Challenges and opportunities around submitting patient experience data (PED) to HTA agencies

HTA55

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Objective

To identify challenges and opportunities around submitting and incorporating PED from patients and patient organisations in HTA processes

Introduction

- **HTA** is a systematic and multi-disciplinary process that evaluates new health technologies to determine whether they offer significant improvements or value for money compared to existing technologies.[1]
- **PED** are data from patients, their families or caregivers, or patient organisations about their experiences related to a health condition or technology.[2]
- **HTA agencies** increasingly incorporate PED from patients and patient organisations into HTA processes to ensure that decisions account for the needs of patients.[3,4]
- **Challenges:** HTA agencies often lack clarity on the specific types of evidence they prefer, and patients and patient organisations are generally not well-trained in generating PED. Additionally, it is often unclear how HTA agencies incorporate PED from patients and patient organisations into their assessments.

Methods

HTA Review

- **Purpose:** To identify publicly available case studies of PED submitted by patients and patient organisations to HTA agencies (published in English from 2014–2023)
- **HTA agencies:** Canada's Drug Agency (CDA), Institute for Clinical and Economic Review (ICER), National Institute for Health and Care Excellence (NICE), Pharmaceutical Benefits Advisory Committee (PBAC), Scottish Medicines Consortium (SMC)

Targeted Literature Review (TLR)

- **Purpose:** To identify additional case studies (published in English from 2021–2023)
- **Searches:** MEDLINE, Embase, Google Scholar, and reference lists of relevant articles

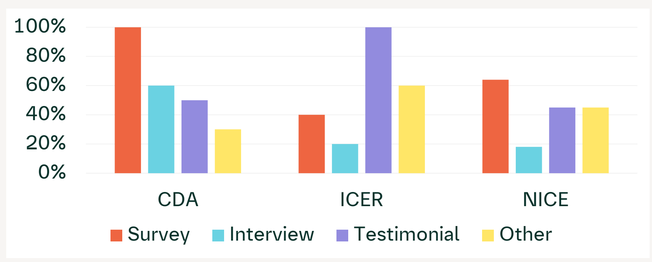
Workshop

- **Purpose:** To discuss the findings from the HTA review and TLR (conducted in January 2024)
- **Attendees:** Patients, patient advocacy group (PAG) representatives, and patient engagement experts from the US, UK, and Canada

Results: HTA Review and TLR

- In the HTA review, 45 HTA submissions across 17 disease areas were identified (atopic dermatitis, n=10; relapsed/refractory multiple sclerosis (MS), n=9; rheumatoid arthritis, n=6; idiopathic pulmonary fibrosis, n=6; other disease areas, n=14). The HTA agencies were CDA (n=10), ICER (n=11), NICE (n=11), PBAC (n=6), and SMC (n=7).
- Surveys were the most used method of data collection **(Figure 1; Figure 2)** and were included in all submissions from CDA. Patient testimonials were included in all submissions from ICER **(Figure 1).** Several case studies highlighted how PED from patient organisations can enhance HTA processes by focusing on patient-relevant outcomes and identifying unmet needs **(Table 1)**.
- In the TLR, 129 abstracts were screened (MEDLINE and Embase, n=105; Google Scholar, n=17; journal-specific searches, n=7). Eleven full-text articles were reviewed.

Figure 1. Types of PED submitted to the three main HTA agencies.
N=26. Subset analysed. 'Other' included all other methods of data collection (i.e., literature review, external research, guidelines, expert input, social media, helpline, and web seminar).



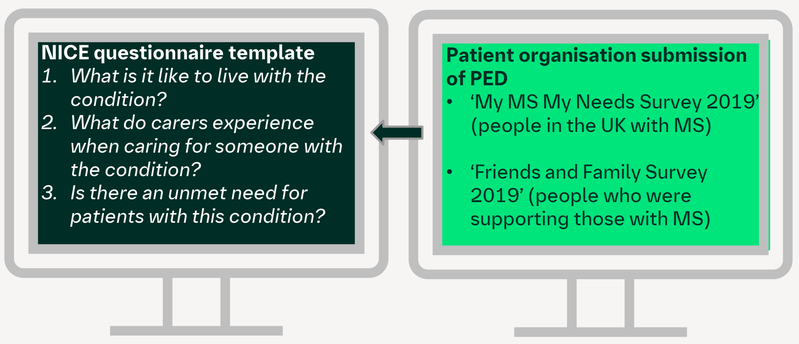


Figure 2. Example of survey-based PED submitted to an HTA agency.
NICE invited views from patient organisations on ofatumumab for treating relapsing MS. The MS Society submitted survey data.

Table 1. Case studies from the HTA review highlighting the value of PED in HTAs.

HTA agency	Disease area	PAG(s)	Type of PED	Description of PED
ICER	Duchenne Muscular Dystrophy	Parent Project Muscular Dystrophy	Registry data from Treat-NMD	The costs of supportive care used by ICER underestimated the economic burden experienced by families.
NICE	Atopic dermatitis	National Eczema Society	Calls to nurse-supported helpline	Patients experience daily suffering. The physical, psychological, and social impacts of the condition on quality of life were not fully considered.
SMC	Multiple sclerosis	MS Society Scotland MS trust	Qualitative testimonials from patient groups	Different treatments are available, and different factors affect patients' preferences. Patients value having a choice between a range of treatments with different routes of administration.
CDA	Solid tumours with NTRK gene fusion	8 PAGs*	Patient interviews	Conventional therapies are associated with low efficacy and poor safety. Patients want a treatment that targets the underlying NTRK mutation, improves quality of life, and prolongs life.

*PAGs included the Canadian Breast Cancer Network, Canadian Cancer Survivor Network, Advocacy for Canadian Childhood Oncology Research Network, Colorectal Cancer Resource & Action Network, GIST Sarcoma Life Raft Group Canada, Colorectal Cancer Canada, Lung Cancer Canada, and Sarcoma Cancer Foundation of Canada.

Results: Workshop

In the workshop, the challenges, opportunities, and best practices around submitting and incorporating PED from patients and patient organisations in HTA processes were discussed **(Figure 3)**.

Challenges

- Limited current scientific evidence of the use of PED
- Lack of transparency on how PED impact HTA decision-making
- Lack of guidance and standards
- Rigid templates with inconsistent formats
- Short timeframes to generate robust PED

Opportunities and Best Practices

- Focus on patient-relevant outcomes and unmet needs
- Clear narratives and poignant testimonials
- Clear answers to HTA agency's questions to compliment other evidence
- Collaboration between patient organisations and joint submissions
- Education and training on HTA requirements

Figure 3. The challenges, opportunities, and best practices around submitting and incorporating PED from patients and patient organisations in HTA processes.

Call to Action and Conclusions

- Although PED from patients and patient organisations improve the quality of HTA decision-making, current HTA practices limit the ability of patients and patient organisations to submit high-quality PED.
- To integrate PED from patients and patient organisations in HTA processes, we call on all relevant stakeholders to work together:
  - ✓ **HTA agencies:** increase transparency around decision-making
  - ✓ **Researchers and patient organisations:** collaborate to generate PED and publish PED openly to avoid duplication
  - ✓ **Industry:** support patient organisations in generating PED and building the capacity of relevant stakeholders
  - ✓ **All stakeholders:** work together to develop resources and share learnings
- Developing best practices for generating and submitting PED from patients and patient organisations in HTA processes alongside systematic and meaningful involvement of patients in the HTA process will ensure that the patient perspective is actively considered when evaluating new health technologies.

Abbreviations
CDA, Canada's Drug Agency; HTA, health technology assessment; ICER, Institute for Clinical and Economic Review; MS, multiple sclerosis; NICE, National Institute for Health and Care Excellence; NMD, neuromuscular diseases; NTRK, Neurotrophic tyrosine receptor kinase; PAG, patient advocacy group; PBAC, Pharmaceutical Benefits Advisory Committee; PED, patient experience data; SMC, Scottish Medicines Consortium; TLR, targeted literature review.

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