Analysis of Reimbursement Outcomes for Oncology Therapies in Quebec, Canada

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INTRODUCTION

- Recommendations from the *Institut national d'excellence en santé et en services sociaux* (INESSS) are crucial in ensuring patient access to new oncology drugs in Quebec.
- While overall survival (OS) remains the gold standard of oncology drug approvals, overall response rate (ORR) can be assessed earlier than OS and is useful in trials with limited patient numbers.[1]
- In some circumstances, manufactures may seek reimbursement of oncology treatments based on Phase 2 trials; ORR is the most common co-primary endpoint in single-arm Phase 2 oncology trials.[2]

OBJECTIVE

We analyzed INESSS reimbursement recommendation reports for oncology drugs to understand characteristics of positive and negative reimbursement recommendations, with a focus on submissions using Phase 2 data and with ORR as a primary or secondary endpoint.

METHODS

- Reimbursement recommendation reports published Jan 1, 2022–Aug 1, 2024, from first INESSS evaluations for general or innovative oncology medicines were retrieved from the INESSS website and were reviewed by one investigator (RC, NN, EA, or LB) to extract information on the following:
  - Drug under review (e.g., brand and generic name, indication),
  - Recommendation details (e.g., recommendation type, recommendation conditions),
  - Clinical evidence deliberated by INESSS (e.g., details of pivotal trial submitted, indirect treatment comparisons included in submission, inclusion of ORR as an endpoint), and
  - Committee commentaries (e.g., rationale for recommendation).

RESULTS

Identification of relevant recommendation reports

- Of 310 reimbursement recommendation reports published by INESSS between Jan 1, 2022–Aug 1, 2024, 67 were first INESSS evaluations for general or innovative oncology medicines (**Fig. 1**).

Characteristics of included recommendation reports

- The most common indications for which treatments were evaluated were NSCLC (9 reports), breast cancer (8), and multiple myeloma and prostate cancer (5 each).
- Of 67 reports, 46 included Phase 3 trials in the clinical evidence, and 19 Phase 2 as the pivotal evidence.
- Eighty-nine percent (41/46) of assessments based on Phase 3 trials and 63% (12/19) based on Phase 2 trials received a positive reimbursement recommendation (**Fig. 2**).

Figure 1. Overview of identification and selection process for INESSS reimbursement recommendation reports

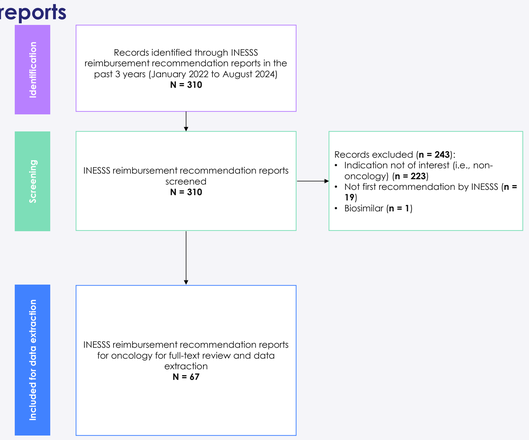
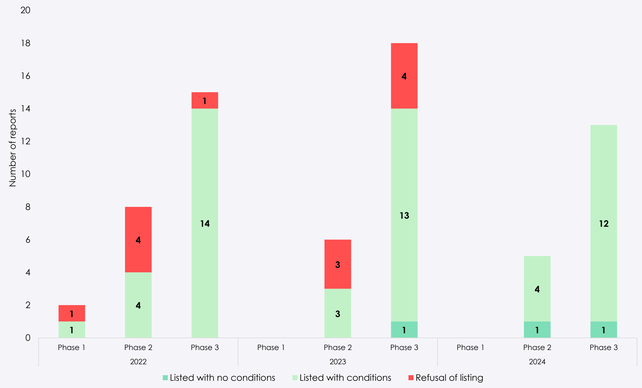


Figure 2. Recommendation type according to trial phase and publication year (N = 67 reports)



Note: For the analysis depicted in this figure, Phase 1/2 trials were classified as Phase 2 trials and Phase 2/3 as Phase 3.

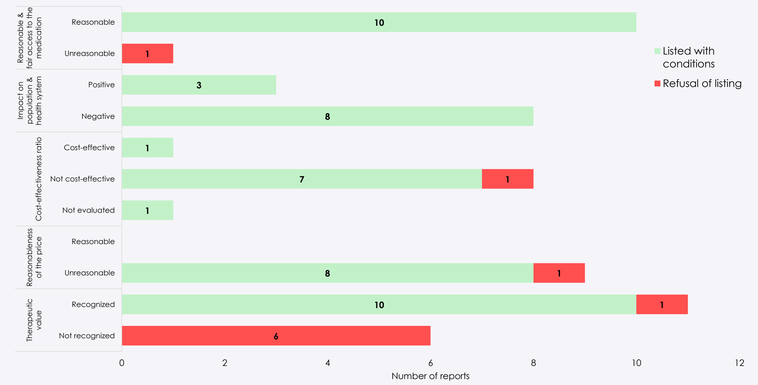
RESULTS *(cont.)*

Characteristics of assessments – submissions based on Phase 2 evidence with ORR

- There were 17 drug reviews with pivotal Phase 2 trials that included the ORR outcome as a primary or secondary endpoint; of these, 7 had negative reimbursement decisions (**Fig. 3**).
- Most (6/7) of these negative decisions were due to therapeutic value not being recognized; in one case INESSS judged that reimbursement would not be appropriate from a distributive justice standpoint.
  - The treatment assessed not to be appropriate from a distributive justice standpoint was a CAR-T therapy. INESSS specified its high cost would be excessive for a small number of patients treated.
  - All six oncology drugs whose therapeutic value was not recognized received negative recommendations from INESSS. In five of those decisions, the committee was not unanimous on the therapeutic value, and two of these five recommendations were based on pivotal Phase 1/2 data.
- Improvement of ORR in submitted evidence was reported in 94% (16/17) of submissions.
  - The sole submission where ORR was not improved had ORR as a primary endpoint and the submission ultimately received a refusal of listing.
  - Six Phase 2 trials with negative reimbursement decisions had improvement in ORR in submitted evidence, however in all six cases INESSS noted substantial uncertainty or critiqued methodological robustness in the findings.

- Fifteen submissions included ORR as a primary endpoint, of which 8 received a positive recommendation
  - 2 included ORR as a secondary endpoint, both received a positive recommendation (primary endpoints in these studies were complete response and % participants alive without disease progression at 6 months, respectively).

Figure 3. Summary of reasons for INESSS recommendation and rejection for reports with Phase 2 pivotal evidence and ORR outcome as a primary or secondary endpoint (N = 17 reports)



Note: the sum of reasons for recommendations exceeds the total number of drug assessments, because multiple reasons could be mentioned in a single recommendation. If a recommendation included a majority and a minority position, only the majority position is presented in this figure.

DISCUSSION & CONCLUSIONS

- While a substantial proportion of submissions (21/67) were based on earlier than Phase 3 trial evidence, only 62% of those submissions received a positive reimbursement recommendation.
  - Notably, none of the reports mentioned lack of feasibility of conducting a Phase 3 trial. A study of Phase 2 evidence submitted to pERC (now CDA) from July 2011–July 2019 reported that lack of feasibility of conducting a Phase 3 trial was an important factor associated with positive or conditional recommendations for reimbursement (P=0.04).[3]
- More than half of all submissions for oncology therapies based on Phase 2 evidence that included ORR received positive recommendations for reimbursement.
  - Negative decisions were most often related to therapeutic value not being recognized.
- These findings contribute to our understanding of assessments of therapeutic value and factors influencing reimbursement decisions.

REFERENCES

1. Merino *et al.* J Clin Oncol. 2023;41(15):2706-2712; 2. Grayling *et al.* J Natl Cancer Inst. 2019;111(12):1255-1262; 3. Li et al. Curr Oncol. 2020;27(5):e495-e500.

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