The Frequency Of Mentions And Impact Of Patient Input In HTA Decision Making In Cell And Gene Therapies Across The UK, Canada, & Australia

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Presentation

The aim of this research was to understand the influence of patient input in HTA decision making

Patient input is considered in health technology assessments (HTAs) by:

Country	HTA Body	
England	National Institute for Health and Care Excellence **(NICE)**	NICE
Canada	Canada's Drug Agency **(CDA)**	CDA-AMC
Australia	Medical Services Advisory Committee **(MSAC)**	Medical Services Advisory Committee
	Pharmaceutical Benefits Advisory Committee **(PBAC)**	

Research Objective

This research explored the frequency of mentions and impact of patient input in HTA reports of cell and gene therapies (CGTs)

Two types of patient input were considered:

- ✓ **Patient-reported outcomes (PRO)**
- ✓ **Supplementary patient inputs (SPI)**

Abbreviations: CDA=Canada's Drug Agency; CGT=Cell and gene therapies; HTA=Health Technology Assessments; MSAC=Medical Services Advisory Committee; NICE=National Institute for Health and Care Excellence; PBAC=Pharmaceutical Benefits Advisory Committee; SPI=Supplementary patient inputs; PRO=Patient-reported outcomes.

Analysis included 20 HTA appraisals of CGTs spanning seven indications

1

HTA report identification

HTA reports of CGTs assessed by NICE, CDA and PBAC or MSAC since 2020 were identified

zolgensma® (onasemnogene abeparvovec-xioi)	• Spinal Muscular Atrophy[1-5] • Pre-symptomatic Spinal Muscular Atrophy[2,6-8]
YESCARTA® (axicabtagene ciloleucel)	• Large B-cell Lymphoma[9-12] • Follicular Lymphoma[13-15]
TECARTUS® (brexucabtagene autoleucel)	• Mantle Cell Lymphoma[16-18] • B-cell Acute Lymphoblastic Leukaemia[19-22]
HEMGENIX® etranacogene dezaparvovec-drlb	• Haemophilia B[23-25]

2

Frequency Analysis

A **frequency analysis** of **predefined patient input terms** was conducted to quantify their **mentions** in each **HTA report**

A **negative binomial generalized linear model** was applied to **compare mention frequencies across HTA bodies**

3

Contextual Analysis

A **large language learning model** (LLM*) was employed to extract relevant quotes and assess their relevance to each decision.

The generated outputs were validated through human review

Outcomes characterised the influence of PRO & SPI on decision making as follows:

- **Direct Impact**
- **Indirect Impact**
- **No Impact**

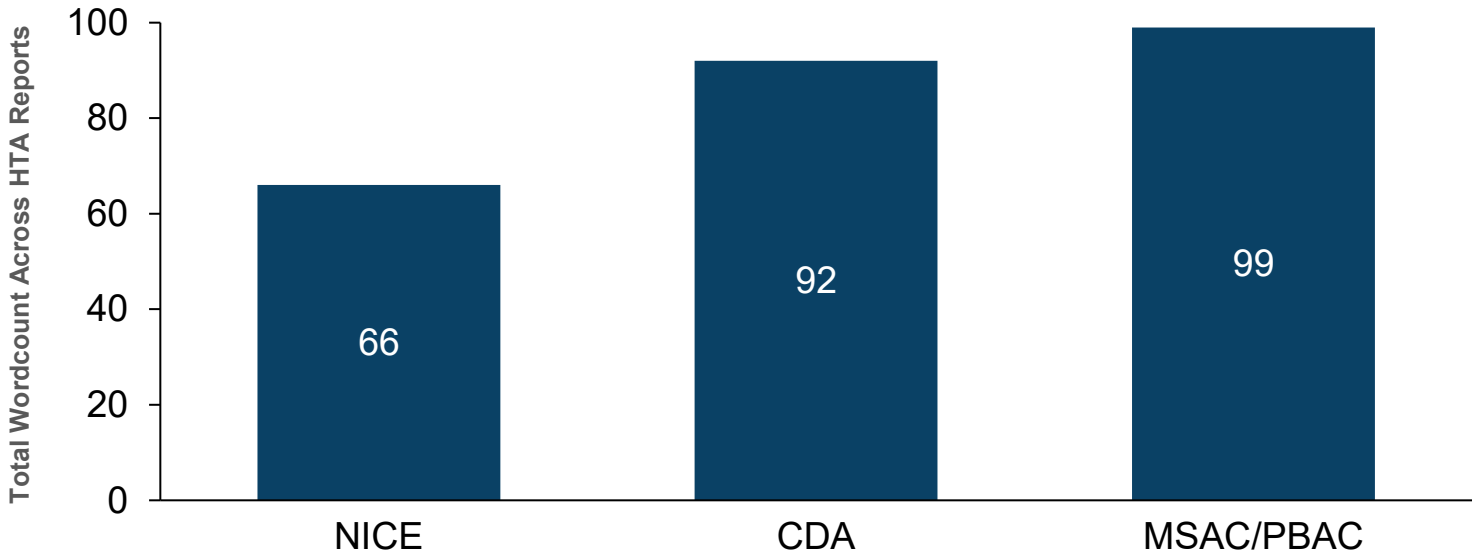
** OpenAI o4-mini. Abbreviations: CDA=Canada's Drug Agency; CGT=Cell and gene therapies; HTA=Health Technology Assessments; LLM= Language Learning Model; MSAC=Medical Services Advisory Committee; NICE=National Institute for Health and Care Excellence; PBAC=Pharmaceutical Benefits Advisory Committee; QALY= Quality-adjusted life year; SPI=Supplementary patient inputs; PRO=Patient-reported outcomes.*

No differences were observed in the frequency of patient-related terms across HTA bodies

Mentions of Patient Input in HTA Reports

Example words and phrases included in the frequency analysis:

- *"Patient input"*
- *"Public consultation"*
- *"Patient-reported outcomes"*
- *"Quality of life"*



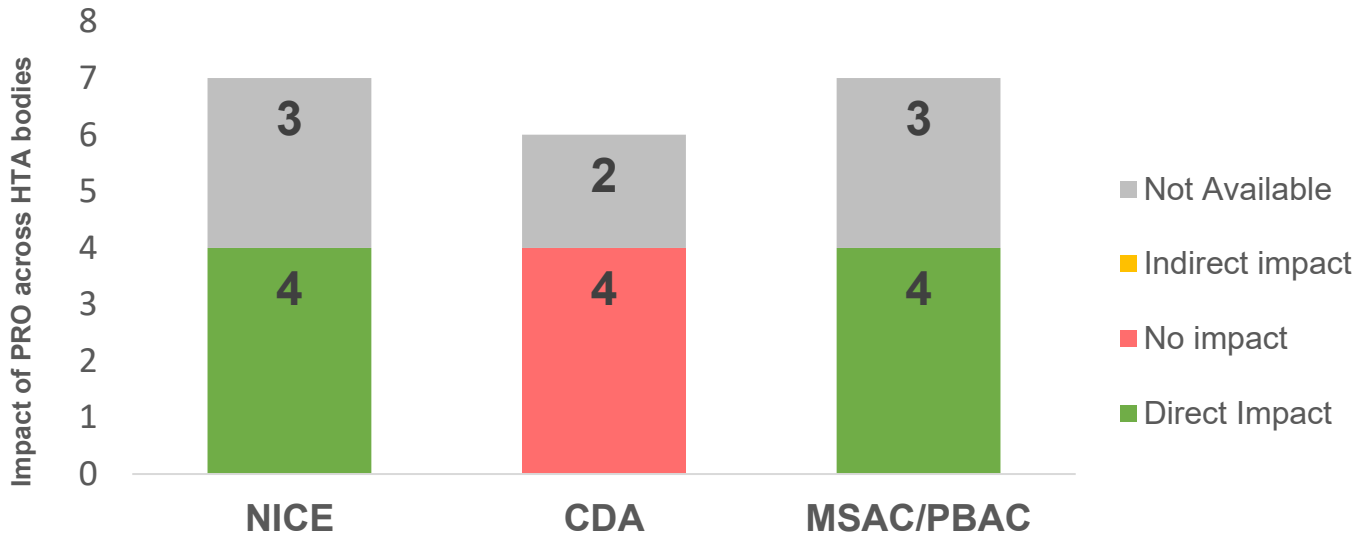
The variation in the total wordcount of predefined relevant terms, included in HTA reports across HTA bodies, was not statistically significant (NS) (P>0.05)

Abbreviations: CDA=Canada's Drug Agency; HTA=Health Technology Assessments; MSAC=Medical Services Advisory Committee; NICE=National Institute for Health and Care Excellence; NS=Not statistically significant; PBAC=Pharmaceutical Benefits Advisory Committee.

PROs directly influenced NICE and MSAC/PBAC, but had no impact on CDA decisions due to data uncertainties

Impact of Patient Reported Outcomes

- **PRO data** were **unavailable** in **8 of 20 HTA** appraisals
- Among the remaining **12 appraisals, PROs directly influenced 8 HTA outcomes from NICE and MSAC/PBAC**
- In **4 CDA appraisals, PROs** had **no direct impact** due to **uncertainties** in **data robustness**



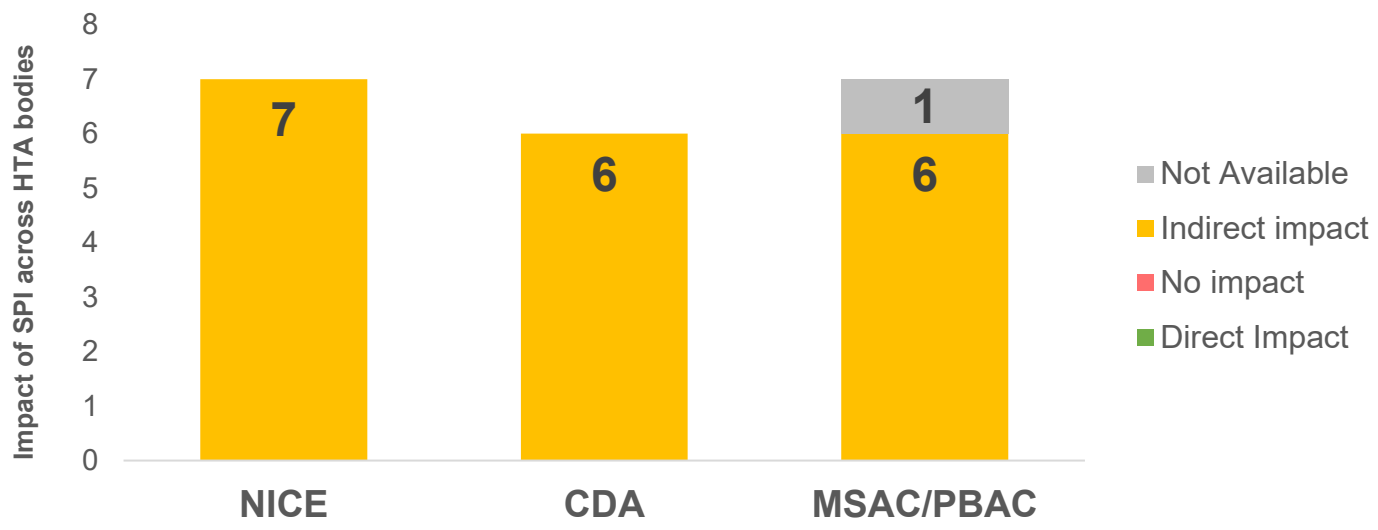
Where PROs were available and robust, they directly influenced the HTA outcome through their incorporation in economic analyses

Abbreviations: CDA=Canada's Drug Agency; HTA=Health Technology Assessments; MSAC=Medical Services Advisory Committee; NICE=National Institute for Health and Care Excellence; PBAC=Pharmaceutical Benefits Advisory Committee; PRO=Patient-reported outcomes.

Although considered in the majority of reports, SPIs had no direct influence on HTA outcomes

Impact of Supplementary Patient Inputs

- **SPI was available in 19 of 20 HTA appraisals** and had an **indirect influence** on the **HTA** outcome
- **Only one appraisal did not reference SPI**



**Tecartus MSAC/PBAC appraisal in Mantle Cell Lymphoma. Abbreviations: CDA=Canada's Drug Agency; HTA=Health Technology Assessments; MSAC=Medical Services Advisory Committee; NICE=National Institute for Health and Care Excellence; PBAC=Pharmaceutical Benefits Advisory Committee; PRO=Patient-reported outcomes.*

Findings reinforce the value of PROs and highlight opportunities to strengthen SPI influence in shaping more patient-centered HTA

Key Conclusions

1

While **variation in patient input mentions** across HTA bodies **was not statistically significant, distinct patterns were observed**

2

Robust PROs directly **influenced MSAC/PBAC** and **NICE** assessments

In **CDA appraisals**, **uncertainties in PRO robustness reduced their impact**, highlighting **differing interpretations** across **HTA bodies**

3

SPIs, though only indirectly impactful, were **consistently considered in CGT assessments,** reflecting their **growing role in decision-making**

Abbreviations: CDA=Canada's Drug Agency; HTA=Health Technology Assessments; CGT= Cell and Gene Therapy; MSAC=Medical Services Advisory Committee; NICE=National Institute for Health and Care Excellence; PBAC=Pharmaceutical Benefits Advisory Committee; PRO=Patient-reported outcomes; SPI= Supplementary Patient Input.

Sources

1. Zolgensma® NICE guidance HST15 (Apr 2023);
2. Zolgensma® CADTH Recommendation (Mar 2021);
3. Zolgensma® PBAC Public Summary Document (Sep 2021);
4. Zolgensma® PBAC Public Summary Document (May 2021);
5. Zolgensma® PBAC Public Summary Document (Nov 2020);
6. Zolgensma® NICE guidance HST24 (Apr 2023);
7. Zolgensma® PBAC Public Summary Document (Jul 2023);
8. Zolgensma® PBAC Public Summary Document (Nov 2022);
9. Yescarta® NICE guidance TA895 (Jun 2023);
10. Yescarta ® CADTH Recommendation (Feb 2023);
11. Yescarta ® MSAC Public Summary Document (Apr 2024);
12. Yescarta ® MSAC Public Summary Document (Mar 2023);
13. Yescarta® NICE guidance TA894 (Jun 2023);
14. Yescarta ® CADTH Recommendation (Nov 2023);
15. Yescarta ® MSAC Public Summary Document (Jan 2020);
16. Tecartus® NICE guidance TA677 (Feb 2021);
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23. Hemgenix® NICE guidance TA989 (Jul 2024);
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