PATIENT VOICES IN RARE DISEASE HEALTH TECHNOLOGY ASSESSMENTS (HTA)

RECENT TRENDS IN THE USE OF QUALITATIVE RESEARCH IN REIMBURSEMENT SUBMISSIONS TO CANADA'S DRUG AGENCY (CDA-AMC)

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HTA10

BACKGROUND

- Qualitative research can inform healthcare decision making by understanding lived experiences (particularly for rare diseases), assessing treatment acceptability and value, and supplementing quantitative data.[1]
- Interest in how extensively qualitative research is used in healthcare decision making, specifically in HTA is expanding.[2-5]
- In Canada, sponsors of reimbursement submissions may submit qualitative evidence to HTAs – CDA-AMC and Institut national d'excellence en santé et services sociaux (INESSS).
- Additionally, these HTAs collect patient group input which often includes qualitative data.
- How extensively sponsors and patient groups are using qualitative evidence and what influence qualitative research has on reimbursement decisions remains unclear.

OBJECTIVE

To characterize the contemporary use of patient- or caregiver-centred qualitative research by sponsors and patient groups in Canadian reimbursement reviews of pharmaceuticals for rare diseases, with a focus on CDA-AMC.

METHODS

DATA EXTRACTION AND SYNTHESIS

- Sponsored reimbursement reviews of non-oncology pharmaceuticals for rare diseases, with recommendations issued in 2024, were retrieved from the CDA-AMC website in December 2024 and January 2025.[6]
  - Reimbursement reviews for oncology pharmaceuticals were excluded because of the inherent differences in their reimbursement submissions.
  - Rare diseases were defined as conditions affecting <1/2,000 as listed on orpha.net.[7]
- Qualitative research used or cited in the clinical review, pharmacoeconomic review, and patient group input report were extracted and synthesized.
- The following information was extracted:
  - Disease, pharmaceutical, and sponsor details.
  - *De novo* qualitative research used.
  - Additional qualitative research cited.
  - Reimbursement recommendation and date issued.
- *De novo* qualitative research was defined as qualitative methods used or qualitative data collected to support the submission.
- These studies and data were categorized using a published framework,[1] and the quality was assessed using the CASP checklist.[8]
- The corresponding reimbursement reviews conducted by INESSS in the province of Quebec were also reviewed.

GUIDELINE REVIEW

- Available information from CDA-AMC and INESSS on the use of qualitative research and patient involvement in reimbursement submissions was reviewed and summarized.

RESULTS

- Ten CDA-AMC reimbursement reviews were identified and reviewed (**Table 1**).

De novo qualitative research by sponsors

- Two sponsors submitted *de novo* patient- or caregiver-centred qualitative research (20%; **Table 1**).
- In SR0799-000, Appendix 5, CDA-AMC cite a qualitative study, submitted by the sponsor in their pharmacoeconomic evaluation, which explored disease impacts and treatment effects through qualitative interviews with caregivers and informed the selection of appropriate outcome measures for future studies.[9]
  - This study scored 9/10 on the CASP criteria.
- In SR0788-000, two qualitative studies were included:
  1. One assessed the content validity of clinical trial outcome measures through qualitative interviews with patients and caregivers.
     - This study scored 8/10 on the CASP criteria.[10]
  2. Another explored disease impacts via interviews with patients and caregivers, informing the disease background in the clinical review.
     - This study did not include sufficient detail to assess its quality (abstract only).[11]
- Based on the CDA-AMC reimbursement reviews, it appears that the sponsors used the qualitative research to: understand perspectives and provide context (n=2), inform subsequent quantitative exercises (n=2), assess treatment acceptability and subjective value (n=1), reach groups other methods cannot reach (n=1), and contribute to economic model development (n=1; **Figure 1**).
- Of the ten corresponding INESSS reimbursement reviews, none detailed qualitative research included by the sponsors (0%).

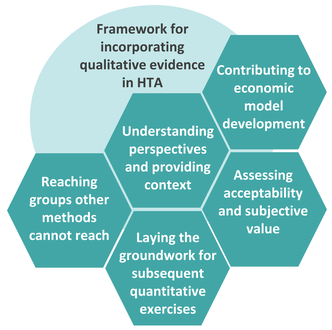
Additional qualitative research cited

- Two qualitative studies were used to understand perspectives and provide context in the disease background sections of the clinical review.[12,13]

De novo qualitative research by patient groups

- In all reviews, patient groups provided input, eight of which included qualitative data from patients/caregivers (80%; **Table 1**).
- Data presented included quotes from interviews, social media posts, and free-text responses in surveys.
- However, there was little methodological information provided to assess the quality of these data collection methods, and no indication that qualitative analysis methods were applied.
- Based on the patient input reports, it appears that the patient groups used these qualitative data to: assess treatment acceptability and subjective value (n=8); and understand perspectives and provide context (n=6; **Figure 1**).
- Only four (40%) INESSS reimbursement reviews included qualitative data submitted by patient groups.

Figure 1. Framework for incorporating qualitative evidence in HTA



Abbreviation: HTA, Health technology assessment.

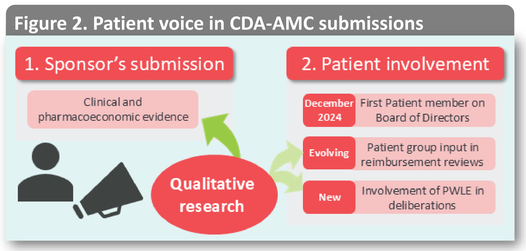
Table 1. Summary of patient-based qualitative research in 10 rare disease HTA submissions to CDA-AMC

Project number	Disease	Pharmaceutical		Sponsor	*De novo* qualitative research		Additional qualitative research cited?	CDA-AMC recommendation	Date recommendation issued
		Generic	Brand		Submitted by sponsor	Submitted by patient group			
SR0721-000	Chronic immune thrombocytopenia	avatrombopag	Doptelet	Sobi Canada, Inc.	No	Yes	No	Do not reimburse	08-May-24
SR0780-000	Alagille syndrome	maralixibat	Livmarli	Mirum Pharmaceuticals Inc.	No	Yes	No	Reimburse with clinical criteria and/or conditions	22-Apr-24
SR0799-000	Dravet syndrome	cannabidiol	Epidiolex	Jazz Pharmaceuticals Canada, Inc.	Yes[9]	No	Yes[12]	Reimburse with clinical criteria and/or conditions	18-Apr-24
SR0800-000	Lennox-Gastaut syndrome	cannabidiol	Epidiolex	Jazz Pharmaceuticals Canada, Inc.	No	No	No	Reimburse with clinical criteria and/or conditions	18-Apr-24
SR0798-000	Seizures associated with Tuberous Sclerosis Complex	cannabidiol	Epidiolex	Jazz Pharmaceuticals Canada, Inc.	No	Yes	No	Reimburse with clinical criteria and/or conditions	18-Apr-24
SR0785-000	Neuromyelitis optica spectrum disorder	ravulizumab	Ultomiris	Alexion Pharma GmbH	No	Yes	No	Reimburse with clinical criteria and/or conditions	13-Mar-24
SR0793-000	Neuromyelitis optica spectrum disorders	inebilizumab	Uplizna	Horizon Therapeutics Canada	No	Yes	Yes[13]	Reimburse with clinical criteria and/or conditions	13-Mar-24
SR0788-000	Progressive familial intrahepatic cholestasis	odevixibat	Bylvay	Medison Pharma Canada Inc.	Yes[10,11]	Yes	No	Reimburse with clinical criteria and/or conditions	13-Feb-24
SR0801-000	Hereditary transthyretin mediated amyloidosis	vutrisiran	Amvuttra	Alnylam Netherlands B.V.	No	Yes	No	Reimburse with clinical criteria and/or conditions	29-Jan-24
SR0778-000	Homozygous familial hypercholesterolemia	evinacumab	Evkeeza	Ultragenyx Pharmaceutical Inc.	No	Yes	No	Reimburse with clinical criteria and/or conditions	12-Jan-24

Available information on qualitative research and patient involvement

- **Methods guide:** In March 2025, CDA-AMC released a new methods guide which provides some guidance on the use of qualitative research – gathered through formal qualitative studies or systematic reviews – to support elements of value.[14]
- **Patient leadership:** In December 2024, CDA-AMC appointed the first-ever patient member to its board of directors.[15]
- **Patient input:** CDA-AMC recently announced their plans to evolve the patient group input processes.[16,17]
- **Deliberations:** This year, they published a new deliberative framework and launched the inclusion of a person with lived experience presentation in the deliberation committee meeting.[18,19]
- **INESSS:** INESSS appointed a dedicated patient consultation professional in 2022.
  - Their methodological guide recommends the use of qualitative data and patient input (via questionnaires and interviews) to inform evaluations.[20]

Figure 2. Patient voice in CDA-AMC submissions



Abbreviation: PWLE, Persons with lived experience.

LIMITATIONS

- We relied on the information included by CDA-AMC and INESSS in their reimbursement reviews which may not fully describe the qualitative evidence submitted by sponsors in their submissions.
- The impact of including high quality patient-based research on reimbursement decisions cannot be determined from this work and requires further investigation.

CONCLUSION

- Few sponsors submitted patient- or caregiver-centred qualitative evidence; however, CDA-AMC received qualitative data from patient groups to consider for most submissions.
- Limited inclusion of qualitative research by sponsors may be explained by the absence of clear guidance from decisionmakers on its use and benefits to HTA.
- While qualitative data submitted by patient groups provide important insight into patient priorities and values, these data could be strengthened by the inclusion of greater methodological detail.
- HTA processes in this area are evolving, and experts (such as the Health Economics Methods Advisory) could play a key role in developing future guidance for sponsors and patient groups.[21]

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