Patient Involvement in Regulatory and HTA Processes: A Call for Enhanced Alignment

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Background & Objectives

- Including patients in decision-making is considered as an important part of Patient-Focused Drug Development by Regulators, Health Technology Assessment (HTA) bodies and Pharmaceutical Industry.
- This study aims to compare the level and process of Patient Involvement in regulatory and HTA assessments, to analyse convergence and divergence of approaches.

Methods

- A literature search was performed using the search terms for Patient Involvement, HTA and Regulators.
- Multiple databases including the EMBASE, Health Technology Assessment Database and MEDLINE were searched in the OVID® platform from 2015 and 2021.
- All the records retrieved in English from the literature search were screened by one reviewer and decisions were validated by a second reviewer to include relevant papers.
- Relevant data on how patients are involved by regulatory & HTA agencies in assessment processes and to what extent their inputs are considered in decision making from all included studies were extracted by a single reviewer by using a pre-defined extraction grid, which was subsequently validated by an independent reviewer.
- Additional data sources include regulatory and HTA websites.

Results

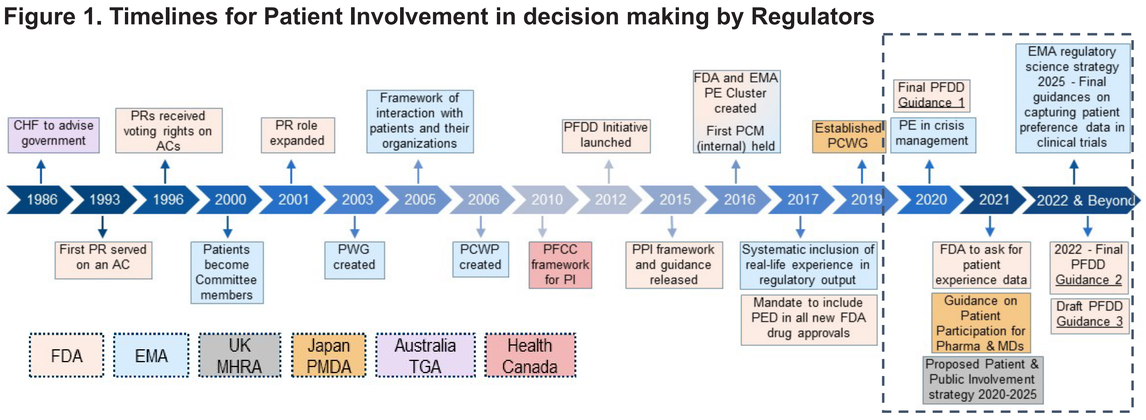
- We retrieved a total of 146 publications from the search performed, only 75 publications contained relevant data including 26 regulatory articles and 49 papers mentioning Patient Involvement in HTA process.

Patient Involvement with Regulators in Decision Making:

- Out of 26 publications on Regulatory Patient Involvement process, US (n=8); CA (n=6); EU (n=6); UK (n=2); JP (n=3); AU (n=1) and Global (n=1) were identified. Figure 1 outlines timelines for Patient Involvement for six Regulators across geographies in decision making.
- Both Australia (TGA) & US (FDA) are most progressive in engaging with patients. In US (FDA), Europe (EMA), Australia (TGA) & Canada (Health Canada), patients are committee member whereas Patient Involvement is optional in regulatory processes in other countries e.g. in Brazil (ANVISA).
- There is accelerating activity among Regulators in the last 2 years that illustrates Patient Involvement is increasingly adopted as a practice in decision making. Frameworks are developed by almost all Regulators (FDA, EMA, Health Canada, TGA, MHRA, & PMDA) to make Patient Involvement more structured, consistent and efficient.
- Japan's PMDA has published guidelines on Patient Involvement in medicines development and regulations which provide structure to patient input.

A Continuum of Patient Involvement with Regulators in Decision Making

Figure 1. Timelines for Patient Involvement in decision making by Regulators



AC, Advisory Committee; CHF, Consumer Health Forum; EMA, European Medicines Agency; FDA, Food Drugs & Administration; MDs, Medical Devices; MHRA, Medicines & Healthcare Products Regulatory Agency; PCM, Patient Council Meeting; PCWG, Patient Centricity Working Group; PCWP, Patient & Consumer Working Party; PE, Patient Engagement; PEAC, Patient Engagement Advisory Committee; PED, Patient Experience Data; PFCC, Patient- and family-centred care; PFDD, Patient-focused drug development; PI, Patient Involvement; PMDA, Pharmaceuticals and Medical Devices Agency; PPI, Patient Preference Information; PRs, Patient Representatives; PWG, Patient Working Group; TGA, Therapeutic Goods Administration

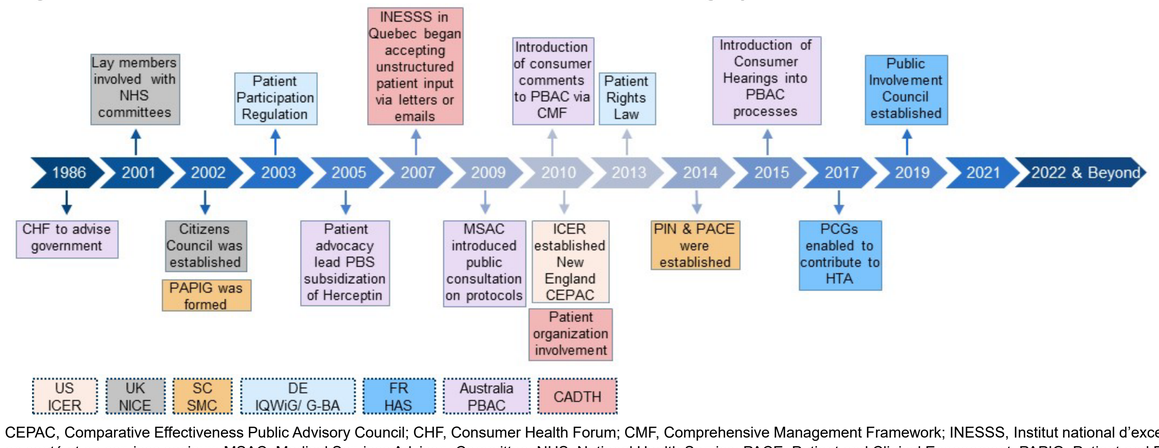
Source: FDA Patient Engagement; Partners & networks - Patients and consumers; Clinigma; Gov.UK; PMDA; TGA; Health Canada

Patient Involvement with HTAs in Decision Making

- HTA bodies in countries like Australia & UK are progressing fastest with Patient Involvement through committee representation already almost two decades back whereas France (HAS) recently began with PCGs involvement in the last few years (**Figure 2**).
- Significant variability was identified in the level of Patient Involvement in HTA decision making as shown in **Table 1**, ranging from formal processes, e.g. involvement in submissions/consultations and representation in committees (UK, Canada, Germany, Australia, France, Scotland, Japan) to limited Patient Involvement (Taiwan, New Zealand, Korea). Voting rights were given to patients in CADTH of Canada.
- Patient support to simplify their inputs using guides, templates etc. are provided by almost all HTA bodies (UK, Canada, Germany, Australia, France, Scotland, Japan). In training patients to facilitate Patient Involvement UK and Canada are leading.
- Sharing back with patients either in the form of feedback on patients' input or how their input has been used is an important step that is taken up by few HTAs (US, France).

A Continuum of Patient Involvement with HTAs in Decision Making

Figure 2. Timelines for Patient Involvement in decision making by HTAs



CEPAC, Comparative Effectiveness Public Advisory Council; CHF, Consumer Health Forum; CMF, Comprehensive Management Framework; INESSS, Institut national d'excellence en santé et en services sociaux; MSAC, Medical Services Advisory Committee; NHS, National Health Service; PACE, Patient and Clinical Engagement; PAPIG, Patient and Public Involvement Group; PBS, Pharmaceutical Benefits Scheme; PCG, Patient and Consumer Groups; PIN, Public Involvement Network

Source: ICER; NICE; SMC; IQWiG/ G-BA; HAS; PBAC; CADTH

Level of Patient Involvement varies within HTAs

Table 1. Comparison of Patient Involvement by international HTAs

Patient Involvement (PI) Activities/ Opportunities*	HTAs							
	ICER	CADTH	NICE	SMC	IQWiG & GBA	HAS	Chuikyo	PBAC
1. Ways of patient participation								
Patient submissions (verbal/ written) by individuals, comments from experts & associations / by patient groups and network consortiums	✓	✓	✓	✓	✓	✓	✓	✓
Patient participation by representation on working groups and committees	✓	✓	✓	✓	✓	✓	✓	✓
Patient participation by voting rights in decision making		✓						
2. Capability Support / Contribution Support for PI								
Patient support to simplify their input (process, guides, templates & questionnaires)	✓	✓	✓	✓	✓	✓	✓	✓
Capability building/ Training to facilitate PI		✓	✓	✓				
Plain-language materials from manufactures for PI			✓	✓				
3. Sharing back with Patients								
Feedback on patients' input received for the respective application evaluation	✓	✓				✓		
Report back on how patient input will be or has been used	✓					✓		

* PI activity: ✓ exists; either the activity does not exist or no information available

ICER, Institute for Clinical and Economic Review; CADTH, Canadian Agency for Drugs and Technologies in Health; NICE, National Institute for Health and Care Excellence; SMC, Scottish Medicines Consortium; IQWiG, Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen (Institute for Quality and Efficiency in Health Care); GBA, Gemeinsamer Bundesausschuss (Federal Joint Committee); HAS, The Haute Autorité de santé (French National Authority for Health); PBAC, Pharmaceutical Benefits Advisory Committee

Convergence & Divergence in Patient Involvement by Regulators & HTAs within countries

- Level of involvement between regulatory and HTA body, varied within countries e.g. Canada has established Patient Involvement in policy-based regulatory decision making and in the HTA agency (CADTH), patients have voting rights (see **Table 2**).
- The Japanese Pharmaceutical Agency (PMDA) has established a process of Patient Involvement, in contrast in HTA activities the patient community is not formally included. Similar efforts were seen with capability support to patients, PMDA is more active than the HTA body.
- In 2021, MHRA in UK has initiated a pilot for Patient Involvement in new applications asking the manufacturer to submit data from Patient Involvement activities whereas patients are key stakeholders in UK NICE decision making since early 2000.
- Sharing back with patients is not very common among Regulators.

Level of Patient Involvement varies within countries between Regulators & HTAs

Table 2. Comparison of Patient Involvement by international Regulators & HTAs within countries

Patient Involvement (PI) Activities/ Opportunities*	FDA	ICER	Health Canada	CADTH	EMA	IQWiG & GBA	HAS	SMC	MHRA	NICE	PMDA	Chuikyo	TGA	PBAC
1. Ways of patient participation														
Patient submissions (verbal/ written) by individuals, comments from experts & associations / by patient groups and network consortiums	✓	✓	✓	✓	✓	✓	✓	✓		✓	✓		✓	✓
Patient participation by representation on working groups and committees	✓	✓	✓	✓	✓	✓	✓	✓		✓	✓			✓
Patient participation by voting rights in decision making	✓			✓	✓									
2. Capability Support / Contribution Support for PE														
Patient support to simplify their input (process, guides, templates & questionnaires)	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓		✓	✓
Capability building/ Training to facilitate PE	✓			✓	✓			✓	✓	✓	✓			
Plain-language materials from manufactures for PE			✓					✓		✓				
3. Sharing back with Patients														
Feedback on patients' input received for the respective application evaluation		✓		✓			✓				✓			
Report back on how patient input will be or has been used		✓					✓							

* PI activity: ✓ exists; either the activity does not exist or no information available

FDA, Food Drugs & Administration; ICER, Institute for Clinical and Economic Review; CADTH, Canadian Agency for Drugs and Technologies in Health; EMA, European Medicines Agency; IQWiG, Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen (Institute for Quality and Efficiency in Health Care); GBA, Gemeinsamer Bundesausschuss (Federal Joint Committee); HAS, The Haute Autorité de santé (French National Authority for Health); SMC, Scottish Medicines Consortium; MHRA, Medicines & Healthcare Products Regulatory Agency; NICE, National Institute for Health and Care Excellence; PMDA, Pharmaceuticals and Medical Devices Agency; TGA, Therapeutic Goods Administration; PBAC, Pharmaceutical Benefits Advisory Committee

Regulators **HTAs**

Table 1 & Table 2 show the level of information available at face value and does not necessarily inform of the breadth and depth of the engagement and inclusion of patients' input in the decision making.

Conclusions

Though Regulators & HTA bodies put strong efforts to embed patient feedback and input into patient informed decision making, more alignment between regulatory and HTA bodies may be desirable to further strengthen the impact. Ideally, Patient Involvement efforts are designed to be responsive to both, the needs of regulatory and HTA bodies, to minimize efforts for the patient community and involved bodies and to further drive consistency.

References

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Disclosures

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