated by CDA (e.g., details of pivotal trial submitted, indirect treatment comparisons included in submission), and
  - Committee commentaries (e.g., rationale for recommendation).
- The corresponding INESSS recommendation reports for these blood disorder submissions were retrieved from the INESSS website[4] and reviewed to extract information on the final reimbursement decision by INESSS, the Minister of Health and Social Services' decision for listing the medication for reimbursement, and rationale for recommendation.
  - Any discrepancies between INESSS and CDA reimbursement decisions were noted.

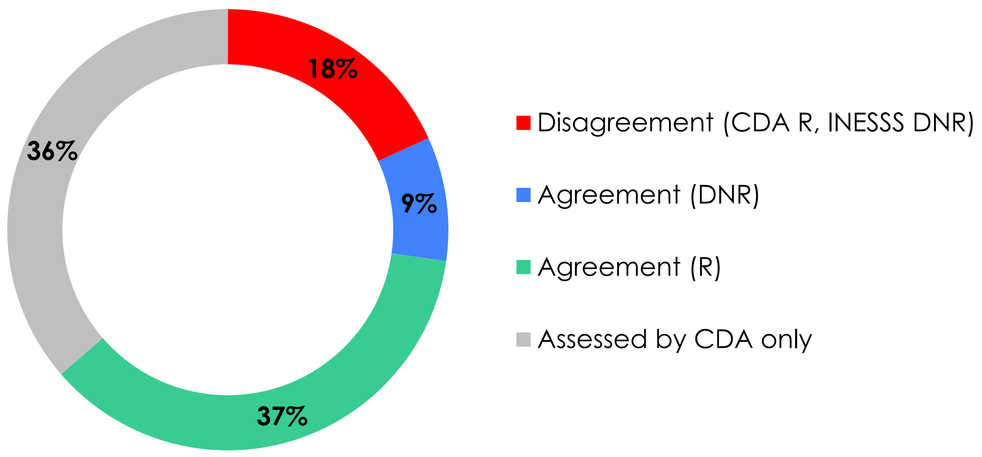
## RESULTS

**Figure 1. Identification and selection process for inclusion of CDA submissions and corresponding INESSS submissions**



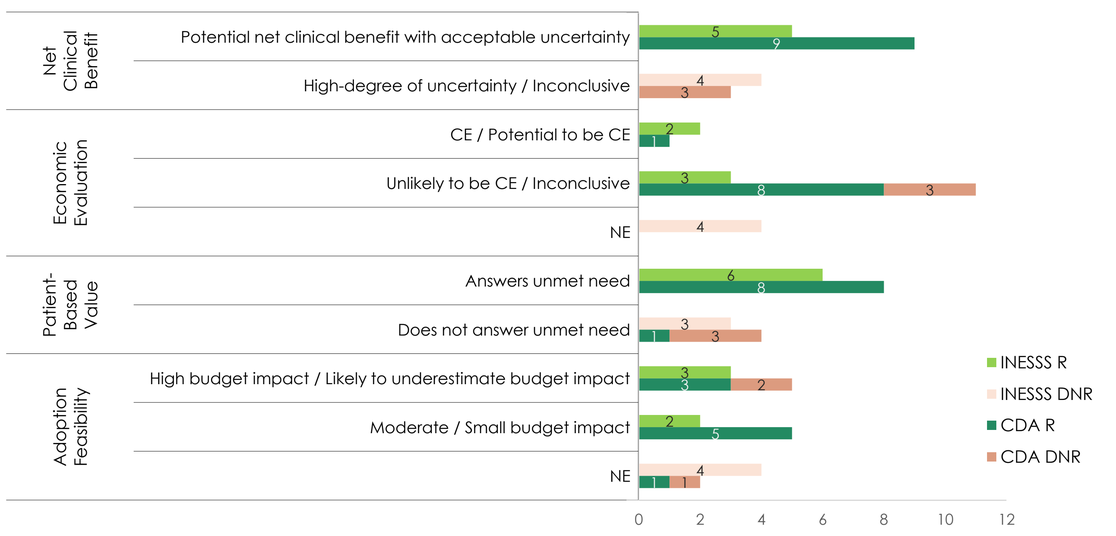

Abbreviations: CDA: Canada's Drug Agency; INESSS: Institut national d'excellence en santé et services sociaux

**Figure 2. Agreement and disagreement between CDA and INESSS reimbursement decisions on rare blood disorder products (N = 11)**




Abbreviations: CDA: Canada's Drug Agency; DNR: do not reimburse; INESSS: Institut national d'excellence en santé et services sociaux; R: reimburse with conditions

**Figure 3. Summary of reasons for CDA/INESSS recommendation and rejection (N = 21 reports)**




Abbreviations: CE: cost-effective; CDA: Canada's Drug Agency; DNR: do not reimburse; HE: health economics; INESSS: Institut national d'excellence en santé et services sociaux; NE: not evaluated; R: reimburse with conditions

### Overall submissions

- Of 309 submissions assessed by CDA, with recommendations published Jan. 2019–June 2024, 16 were for blood disorder-related therapies, including 11 submissions and one resubmission for rare blood disorders (**Fig. 1**).
  - Seven of these products were also assessed by INESSS during this period.

### Reimbursement decisions

- Of the 11 rare blood disorder products evaluated, two (caplacizumab, avacopan) received a negative reimbursement decision by CDA, of which one (caplacizumab) also received a negative decision from INESSS.
  - Caplacizumab received a negative decision from both INESSS and CDA and was resubmitted for review but both agencies' decisions remained unchanged.
- Two rare blood disorder products had discordant recommendations between the agencies, in which CDA provided a positive recommendation for reimbursement whereas INESSS did not (**Fig. 2**).
- Submissions for the majority (72%) of the rare blood products evaluated included a phase III randomized control trial (RCT) as part of the clinical evidence.
  - Four products included an indirect treatment comparison (ITC) in their submission, which all received a positive recommendation from CDA and one (pegcetacoplan) received a positive recommendation from INESSS. INESSS did not evaluate two of these products and one product (emicizumab) received a negative recommendation.
  - One product included real-world evidence in the resubmission, however, decisions remained unchanged for both agencies.
- Two single-arm trial-based submissions for hemophilia B treatment received positive CDA recommendations; CDA noted both therapies addressed patient important needs as gene therapies.
  - One product provided an ITC in their submission; the approach used for generating comparative efficacy data in the CDA submissions was a matching-adjusted indirect treatment comparison.
  - Conversely, one of these products received a negative recommendation by INESSS due to the lack of therapeutic value demonstrated.

### Rationale for recommendation and critique

- Main criticisms mentioned in the negative recommendations were related to limitations in the clinical evidence submitted and uncertainty around the clinically meaningful benefit of the product (**Fig. 3**).

## CONCLUSIONS

- Most therapies assessed for rare blood disorders received positive recommendations from CDA, however, there was moderate agreement in CDA and INESSS decisions.
- Unmet needs and rarity of the conditions were frequently noted in the rationale for positive recommendations by Canadian HTA agencies.
- In contrast with CDA and INESSS, other HTA agencies have specific or modified processes (e.g., NICE, PBAC) that have adopted flexible and pragmatic approaches towards uncertainties around evidence for DRDs.[5]
- Future studies can build on this research by assessing concordance between CDA recommendations and listing decisions of participating drug plans.

## REFERENCES

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