Text Analysis of Patient Engagement in HTA

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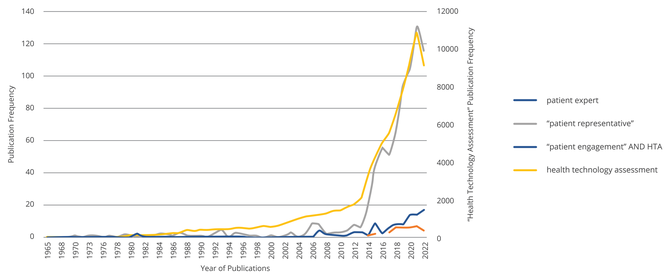
Introduction

Patient engagement with HTA bodies remains of interest to many patients, despite strides and greater inclusion. Since the 1990s, there has been increasing interest in patients, as reflected in discussions in the literature with terms like "patient engagement" and HTA. Figure 1.

Figure 1 The publication frequency of search terms used for literature review (measured by publication in PubMed)



Objective

Patient engagement with HTA bodies remains of interest to many patients, despite strides and greater inclusion. Engagement with regulatory agencies can provide a benchmark for comparison. Here, we use agency documents on patient engagement to assess their approach to patient engagement, seeking to better understand how they present the process for patients and other interested stakeholders.

Methods

As we use text analysis, we identified 10 organizations that had published descriptions of their patient engagement approach in English. We included four public HTA agencies, two private HTA agencies, two regulatory bodies, and two patient-focused organizations.

Table 1: The text data of the organizations included in the analysis.

Organizations	Location	Type	# Of Documents	# Of Words
CADTH	Canada	HTA	5	7038
EMA	Europe	Regulator	3	6324
FDA	USA	Regulator	5	20824
NICE	UK	HTA	2	5723
CDE	Taiwan	HTA	1	1810
PBAC	Australia	HTA	2	555
ICER	USA	Non-profit HTA	4	3389
IVI	USA	Non-profit HTA	2	4213
EUPATI	Europe	Other	6	37210
PCORI	USA	Other	4	1900

Patient Engagement Process

To provide a consistent frame for the analysis, we divided the patient engagement process into four stages: topic selection, scoping, information gathering, and report drafting, including direct participation and opportunities for outside comment.

- **Topic Selection:** Patient, patient expert, or patient groups submitting a topic request.
- **Developing the Report Framework/Scoping:** This can include "formulating questions, identifying and selecting interventions, outcomes, and questions of high priority for patients, and identifying new patient partners."
- **Information Gathering:** Gathering patient input for the report.
- **Comment on Drafts of the Report:** Patients, patient experts, and patient groups can comment on the study report before finalized.

Results

Individual Patient Involvement

- The literature reviewed identifies and describes opportunities for any individual patient, whether acting as a patient representative or not, to share their experiences with their disease and views on design and outcomes from the clinical trials either orally or by responding to the written submissions.
- From reviewing the organizations' text, it follows the literature that individual patients were found to be mainly involved with providing their disease experience during the information-gathering stage (Table 2).

Table 2: Stages where can Individual Patient (P), Patient Expert (PE), or Patient Group (PG) could be involved in the engagement process.

Organizations	Where can Individual Patient (P), Patient Expert (PE), or Patient Group (PG) get involved?			
	Topic Setting	Scoping	Information Gathering	Comment on the Draft Reports
CADTH			P, PG	PG
EMA			P	
FDA	P, PE, PG		P	
NICE	PG	PG	P, PG	P, PG
CDE			P	
PBAC			P	
ICER	PG	PG	P, PG	P, PG, PE
IVI		P, PG	P, PG	P
EUPATI*	N/A			
PCORI	P	P	P	

Patient Representative and Expert

- In contrast, patient representatives, who may be selected in consultation with a patient organization, can engage where all patients would be too large a group to be unmanageable. Some documents have also intimated a preference for patient experts as patient representatives, as they combine both knowledge of the disease at issue and the HTA process. Reviewing each example of "patient representative" and "patient expert," both are used to describe an individual with direct contact with an organization when sharing perspectives of a disease. However, patient representative still appears to be more commonly discussed. See Table 3.

Table 3 Word Frequency of patient expert and representative in the organizations' text.

Organizations		CADTH	EMA	FDA	NICE	CDE	PBAC	ICER	IVI	EUPATI	PCORI
Word Frequency	Patient Expert			1				1			
	Patient Representative	1	2	9		8		2	2	50	2

- An example of patient representative engagement from outside HTA is the FDA's Patient Representative Program, where select patients with experience and extensive knowledge of a disease and its treatments provide direct input to the FDA.
- To help patients fill these roles as both a patient representative and patient expert, organizations such as EUPATI offer a training program to help patients become recognized as certified patient experts familiar with the medicine development process, including regulatory and HTA processes. Similarly, PCORI also focuses on empowering patients and other stakeholders with actionable information about their health and healthcare choices. It provides on-demand training packages for people new to research and patient-centered research.

Methodology used for Patient Based Evidence

- Using the methods to gather patient-based evidence from de Wit as search terms, surveys, interviews, and focus groups are popular among HTA bodies to collect patient-based evidence (Table 4).

Table 4 Word frequency of the methods to gather patient-based evidence suggested by de Wit et al. (2020).

Organizations	Type	Methods to Gather Patient Evidence			
		Word Frequency			
		Survey	Focus Group	Interview	Mixed Methods*
CADTH	HTA	8	1	7	
EMA	Regulator	13	2	1	
FDA	Regulator	101	53	147	1
NICE	HTA	13	7	2	
CDE	HTA	2	1	3	
PBAC	HTA				
ICER	Private HTA	10			
IVI	Private HTA	12	1	2	
EUPATI	Other	16		6	
PCORI	Other			1	

Mixed Methods include both Qualitative and Quantitative methods

Results (Continued)

Patient Evidence

- HTA/Reg vary in how much guidance they offer patients on what evidence they seek.
- We found the word frequency of terms from the Health Equality Europe list of what patient evidence HTA might gather. HTAs are interested in cost but seek more information on disease and treatment experience from patients (Table 5). Quality of life, disease burden, and social life were less often mentioned explicitly, outside the expressed interest in the disease per se.

Table 5 Word frequency of the search terms from Health Equality Europe that highlight the patient evidence that HTA might gather.

Org	Word Frequency											
	Social Life	Disease	Drugs	Treatment	Technologies	Price	Cost	Daily	Mental	Caregiver	Quality of Life	Disease Burden or of illness
CADTH		30	139	54	12	1	14	1		28	5	1
EMA		11	5	2	1						1	1
FDA	2	191	150	140	2	1	24	30	1	61	2	4
NICE		1	1	6	7		3		1			
CDE		10	10	8	2		1	3		9	1	
PBAC				1			2					
ICER		17	19	42		5	4		2	4	1	3
IVI		8	2	13	1		7	1	1	7		
EUPATI		128	14	44	32		16	2	8	22		
PCORI		3	5									

Discussion

Overall, while patient engagement has increased, there is ongoing interest in discussing how effective current efforts have been in reflecting the patient voice. Our text analysis found that individual patients are mainly involved when organizations gather information primarily from surveys, interviews, and focus groups—providing information on cost, disease, and treatment experience. We discovered that patient representatives and experts are becoming more sought after, particularly in expressing the collective views of the appropriate patient organization, reviewing and commenting on the report, or could be involved in advice or decision-making. Although patient involvement has increased, from our analysis, organizations display varying levels of patient involvement. Even though organizations describe the importance of patient involvement, they are not always part of the decision-making committee and there is little transparency on how patient involvement impacts patient organizations.

References

1. Commissioner, O. of the. (n.d.). FDA patient representative program. U.S. Food and Drug Administration. Retrieved April 7, 2023, from https://www.fda.gov/patients/learn-about-fda-patient-engagement/about-fda-patient-representative-program https://learning.eupati.eu/mod/page/view.php?id=438&forceview=1
2. About PCORI. PCORI. (2023, March 31). Retrieved April 7, 2023, from https://www.pcori.org/about/about-pcori
3. Research fundamentals: Preparing you to successfully contribute to research. PCORI. (2022, December 8). Retrieved April 7, 2023, from https://www.pcori.org/engagement/research-fundamentals
4. de Wit, M., Guillemin, F., Grimm, S., Boonen, A., Fautrel, B., & Joore, M. (2020). Patient engagement in health technology assessment (HTA) and the regulatory process: what about rheumatology?. RMD open, 6(3), e001286. https://doi.org/10.1136/rmdopen-2020-001286
5. Health Equality Europe. (2008). (rep.). Understanding Health Technology Assessment (HTA) (pp. 18–20).
6. Scott, A. M., Wale, J. L., & HTAi Patient and Citizen Involvement in HTA Interest Group, Patient Involvement and Education Working Group (2017). Patient advocate perspectives on involvement in HTA: an international snapshot. Research involvement and engagement, 3, 2. https://doi.org/10.1186/s40900-016-0052-
7. WP7 activity 1 report - eunethta. (n.d.). Retrieved April 7, 2023, from https://www.eunethta.eu/wp-content/uploads/2018/01/WP7-Activity-1-Report.pdf