HTA133 (ISPOR)

PATIENT INVOLVEMENT INITIATIVES IN HTAS; HOW CAN WE DO BETTER?

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BACKGROUND

- Patient representatives (PRs; individual patients, carers, patient organization representatives and patient experts) provide important data around the burden of living with a medical condition, and day-to-day experience of receiving current treatment options.
- Patient Involvement initiatives (PIIs) play an important role within HTA processes, ensuring the patient perspective is meaningfully included in decision-making. However, some HTA bodies have been criticized for showing a lack of willingness to fully integrate patient input[1].

OBJECTIVE(S)

- This study aims to evaluate the role of PIIs in HTA in four European countries, provide insight, and recommend improvements for how the patient perspective can be integrated into HTA.

RESULTS

Table 1: Rating output

Metric	UK	Germany	Spain	Poland
Transparency	High	High	Low	Low
Involvement	High	Medium	Medium	Low
Support	High	High	Low	Low
Inclusion	High	Medium	Low	Low
Consideration	High	Low	Low	Low

The results are provided in **Table 1**.

Rating outputs

- **Transparency:** Both NICE and IQWIG publish guidance that details stages of the HTA process where patient input is included[2,3]. In Germany, patient involvement is legally ensured through social security laws that guarantee consultation rights in reimbursement decisions[4]. Conversely, AOTMiT and Spain's regional HTA bodies lack published materials on patient involvement in HTA. RedETS has produced a framework for PIIs in HTAs, which has been incorporated by six out of the eight regional agencies[5].
- **Involvement:** In the England and Germany, PRs contribute at key HTA stages such as scoping, evidence appraisal, and consultation, providing input via working groups, committees, and testimonials[2,3]. English PRs can also contribute to final guidance development and appeal HTA decisions[6], unlike in Germany, where PRs cannot appeal[7]. In most Spanish regions, PII involves focus groups and draft guidance reviews, while Poland's AOTMiT limits input to a single written submission.
- **Support:** England and Germany offer financial compensation, training on their respective HTA processes and plain language materials to allow PRs to better understand the technical details of assessment [8,9]. NICE's Public Involvement Program (PIP) and The Patient Participation Unit leads these efforts in England and Germany respectively[2,3]. Poland's AOTMiT held a training session in 2017[10], and the Basque region Osteba provides a brief HTA overview, but further support in Spain and Poland is limited.
- **Inclusion:** NICE's PIP recruits PRs through open website advertisements which are distributed to relevant patient networks and organizations who are encouraged to share amongst their members[8]. In contrast, Germany restricts PR recruitment to patient organizations within a select group of approved patient group associations[12]. There is no publicly available information on patient representative recruitment processes in Poland or Spain.
- **Consideration:** In England PRs to complete exit surveys, and NICE reviews PII processes annually. The Department of Health and Social Care conducts a triennial review which includes an assessment of PIIs[8,13]. No publicly available information from Germany, Poland or any of the Spanish regions indicate any efforts to review PIIs.

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16. Guidance for patient involvement in HTA - EUPATI Toolbox

METHODS

- Methods are summarized in **Figure 1**. Current general methods, methodologies, guidelines, published articles, and surveys of HTA agencies were analyzed to assess the extent of PII impact in HTAs in each country. The HTA agencies analyzed were NICE (England), IQWiG (Germany), AOTMiT (Poland) and all regional agencies within the Spanish Network of Agencies for Assessing National Health System Technologies and Performance (RedETS). These countries were chosen to reflect the diversity in PII approaches and levels of integration across Europe.
- Each country's PIIs were assessed on the extent of agreement with the domain definitions, across 5 domains using a three-point score (low, medium, and high)

  Transparency: The organizations responsible for HTA have clear and publicly available processes for how PIIs are incorporated as part of HTAs.

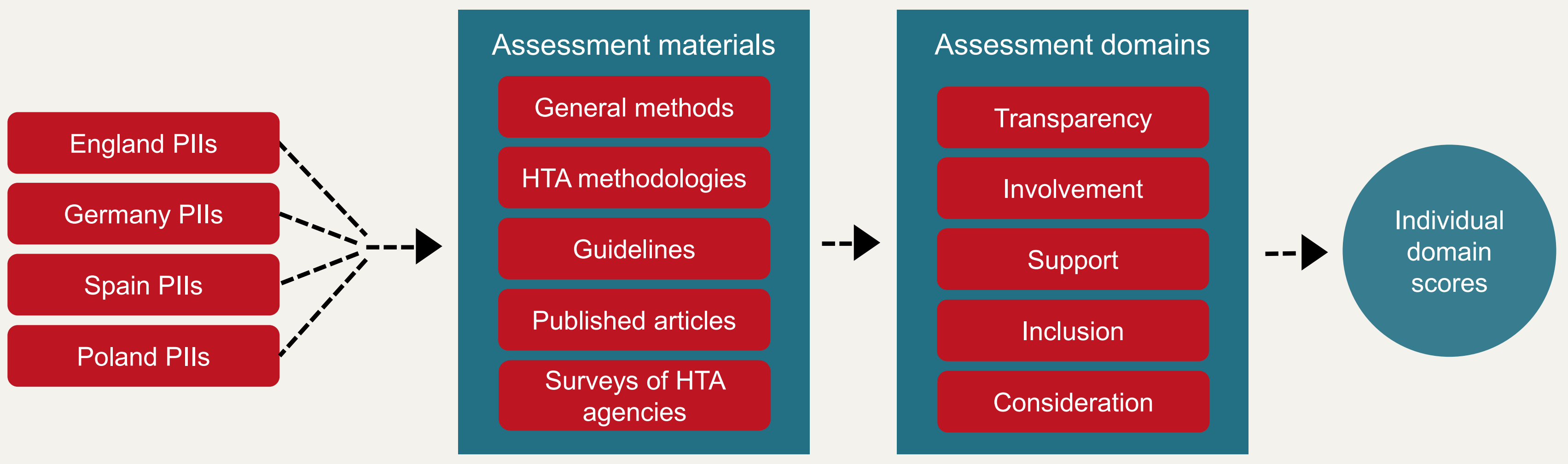
  Involvement: Patient perspectives are considered at every pertinent stage of the HTA process, ensuring their input has a meaningful effect on decision-making.

  Support: Appropriate resources are allocated to ensure effective patient involvement in HTAs, including training and financial support for those participating.

  Inclusion: Efforts are made to engage a diverse range of patient perspectives, ensuring that all relevant segments of the patient community are represented.

  Consideration: PII processes in HTAs are regularly reviewed, considering the experiences of all those involved.

Figure 1: Methods diagram



England PIIs
Germany PIIs
Spain PIIs
Poland PIIs

Assessment materials
General methods
HTA methodologies
Guidelines
Published articles
Surveys of HTA agencies

Assessment domains
Transparency
Involvement
Support
Inclusion
Consideration

Individual domain scores

DISCUSSION

- PIIs are most impactful when there is inclusive and transparent participation of PRs in the HTA decision-making process, leading to improved outcomes where patient's real-world experiences and needs are considered.
- Common stumbling blocks to meaningful PIIs include inadequate support and education, the technical nature of HTA, and a lack of resources to facilitate effective engagement.
- Granting PRs the right to appeal on final reimbursement decisions and establishing a legal requirement for patient input in HTA are crucial to ensure patient perspectives actively influence outcomes, aligning decisions with patient needs. However, there is still debate around the level of decision-making power and involvement that patients should have in an HTA, because reimbursement decisions affect the population as a whole, through the impact on the budgetary constraints of the health system.

CASE STUDY

- In England, the Sickle Cell Society (SCS) appealed NICE's final guidance decision not to recommend voxelotor for haemolytic anaemia caused by sickle cell disease (SCD)[14]. The SCS argued that the NICE committee failed to consider barriers to access and uptake and racial health inequalities faced by SCD patients, who are disproportionately of African or Caribbean descent, and cited NICE's obligations under the 2010 Equality Act[15]. Following the appeal, and a price reduction, voxelotor was approved through NHS England's Innovative Medicines Fund[14].
- This case study highlights several attributes of a successful PII involvement in HTA. The ability to appeal final guidance as part of a formal framework for PIIs adds an extra layer of scrutiny to the HTA process and gives patients a direct mechanism to influence outcomes. The inclusion of legal frameworks, such as equality legislation, strengthens the role of patients by obligating HTA agencies to adequately consider patient input fairly and transparently.

CONCLUSIONS

- To maximize patient input throughout the HTA process, agencies should implement mandatory reviews of current engagement processes and create improvement plans. We also recommend establishing consistent frameworks across European countries to clearly define the mechanisms for PIIs, supported by legislation guaranteeing a minimum standard for patient participation.
- To further support PIIs, agencies should allocate resources to engaging a diverse range of patient perspectives during recruitment and provide clear, accessible resources for participating PRs, covering both the HTA process and the clinical and economic aspects of the assessment.