Patient Centricity in HTA: Fact or Fable?

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Introduction

The importance of patient involvement in HTA is now widely acknowledged given that patients can provide a unique perspective on first-hand experience of the disease and available treatments, but also to ensure fairness and transparency of the outcome.[1,2,3]

Many HTA bodies have developed their own processes for incorporating the patient perspective with varying definitions, which may include the individual patient, carers, patient advocates, patient organisation representatives and patient experts.[2]

The aim of this research was to understand how HTA bodies incorporate patient input to inform their decision-making and whether certain approaches are more impactful.

Methodology

The scope of this research focused on 12 countries (9 x European, Australia, Brazil and Canada) and utilised information retrieved from a targeted search of peer-reviewed publications, grey literature, and publicly available information from national and regional HTA bodies.

Information was reviewed to:

- Identify and map the formal pathways for patient input within the HTA appraisal and decision-making process
- Evaluate eligibility criteria, submission process and format and level of interaction
- Determine the potential for the identified patient input to impact HTA outcomes

Results

Part 1: Identified opportunities for patient involvement in HTA

Table 1: Patient involvement across the key national and regional HTA agencies

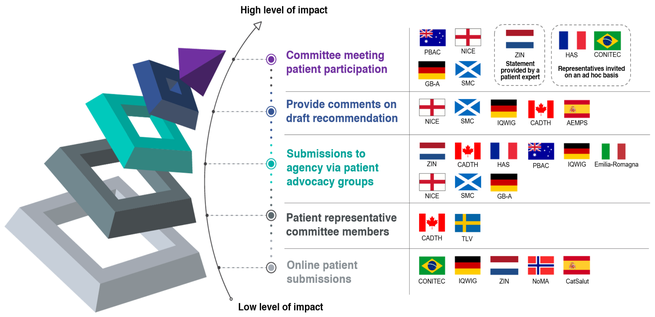
Country	Agency	Scope	Patient input opportunities	Impact*
Australia	PBAC	National	• Carers, patients, member of the public, consumer interest group or health professionals can submit comments on a HTA assessment • Patient groups representatives can be invited to PBAC meetings which consist of PBAC chair, PBAC members, clinicians, manufacturer representative, patient groups, departmental personnel. Also PBAC has additional stakeholder meetings for medicines that are used to treat serious / life threatening disorders but have not been recommended due to cost-effectiveness	High
Brazil	CONITEC	National	• Since 2012 – July 2017 CONITEC has received 30,000 online submissions from patient groups, patients, carers, family members and healthcare professionals • On a case-by case basis patient experts are invited to attend committee meetings	Medium
Canada	CADTH	National	• CADTH calls for patient group comments via a formal online submission process. Patients have a total of 35 days to prepare, provide and submit their online submission • The input is reviewed and considered by CADTH advisory body known as Canadian Drug Expert Committee (CDEC)	Medium
England	NICE	National	• Patient / carer groups can recommend a patient expert to take part in committee meetings and provide comments on appraisal consultation document and attend NICE appraisal meetings to participate in discussions • Patient organisations can also provide written submissions to NICE	High
France	HAS	National	• Formal patient input is restricted to patients submitting written contributions to TC but invitations to such TC meetings are not commonly routine • A template document provided by HAS is sent to patient organisations to fill out with specific areas mentioned – a patient organisation can submit only one submission per topic. Individual patients / carers are not authorised to contribute • Disease specific patient representatives are not included as full voting members of the committee, instead general patient representatives are included	Medium
Germany	Gemeinsamer Bundesausschuss	National	• Recognised nationwide advocacy groups can take part in petitions and G-BA committee meetings, plenary meetings and sub-committee meetings • However, patient representatives are not allowed to vote	High
	IQWiG	National	• Patient input provided on IQWiG assessment report plans, preliminary report results and preliminary HTA assessment results • IQWIG contacts the patient representative of the relevant patient group and input is collected via a standardised questionnaire	Medium
Italy	AIFA	National	• The AIFA initiative (Open AIFA) to include patient input is limited to regulatory issues. Requests or input regarding issues on ongoing P&R procedures are currently not accepted • The current opportunity for patient involvement is low, however an AIFA press release in December 2011 supported patient input from patient representatives in a technical committee discussion for a Hepatitis C drug	Low
	SANITARIO EMILIA-ROMAGNA	Regional	• Patient advocacy groups can contribute data and information in the form of written memoirs and reports • Also there is 1 patient representative present in the HTA regional commission which is composed of 16 external commission members	Medium
Netherlands	Zorginstituut Nederland	National	• At the appraisal stage, ZIN consults with various stakeholders. Patient organisations have a duration of 2 weeks to provide comments via online patient submissions. This is followed up by a statement at a public meeting	Medium
Norway	Norwegian Medicines Agency	National	• Pilot scheme for patient organisations to formally submit online comments. A recent single technology appraisal (STA) of a gene therapy had included the patient comments in the appendix of the STA report • The current status of the pilot scheme is not well known and whether it will be fully adopted by NoMA	Low
Scotland	Scottish Medicines Consortium	National	• Standard appraisal pathway: registered SMC patient groups can provide patient input forms via patient or carer perspective and one representative per group can partake in SMC committee meetings to clarify comments and answer questions • PACE process: The manufacturer can request a PACE meeting for eligible rare disease or end of life treatments that receive a preliminary negative decision. The meeting involves SMC representatives, clinical experts, patient representatives and a written statement from the manufacturer to be considered for the final decision	High
Spain	aem	National	Patient organisations can challenge the HTA report recommendation and express their opinions, however, there is a lack of transparency to determine how many patient groups have expressed their thoughts on AEMPS HTA reports	Low
	CatSalut Servei Català de la Salut	Regional	• Formal comment submissions and interaction of patient, professionals and carers on ongoing HTA decision is facilitated by AQuAS (CatSalut's public entity), whilst collaborating with the patient advisory council • The patient advisory council has 15 disease specific groups registered • Feedback from the regional report is shared with the AEMPS. Due to a lack of transparency the level of impact on the HTA report is unknown	Medium
Sweden	TLV Tandvårds- och läkemedelsförmånsverket	National	Non-disease specific patient organisation representative are appointed by the government to participate in TLV committee meetings to provide a general patient perspective	Low

**Researchers interpretation of the potential for identified patient input opportunities to impact the final decision*

Secondary research identified various different approaches for the consideration of patient involvement across the key national & regional HTA agencies from the scope countries (Table 1). Agencies developed multiple opportunities for patient involvement levels at different stages of the HTA process.[4-18,22,32]

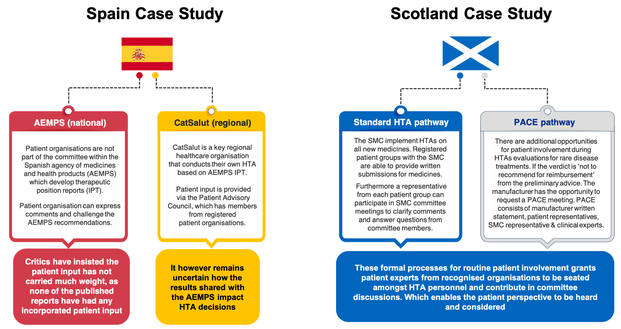
Part 2: Patient input opportunities

Fig 1: Classification of patient input opportunities



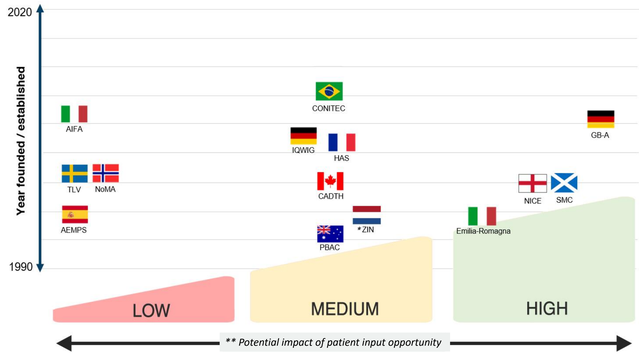
The commonalities and differences of the different types of patient input were compared and categorised into five broadly distinct groups (Figure 1). However, there remains variation between the approaches taken by each agency within each category. For example the level of patient representatives participation in committee meetings ranges from routine involvement in discussions, presenting a statement to ad hoc participation.[4-23]

Fig 2: Case studies of patient input opportunities in Scotland & Spain



Two case studies demonstrating different patient involvement opportunities and their potential impact on HTA decision for Scotland and Spain are presented in Figure 2. Opportunities in Scotland ranging from patient representative participation in committee meetings for rare diseases to written submissions and committee discussion for non-rare diseases. While Spain has varying patient involvement, ranging from regional online patient submissions to providing comments on draft HTA recommendations nationally.[15-17, 24-27, 32]

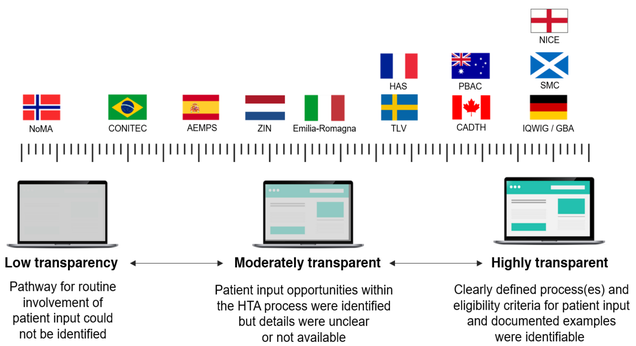
Fig 3: Comparison of potential impact of current patient input opportunities with year of HTA establishment



** ZIN formerly known as CVZ – 2014*
***Researchers' interpretation of the potential for identified patient input opportunities to impact the final decision*

Date of HTA agency establishment (or where possible), the date agencies started conducting HTA activities was identified and compared to perceived potential impact of current patient input opportunities (Fig 3).[4-5, 7-14, 28-31,33]

Fig 4: Transparency of HTA agency websites for identifying patient input pathways & nature of involvement



There was a varying degree in the availability of information identified from public sources as demonstrated below in Fig 4.

Discussion & Conclusion

Payers face an increasingly challenging environment with a burgeoning pipeline of orphan drugs, cell and gene therapies. These treatments are often for diseases with a high unmet need and further raise the importance for considering the patient perspective in decision-making. We identified and categorised the variation of approaches in to 5 groups with varying levels of opportunity to impact decision-making.

We also explored whether agencies with a longer history of HTA have evolved more opportunities for patient involvement in their HTA process. However, a comparison of the date of agency establishment with perceived impact of patient input opportunities does not indicate a trend. Other factors, which were not explored as part of this research, could also have influenced the type of patient input pathways agencies offer, such as the number of employees and budgets or funding.

Identifying details of patient involvement opportunities throughout the HTA process, eligibility for participation and the nature and format of input varied across agencies. These aspects were accessible and easy to find for agencies with highly transparent websites / publications (e.g for NICE, GBA, IQWIG and SMC), but the majority of agencies included in this study should develop transparent and formal processes to ensure patient input is provided by appropriate patient experts to inform reimbursement decision-making.

This research highlights the variety of patient input opportunities available. Having a seat at the table provides greater opportunity for patient input to have an impact on committee discussions compared to written submissions. However, the impact of the different forms of patient input on the final HTA decision largely remains unclear.

The outputs of this research are useful for patients to understand what types of input opportunities are currently available in their country and for other stakeholders to understand how patients have a say in the HTA decisions. Incorporation of the patient feedback in HTA decision making is crucial given patients have first-hand experience of the disease. Perhaps, in the future authorities can become more transparent with robust and clear processes to involve more patients within their decision-making process. Possibly there is a greater need to have these clear and robust pathways, such as PACE pathway, for treatment relating to patients with rare disease who have the highest disease burden and unmet need. This research needs further validation with the different agencies to further explore the impact of patient involvement.

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Acronyms

AIFA: Italian Medicines Agency. **AEMPS**: Spanish Agency of Medicines and Medical **AQuAS**: Agency of Health Quality and Assessment of Catalonia **CADTH**: Canadian Agency for Drugs and Technologies in Health **CatSalut**: Catalan Health Service **CDEC**: Canadian Drug Expert Committee **CONITEC**: National Committee for Health Technology Incorporation. **CVZ**: Health Care Insurance Board. **G-BA**: Federal Joint Committee. **HAS**: French National Authority for Health. **HTA**: Health Technology Assessment. **IQWIG:** Institute for Quality and Efficiency in Health Care. **IPT**: Therapeutic position reports **NICE**: National Institute for Health and Care Excellence. **NoMA**: Norwegian Medicines Agency. **PACE**: Patient and Clinician Engagement Meeting. **PBAC**: Pharmaceutical Benefits Advisory Committee. **SMC**: Scottish Medicine Consortium **STA**: Single Technology Appraisal **TC**: Transparency commission. **TLV**: Dental and Pharmaceutical Benefits Agency **ZIN**: Zorginstituut Nederland – National Healthcare Institute