# WORKSHOP REPORT

## Meaningful patient involvement in regulatory and HTA decision making – Current practices and impact on the final assessment

**1st-2nd October 2025**
**Delta Heathrow Windsor Hotel**
**UK**

CIRS Centre for Innovation in Regulatory Science

# Executive summary

## Background

Patient engagement (PE) and the use of patient experience data (PED) are now recognised as essential elements in the development, regulation, and health technology assessment (HTA) of new medicines, helping ensure decisions reflect patient needs and priorities.

Various regulatory and HTA agencies globally have established mechanisms to enable both PE as well as the collection and use of PED; however, there is a need to identify how best to utilise and articulate how the information has been used as well as its impact on the final decision. A survey conducted by the HTAi Interest Sub-Group for Patient/Citizen Involvement in HTA showed that although patient involvement in HTA is considered important, shortcomings need to be addressed including the lack of systematic and transparent processes for patient involvement.

Building on earlier efforts — including the 2015 Centre for Innovation in Regulatory Science (CIRS) workshop and work by the Clinical Trials Transformation Initiative (CTTI), Patients Active in Research and Dialogues for an Improved Generation of Medicines (PARADIGM), and Patient-Focused Medicines Development (PFMD) — CIRS formed a Topic Group in 2022 of various stakeholders to suggest research activities in this area for the CIRS 2024-2026 Research Agenda. This culminated in a multi-stakeholder workshop held in the UK on 1st – 2nd October 2025, which examined how patient input is being integrated and communicated in regulatory and HTA decision making.

The workshop brought together representatives from patient organisations, industry, regulators, HTA agencies and payers to discuss challenges, opportunities, share case studies, and develop recommendations for improving the measurement and articulation of patient input in agency assessments.

## Workshop objectives

- Discuss the value of engaging patients in early development and how this aids downstream decision making.
- Clarify how regulatory and HTA agencies are utilising PE and PED within their review and assessment frameworks.
- Identify the challenges and opportunities for measuring the utilisation of patient input in the evaluation of new medicines and how this can be best articulated.
- Make recommendations on key components for a systematic structured approach to documenting how PE/PED was used during the assessment and the articulation of its influence on agency decision making.

## Definitions

Adapted from the US Food and Drug Administration (FDA):

**Patient engagement (PE):** Activities that involve patient stakeholders sharing their experiences, perspectives, needs, and priorities that help inform an agency.

**Patient experience data (PED):** Information that captures patients' experiences, needs and priorities related, but not limited to: 1) the symptoms of their condition and its natural history; 2) the impact of the conditions on their functioning and quality of life; 3) their experience with treatments; 4) input on which outcomes are important to them; 5) patient preferences for outcomes and treatments; and 6) the relative importance of any issue as defined by patients.

# GRAPHIC SUMMARY

## Meaningful Patient Involvement in Regulatory and HTA Decision Making - Current Practices & Impact

Patient engagement (PE) and patient experience data (PED) are increasingly valued but lack consistent integration and visibility in regulatory and HTA decision making.

A CIRS multi-stakeholder workshop explored practical steps for embedding meaningful PE and PED in regulatory and HTA processes.

### CURRENT LANDSCAPE

- Regulators and HTA agencies increasingly considering PE/PED
- Visibility of impact remains limited
- Fragmented approaches across agencies

### KEY CHALLENGES

- Limited guidance on PED
- Representativeness and conflict of interest in PE
- Funding of patient organisations

### OPPORTUNITIES

- Early scientific advice
- PE-informed PED
- Collaborative models to reduce duplication and share learnings
- Disease-level engagement for deeper insights and greater efficiency

### RECOMMENDATIONS FOR CHANGE

**Policy support**
Frameworks for meaningful PE & PED

**Transparency**
Show how patient input informs regulatory/HTA decisions

**Alignment**
Coordinate across agencies to reduce duplication

## Key points from the plenary sessions

**Embedding patient involvement into early development to enable downstream decision making**

The workshop began with an introduction to meaningful PE, which can be visualised through Arstein's ladder of citizen participation, though this model requires adaptation for the scientific context of medicine development, regulation and reimbursement. Meaningful PE needs to be mutually understood by patients and the stakeholders they engage. Both sides must explain their limitations and discuss what can be reasonably achieved together, along with plans for future improvement. More research on engagement methods is needed to determine which methods work best and when, and how to create conditions for open dialogue.

Industry needs to be more strategic and systematic in involving patients early in development processes. While there are challenges, such as accessing the right patients at the right time, these can be tackled more easily with proactive planning informed by guidance and resources, such as the Patient Engagement Management Suite developed by PFMD. Requesting patient involvement in early scientific advice can also help to shape patient-centric trial designs and evidence generation.

A variety of methods are available for collecting PED, such as patient-reported outcome measures, patient preference studies, focus groups and in-depth interviews. However, the key challenge is deciding which methods are most appropriate and will be accepted by regulatory and HTA decision makers. For PED to have meaningful impact, agencies must integrate PED into their decision-making processes, treating it with the same importance as other types of evidence. Clear guidance on the generation and use of PED is essential.

**Current practices for patient input into regulatory and HTA decision processes**

The next workshop session focused on current practices for PE and PED in regulatory review and HTA assessment and how these are evolving.

A landscape study coordinated by PFMD has shown that regulators and HTA agencies worldwide are increasingly considering PE and PED in their decision making, although usually as separate elements. Integrating PE into the design and interpretation of PED programmes would help to maximise the value of patient input in regulatory and HTA decisions.

*Agency case studies*

Agencies shared insights into the variety of ways that they engage with patients. For example, in the **Australian HTA system**, the main input from the public (patients, patient groups, carers, general public, healthcare providers and organisations) on an HTA application is in writing. These written submissions enable the HTA committees to better understand individual patient experience and insights alongside population-based clinical and economic evidence.

At the **European Medicine Agency (EMA)**, a permanent working party serves as a forum for discussion with patients and their representatives and monitors the implementation of PE in the agency. Patients give direct input into decision-making committees, either as members (e.g. Committee for Orphan Medicinal Products, Paediatric Committee), or as experts (e.g. Committee for Medicinal Products for Human Use).

The **Scottish Medicines Consortium**'s Patient and Clinician Engagement (PACE) process is specifically for rare and end-of-life conditions and brings together patients, carers and clinicians to discuss the added value of a medicine. The resulting PACE statement provides the HTA committee with insights that would not normally be fully captured

in conventional assessment. Feedback indicates that patients highly value the opportunity to participate in PACE meetings.

The **Dutch HTA agency, ZIN**, has found different experiences with patient involvement between patient organisations/disease areas: for larger disease areas, it is often easier to find patient experts, and while some patient organisations have well-developed expertise in HTA and health economics, others (particularly for rare diseases) do not. Feedback from patients on their involvement has highlighted challenges, such as short timeframes for consultation responses, and lack of clarity in how their comments are incorporated into assessments, appraisals, and final recommendations.

*Company case studies*

Companies shared examples of how they had built PE and PED into their regulatory and HTA submissions. One company focused on a treatment for pruritus (itching) in primary biliary cholangitis (an autoimmune liver disease), highlighting how patient-reported outcomes (PROs) were central to the clinical programme, with itch relief as a primary endpoint and extensive qualitative research to understand patient experiences.

Another company described a similarly detailed approach for alopecia areata, where various methods were used to capture patient quality of life beyond standard measures like EQ-5D. This included vignette/time trade-off studies, where people were asked to compare different health scenarios by indicating how much of their lifespan they would be willing to trade for perfect health, as well as conceptual models and extensive patient interviews.

Both company case studies emphasised the importance of early and frequent patient engagement, the need to go beyond traditional clinical measures, and how patient-centred approaches can drive clinical relevance, regulatory/HTA credibility, and meaningful patient impact.

*CIRS survey*

CIRS conducted a survey of regulatory and HTA agencies focused on agency-level activities related to PE and PED, including how the impact of such activities is measured and communicated, and future thinking on this topic. Responses were received from 13 regulators and 18 HTA agencies covering Latin America, North America, Asia and Europe.

The survey results implied that HTA agencies have broader engagement in PE/PED activities and are more likely to have a formal approach to PE/PED than regulatory agencies, suggesting a stronger integration of patient input in HTA processes. Most agencies (both regulatory and HTA) do not have impact measures or direct feedback to patients on the use of PE/PED in their deliberations and decisions; the main communication channel is through public assessment reports.

Concerns about biases and representativeness are key challenges to agencies undertaking PE or receiving patient evidence. However, over the next five years, most agencies believe PE/PED will have greater influence on assessments and appraisals. Guidelines to ensure quality, rigour, and representativeness of data, along with infrastructure and resources to support patient groups, are needed going forward.

**Visibility of patient input in regulatory and HTA decision making**

This session explored the visibility of patient input in agency decision making, in terms of how it is used, the impact it has, and how these are aspects are communicated.

Visibility of patient input matters because it strengthens the legitimacy of using patients as a source, builds trust in the process, reinforces institutional credibility and increases participation and meaningful input for future. Measuring impact is challenging but important, requiring both quantitative (e.g. number of decisions or reports in which patient input is explicitly quoted) and qualitative approaches (e.g. narrative examples of impactful interactions).

Agencies shared their current practices and future ambitions for PE/PED use and visibility.

The **EMA** has recently published a PED reflection paper to encourage the use of PED in medicine development and increase understanding of the way the agency assesses PED, including the rationale for acceptance or exclusion for benefit-risk decision making. A key message is that PED must be of high quality, and scientific advice and qualification of novel methodologies are the best avenues to address quality concerns. The EMA assessment report template has been updated to improve transparency of submitted PED and how it has been used by the agency.

The **FDA** includes a PED table in all reviews and public assessment reports, which conveys what type of PED was submitted and considered. Examples were presented showing how different types of PED can have different utilities in the review process. Not all PED needs to be submitted to FDA, particularly if it informs internal decisions and processes. Applicants should "tell the story of the PED" so the FDA can understand what was collected, its location within the dossier, and how it will contribute to the review process.

The **National Institute for Health and Care Excellence (NICE)** supports integration of PE and PED into its work through three networks: a People and Communities Network of 250 individuals with lived experience, a Voluntary and Community Sector Organisation Network of 250 patient organisations, and an internal Involvement and Engagement Leaders Network to build capacity within the organisation. In NICE's experience, the impact of patient evidence exists on a continuum: where there is good quality evidence with no gaps, patient information provides context and confirms assumptions; where evidence is limited, patient evidence can have more impact by filling gaps and explaining what's missing.

**Canada's Drug Agency** incorporates patient perspectives into its reimbursement reviews through patient group input, patient committee members, and in certain scenarios, presentations by persons with lived experience. The agency is evolving its patient group input process and focusing on improving how it communicates the impact of patient group input in recommendation reports.

The **Institute for Clinical and Economic Review** in the US has a continuous process of PE with multiple forms and levels of engagement. When measuring how PE/PED impacts decision making, it's important to consider impact from all stakeholder perspectives. While standardisation of communications about PED/PED may be challenging due to variations in processes and resources across organisations, there are opportunities to establish common principles focused on transparency, dialogue, and clear communication.

From an **industry perspective**, though progress has been made with recognition of PED, improvements are needed such as guidance on requirements for including PED in the regulatory label (to ensure relevance in clinical decisions), international convergence and common criteria, and transparency about the relevance of submitted PED and the rationale for not considering it. The challenge is balancing the complex ecosystem of regulatory and HTA decision making, the hierarchy of evidence, resource requirements, the need for predictability, avoiding misunderstandings by the patient community, and preventing undue promotion.

**Supporting patients and patient organisations to share their perspectives**

A panel of patient, company, regulatory and HTA agency representatives reflected on the challenges patients and patient organisations face to provide their perspectives and collaborate with other stakeholders.

Public funding of patient organisations is declining, creating issues around conflict of interest (COI) and private financing. For example, the COI framework of the EU HTA Regulation does not support case-by-case assessments of declared interest for patients and patient groups, which could lead to exclusion of relevant perspectives in EU HTA processes. Patient representatives wish for greater focus on transparency and managing COI rather than automatic exclusion, calling for more flexible context-sensitive COI frameworks, better understanding of what constitutes a COI, and risk assessment tools to help navigate these issues.

Confidentiality agreements between agencies and patient representatives are another challenge; while essential for legal reasons, they prevent individuals from gathering wider - and potentially richer - input from their patient communities.

It is also difficult to determine the appropriate level of patient expertise needed to give input into regulatory and HTA processes. While training is important to support patients through these processes, there are concerns about turning patients into regulatory/HTA experts that are not representative of the population. In addition, it can be challenging to ensure diversity of patient input.

To optimise the value of patient input, engagement needs to begin early, not only between companies and patients but also between companies and agencies through early scientific advice. Although it is challenging to provide feedback to patients about how their input influenced decisions, it is essential to avoid tokenism and provide transparency that will help drive future patient participation.

**Future thinking for PE and PED**

The final session of the workshop explored the future outlook for PE and PED and potential solutions to current challenges.

While the importance of patient evidence data (RWE, PROs, PROMs and patient preferences) is growing, there are challenges in collecting, validating and interpreting these types of data. While methodologies can be iteratively improved, ultimately guidance and alignment are needed on how this evidence will be interpreted and considered, and what the full impact of this evidence will be when submitted to agencies. For patient preferences, more real-world examples of application in regulatory and HTA decision making are needed to promote broader adoption.

There is strong agreement on the importance of PE and PED; the focus now needs to shift from ‘why’ to ‘how’ to integrate PE and PED in development, regulatory and HTA decision making. Nevertheless, it’s important to recognise that implementing meaningful change takes time, as cultural shifts and process modifications are needed.

Continued collaboration across stakeholders to establish a common language for PE and PED, share best practices and test new approaches, is key. A systems-oriented approach is needed, focusing on shared objectives for PE and PED rather than individual organisational requirements. Moving from individual asset-level to disease-area engagement could be a potential strategy to improve the efficiency of PE processes.

PE can be limited by practical, financial and psychological barriers. Overcoming these through increased support, clear expectations, co-creation, sustainable funding of patient organisations, and better knowledge sharing would enable more effective PE and ultimately better outcomes for patients and their families.

## Recommendations from the breakout discussions

**Creating supportive policies for meaningful PE and PED**

- Engage early on PE and PED, using clear criteria to decide relevance. Establish guiding principles, a best practice repository, and a global patient organisation forum to coordinate PE/PED requests, with regulators and HTA bodies working alongside patient organisations.
- Focus PE and PED efforts at the disease level, not just the product level, to align endpoints with both patient and regulatory needs. Consider creating a disease PICO (Population, Intervention, Comparator, Outcomes) framework, involving industry, patient organisations, regulators, and HTA agencies.
- Promote clear internal company messaging on how PE and PED support clinical development and regulatory/HTA interactions, helping teams understand the value and application of PE and PED.
- Collaborate with patient organisations as equal partners, engaging them early with transparent communication, education, and training.

**Showing the impact of PE and PED in public-facing regulatory and HTA documents**

- Conduct research on how to better articulate results from PE/PED to patients to inform their decision making e.g. enhanced patient leaflets.
- Evaluate existing guidance on PED generation and identify gaps where further harmonisation is needed across agencies.
- Describe situations where PED are particularly useful e.g. case studies.
- Harmonise definitions of PE and PED, as well as where evidence is generated from to inform decision making – what does high quality PED look like?
- Train and support patient organisations to generate robust PED to inform regulatory and HTA decision making.
- Evaluate the impact of PED in different HTA systems e.g. utilities vs relative clinical benefit systems.
- Improve communication of patient input in pharmacovigilance.
- Increase transparency of PED and PED activities that have high impact but low visibility by publishing evidence in peer-reviewed journals.
- Increase quality of PE and PED activities that have low impact but high visibility by introducing standards for engagement and evidence.
- Standardise a checklist for PE and PED information for regulatory and HTA agencies. CIRS could perhaps lead this work.
- Regulatory and HTA agencies should provide clarity on the role of PE and PED information in decision making.

**Aligning patient involvement across regulatory and HTA agencies**

- Track how regulatory and HTA agency perspectives evolve over time with increased PE and PED usage. CIRS could perhaps delve deeper into the results of its agency survey and repeat this in future.
- Gather patient perspectives on current gaps in infrastructure and policy in regulatory and HTA processes.
- Seek recommendations from patient advocacy groups and patient experts on how submissions to regulatory and HTA agencies can be made more efficient. CIRS could perhaps lead this work.
- Organise roundtable discussions across stakeholders to avoid duplication in PE/PED.
- Align on definitions of PE and PED.

# Workshop programme

## Day 1: 1st October 2025

| Session 1: Embedding PE into early development – What is the purpose and how can this enable improved downstream regulatory and HTA decision making? | |
|---|---|
| ***09:00*** | **Chair's welcome and introduction – Niklas Hedberg,** Chief Pharmacist, TLV, Sweden |
| ***09:10*** | **Meaningful patient involvement in regulatory and HTA decision making – François Houÿez,** Director of Treatment Information and Access, EURORDIS – Rare Diseases Europe, France |
| ***09:30*** | **Discussion** |
| ***09:35*** | **PED: An overview of methods and practice - Dr Martina Garau,** Director, Office of Health Economics (OHE), UK |
| ***09:50*** | **How can PE build value into the Target Product Profile (TPP): What are the opportunities and challenges? - Kate Trenam,** Head of PE&A Neurodegeneration / Neuroinflammation, UCB, UK |
| ***10:05*** | **Patient involvement at the MHRA- Julian Beach,** Executive Director, Healthcare Quality and Access, Medicines and Healthcare Products Regulatory Agency (MHRA), UK |
| ***10:20*** | **Discussion** |
| ***10:45*** | **Break** |
| **Session 2: Patient input into regulatory and HTA agencies decision processes – What are current practices and how is the landscape changing?** | |
| *11:15* | **PE and PED in regulatory review and HTA: Where are we today? A landscape study - Hayley Chapman,** Senior Program Director, The Synergist |
| *11:30* | **Discussion** |
| *11:40*<br>*11:50*<br>*12:00*<br>*12:10* | **Agency case studies - How are agencies engaging patients directly/indirectly during the review and assessment process?**<br><br>**Use of patient submission summaries – Patient input through a formal written submission during HTA assessment – Prof Andrew Mitchell,** Honorary Professor, Department of Health Economics Wellbeing and Society, The Australian National University, Australia<br><br>**Added value of patient input in regulatory assessment and decision making – Dr Juan García Burgos,** Head of Public and Stakeholders Engagement Department, European Medicines Agency<br><br>**Patient and Clinician Engagement (PACE) process – Pauline McGuire,** Principal Pharmacist, Scottish Medicines Consortium (SMC), UK<br><br>**Patient involvement in the Dutch HTA process - Prof Wim Goettsch,** Professor HTA of Pharmaceuticals, WHO Collaborating Centre for Pharmaceutical Policy and Regulation, Utrecht University, The Netherlands |
| *12:20* | **Discussion** |
| *12:30*<br>*12:40* | **Company case studies – Building PE and PED into the submission so it can be practically utilised by agencies in their decision making: What are the company considerations?**<br><br>**Regulatory submission – Robyn von Malzahn,** Global Head, Patience Centered Outcomes GlaxoSmithKline, UK<br><br>**HTA submission – Dr Alice Biggane**, Associate Director, Outcomes Innovation and Research, Pfizer, UK |
| *12:50* | **Discussion** |
| *13:00* | **Lunch** |

| Session 3: Visibility of patient input in regulatory and HTA decision making - How should the impact be measured, what should be documented, and why? | |
|---|---|
| *14:00* | **Chair's introduction – Amelia Hursey,** Strategic Director, Parkinson's Europe |
| *14:05* | **Visibility of patient input: What is needed and why?**<br><br>**Mencia de Lemus,** Co-Chair Treatment Committee, SMA Europe, Spain |
| *14:20* | **Discussion** |
| *14:25* | **CIRS agency survey on the impact, measures, challenges and future direction for PE and PED in regulatory and HTA decisions**<br><br>**Dr Neil McAuslane,** Scientific Director, CIRS |
| *14:40* | **Discussion** |
| *14:45*<br><br>*15:00* | **How is PE and PED information used in the evaluation and decision-making process?**<br><br>**Regulatory perspective – Dr Juan García Burgos,** Head of Public and Stakeholders Engagement Department, European Medicines Agency<br><br>**HTA agency perspective – Laura Norburn,** Senior Operations Manager - People and Communities Team, National Institute for Health and Care Excellence, UK |
| *15:15* | **Discussion** |
| *15:20* | **Break** |
| *15:35*<br><br>*15:50* | **How are PE/PED activities considered during the assessment/decision process of new medicines communicated publicly?**<br><br>**Regulatory agency perspective – Robyn Bent,** Director of the Patient Focused Drug Development Program, CDER, Food and Drug Administration, USA (Virtual presentation)<br><br>**HTA agency perspective - Michelle Gibbens,** Director, Engagement, Strategic Relationships and Initiatives Business Unit, Canadian Drug Agency |
| *16:05* | **What would companies like to see articulated in public facing documents and why is this important?**<br>**Amaia Clemente,** Regulatory Science & Policy Associate Director Europe, Sanofi, Spain |
| *16:20* | **Panel discussion on what should be communicated and in what way – Is there a need for a systematic set of information to be communicated back to patients and companies? Should this be in the public assessment report and product information/package leaflet?**<br><br>**All session 3 speakers plus**<br><br>**Dr Marina Richardson,** Associate Director, HTA Methods and Health Economics, Institute for Clinical and Economic Review (ICER), USA |
| **Session 4: Breakout discussions** | |
| ***17:00*** | **Introduction to breakout discussions** |

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| | **Topic A: What is needed in the policy space to enable meaningful PE and PED to be generated and integrated into development that would be of value and aid regulatory and HTA decision making?**<br><br>**Chair: Dr Nick Crabb,** Chief Scientific Officer, NICE, UK<br><br>**Rapporteur: Sunera Awan,** Head UKI Regulatory Affairs, Bayer, UK<br><br>**Topic B1: Measuring the impact and visibility of PE and PED on regulatory review and HTA assessment within public facing documents – What could be feasible?**<br><br>**Chair: Kelly Robinson,** Director General, Pharmaceutical Drugs Directorate, Health Canada<br><br>**Rapporteur: Jessica Abel,** Director, PED Policy & Best Practices, Patient-Centered Outcomes Research, AbbVie, USA<br><br>**Topic B2: Measuring the impact and visibility of PE and PED on regulatory review and HTA assessment within public facing documents – What could be feasible?**<br><br>**Chair: Prof Hans-Georg Eichler,** Consulting Physician, Association of Austrian Social Insurance Institutions<br><br>**Rapporteur: Dr Siobhan Connor-Ahmad,** Principal Scientist, Patient-Centred Outcomes Research, Roche, UK<br><br>**Topic C: Cross-Stakeholder alignment – Can there be consensus on involving patients across regulatory and HTA processes to reduce duplication in PE/PED?**<br><br>**Chair: Dr Anke-Peggy Holtorf,** Founder and Managing Director, Health Outcome Strategies, Switzerland<br><br>**Rapporteur: Giorgia Rauso,** Associate Director, International Patient Advocacy, Regeneron, Italy |
| ***18:00*** | **End of day one** |
| ***19:00*** | **Reception and workshop dinner** |

## Day 2: 2nd October 2025

| **Session 4: Breakout discussions (continued)** | |
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| ***08:30*** | **Breakout discussions resume** |
| ***10:15*** | **Break** |

| Session 5: Feedback from breakout sessions and panel discussion | |
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| ***11:00*** | **Chair's introduction – Prof John Skerritt,** Enterprise Professor in Health Research, University of Melbourne, Australia |
| ***11:05*** | **Feedback from breakout rapporteurs, with discussion** |
| ***12:05*** | **Panel discussion – What is needed to address the challenges patients face and ensure support for patient organisations in their advocacy roles both at a company and agency level?**<br><br>**8 Minute perspective followed by open floor discussion**<br><br>**Patient perspective - Josephine Mosset,** Policy Officer, Cancer Patients Europe, Belgium<br><br>**Company perspective – Dr Siobhan Connor-Ahmad,** Principal Scientist, Patient-Centred Outcomes Research, Roche, UK<br><br>**Regulatory agency perspective – Dr Fokaline Vroom,** Medicines Evaluation Board, The Netherlands<br><br>**HTA agency perspective – Dr Anja Schiel,** Senior Adviser; Lead Methodologist in Regulatory and Pharmacoeconomic Statistics, NOMA, Norway |
| ***13:00*** | **Lunch** |
| **Session 6: Future thinking on PE and PED** | |
| *13:45* | **Chair's introduction - Dr Brian O'Rourke,** Chair, CIRS HTA Steering Committee |
| *13:55*<br><br>*14:10* | **From insight to impact: The future of PED in regulatory and HTA decision making**<br><br>**Innovative methodologies: Harnessing RWD and PRO in evidence generation to inform HTA decision making**<br><br>**Dr Thomas Butt,** Executive Director, Health Economics and Outcomes Research, Biomarin, UK<br><br>**Patient preference data – Designing robust patient-centred evidence frameworks**<br><br>**Dr Brett Hauber,** Patient Preference Evidence Integration Lead, Pfizer, USA |
| *14:25* | **Discussion** |
| ***14:35*** | **Panel discussion – Future thinking on PE and PED: How should these evolve and what are the challenges?**<br><br>**Representative perspectives from**<br><br>**Patient – Alastair Kent,** Chair of Trustees, Gene People, UK<br><br>**Company – Gonzalo Linares,** Global Head, R&D Patient Advocacy, Johnson & Johnson, Switzerland Global Head of R&D Patient Advocacy<br><br>**Regulatory agency – Dr Juan García Burgos,** Head of Public and Stakeholders Engagement Department, European Medicines Agency<br><br>**HTA agency – Michelle Gibbens,** Director, Engagement, Strategic Relationships and Initiatives Business Unit, Canadian Drug Agency<br><br>**Payer – Dr Michael Ermisch,** Head, AMNOG G-BA department, GKV-Spitzenverband, Germany<br><br>**Policy - Dr June Cha,** Director, Policy, Milken Institute, USA |

# Session summaries

*Please note that the following summaries represent the views of the individual presenters and do not necessarily represent the position of the organisation they are affiliated with. Included slides are attributed to the individual presenters and have been reproduced with their permission.*

## Session 1: Embedding patient engagement into early development – What is the purpose and how can this enable improved downstream regulatory and HTA decision making?

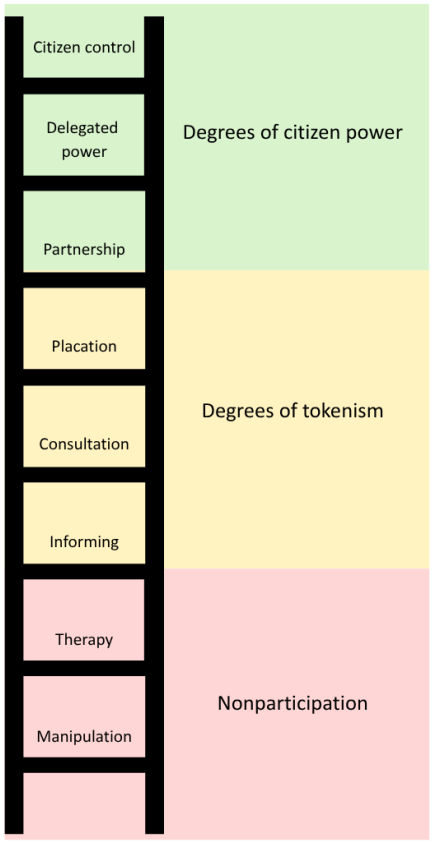
### Meaningful patient involvement in regulatory and HTA decision making: What are current practices and what impact does this have on the final assessment?

**François Houÿez,** Director of Treatment Information and Access, EURORDIS – Rare Diseases Europe, France

**Understanding meaningful patient engagement**

Meaningful patient engagement (PE) can be visualised through Arstein's ladder of citizen participation (see right), though this model requires adaptation for the scientific context of medicine development, regulation and reimbursement.

Arnstein's Ladder of Participation




At the bottom of the ladder are non-participatory methods: manipulation, such as situations where patients are invited to scientific discussions applying evidence-based medicine, but final decisions consider completely different aspects like industrial policy; and therapy, where authorities revert the problem to patients, such as asking them to decide which treatments should be reimbursed.

Although informing and consultation activities can be seen as tokenistic, they still have a role in healthcare, such as when authorities must explain difficult decisions to patients (information after a decision is made) or seek input on complex scientific topics (consultation). An example of placation is when a patient representative is invited to a prestigious committee without proper preparation for meaningful dialogue.

At the top of the ladder are partnership, which centres around shared decision making; delegated power, such as where citizens delegate decision making power to HTA bodies and regulators due to the specialised knowledge required; and citizen control, which may not be a valid way to regulate medicines or make reimbursement decisions.

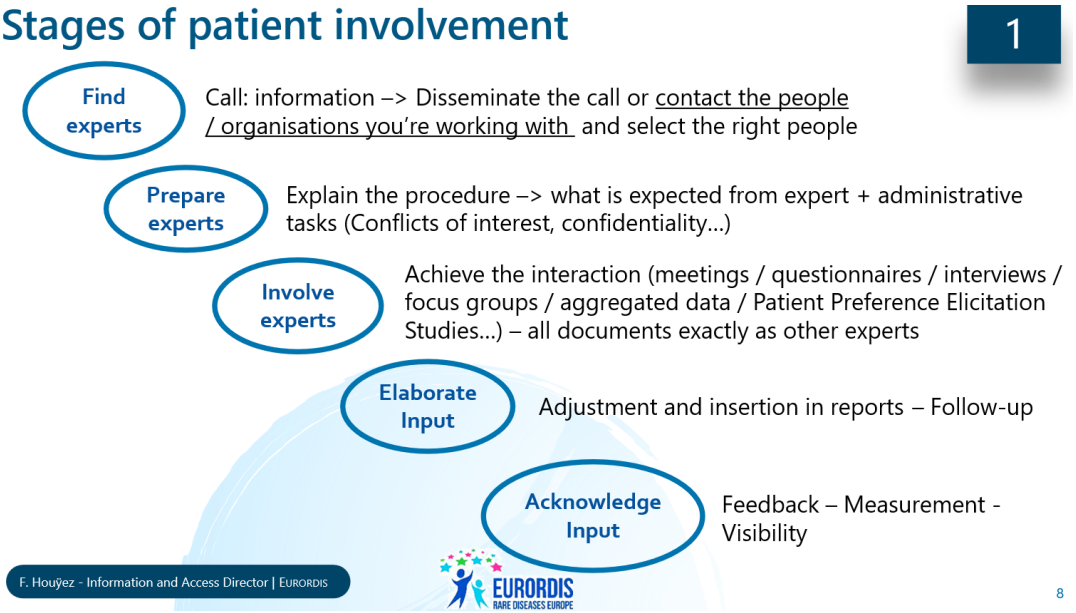
**Dimensions of PE**

PE has been described as having three dimensions: breadth (inclusivity), depth (level of involvement) and texture (quality of the interaction) (Brown & Bahri, 2019). These three dimensions should always be considered when developing a PE policy or discussing new methods to engage patients.

**Patient involvement in different contexts**

Patient involvement has different stages (see below) and varies across different contexts:

- In early development with companies, patient input can influence decisions about dosing and trial design.
- In pharmacovigilance, for example at the EMA, patients participate in a range of activities from spontaneous reporting of suspected side effects to public hearings or committee membership.
- In marketing authorisation assessments, patients can take part in oral explanations with the developer, where major objections could lead to a negative opinion.
- In HTA/coverage processes, patients can help to define to PICO (Population, Intervention, Comparator, Outcomes) questions, contribute to assessments, and even pricing discussions (though this rarely occurs).




**Patient preference studies**

Patient preference studies are extremely valuable, particularly in areas like multiple myeloma, where they can show how much improvement in progression-free survival would be needed to compensate for worsening side effects. These studies can be conducted in parallel to clinical trials to inform developers about how patients weigh benefits and risks. They are part of patient experience data (PED) that can inform assessors and decision makers on the patient perspective e.g. to what extent is the technology useful to patients?

**Conclusion**

Meaningful PE needs to be mutually understood by patients and the stakeholders they engage, and decisions to involve patients should be co-decided. Both sides must explain their limitations and discuss what can be reasonably achieved together, along with plans for future improvement. More research on engagement methods is needed to determine which methods work best and when, and how to create conditions for real dialogue rather than just responding to prepared questions. PED can complete the opinions of patients consulted during assessments or appraisals.

## Patient experience data: An overview of methods and practice

**Dr Martina Garau,** Director, Office of Health Economics (OHE), UK

Patient experience data (PED) encompasses a broad variety of elements including the different people who can be involved, types of data that can be collected, methodologies available, and timepoints when data can be gathered. The spectrum ranges from having patients as study subjects to having them as co-decision makers.

**Types of PED**

Different types of evidence can be collected depending on when it is gathered:

- Clinical outcomes data about symptoms, disease treatments, and health-related quality of life
- Preference data on the process of and/or the outcomes of a treatment
- Value assessment data demonstrating societal and novel elements related to treatments, such as the value of hope.

**Methodologies for data collection**

Many methods are available for collecting PED:

- Traditional methods for evaluating health-related quality of life, such as patient-reported outcome measures
- Quantitative patient preference studies that examine trade-offs between different treatment characteristics such as Discrete Choice Experiments (DCE)
- Qualitative studies, such as interviews and focus groups, can provide important information, particularly in early development, to inform quantitative studies.

**Timing of data collection**

PED should be collected across the entire lifecycle of an intervention to:

- Identify important endpoints aligned with patient priorities
- Gain insights into disease burden, and understand unmet needs
- Demonstrate treatment value beyond clinical measures
- Guide trial design and evidence generation plans.

**Use in regulatory and HTA contexts**

Patient-reported outcomes (PROs) complement traditional endpoints by capturing impacts on daily life beyond clinical measures. PROs have been used regularly by regulators, appearing in almost half of European public assessment reports issued between 2017 and 2022 (Meregaglia et al., 2023). Patient preferences are less systematically integrated into regulatory processes, which may be due to a lack of methodological consensus and guidance.

In the HTA context, traditional elements like health-related quality of life evaluations are generally well-established. However, less formal guidance exists for other types of PED and patient involvement activities, such as patient participation in HTA decisions (Kumar et al., 2024).

**Conclusion**

Embedding patient input across the lifecycle is key to ensuring medicines reflect patients' needs, preferences, and values. The challenge with PED lies not in the availability of methods but in reaching consensus on which methods are most appropriate and ensuring they are accepted by decision makers. To have a meaningful impact, PED must be integrated into decision-making processes and treated with the same importance as other types of evidence. Harmonising existing guidance, addressing methodological challenges, and leveraging existing initiatives and new regulations (e.g. EU Joint Clinical Assessment) may help to improve PED use.

OHE

## What's next

- **Early and continuous patient-centred development**: Embed patient input across the lifecycle to ensure medicines reflect patients' needs, preferences, and values. At early stage, it means informing trials and evidence generation plans.
- **Strengthening evidence base**: Collect and present PED that complements clinical and economic evidence.
- **Integration into regulation and HTA decision-making**: agencies should treat PED on par with other data, supported by clear and aligned guidance.
- **Advancing frameworks and methods**: Harmonise existing guidance, address methodological challenges, and leverage existing initiatives and new regulations (e.g. JCA) to improve PED use.

## How can patient engagement build value into the Target Product Profile (TPP) – What are the opportunities and challenges?

**Kate Trenam,** Head of Patient Engagement and Advocacy - Neurodegeneration/neuroinflammation, UCB, UK

The Target Product Profile (TPP) is a strategic planning tool that captures all product characteristics at a given time, gathering evidence from various sources and stakeholders including regulators, clinicians, and patients. The TPP enables evaluation of a product's value proposition and brings together multiple functions within a company. It evolves with the product through various iterations as development progresses.

**Focus areas for patient input**

Several key areas benefit from patient perspective in the TPP:

- Target population: Understanding demographics, disease stage, and chronicity to define who will benefit most.
- Unmet need: Determining current standard of care, efficacy gaps, and side effects from a real-world perspective.
- Dosage and administration: Exploring delivery methods, pill size/shape, IV, subcutaneous and other factors that affect patient adherence.
- Efficacy endpoints: Ensuring measurements are not only clinically relevant but meaningful to patients.
- Benefit-risk assessment: Understanding trade-offs patients are willing to make rather than making assumptions.
- Efficacy ambition: Determining what a product needs to achieve to be beneficial and meaningful to patients.

**Opportunities and challenges**

Integrating patient perspectives into the TPP offers opportunities such as deeper understanding of unmet needs, improved product usability, regulatory and HTA alignment, and more informed benefit-risk decisions. However, several challenges exist:

- Identifying and accessing the right patients, particularly in rare diseases, ultra-rare conditions, marginalised communities, and paediatrics.
- Integrating patient input into internal processes alongside scientific, regulatory, and commercial priorities.
- Ensuring patients understand the technical context of TPPs.
- Justifying investment in patient engagement (PE) during early development despite high attrition rates.
- Measuring and demonstrating impact, which is often requested for PE but not for other stakeholder input.
- Overcoming habits in clinical development to ask clinicians for the patient perspective, rather than patients themselves.

**Methods and resources**

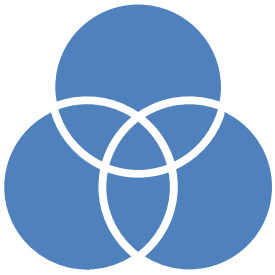
A range of methods exist for gathering patient input, including patient advisory boards, focus groups and interviews, online forums and bulletin boards, desk research and surveys, discussions with patient organisations, and social media listening. Resources like the Patient Focused Medicines Development "How-to guide for patient engagement in the early discovery and preclinical phases" provide valuable guidance for implementing effective PE in early development.

UCB developed a TPP playbook with Parkinson's UK, Parkinson's Foundation, and patient experts to ensure clinical trial teams consider patient involvement from the outset, including who to involve, how to involve them, and what information is needed. The TPP template includes a column to document PE activities, resulting insights, and how these informed the TPP.

**Conclusion**

Early patient involvement is not just good science but good strategy that benefits both developers and patients. Although integrating patient perspectives into the TPP has its challenges, such as ensuring diverse representation and measuring impact, these are not insurmountable. Industry needs to move from ad-hoc PE to systematic and strategic involvement with patients.

# Summary



- Patient Engagement is no longer a "nice to have"—it's a strategic imperative in shaping meaningful, patient-relevant Target Product Profiles.
- **Opportunities:** PE enables deeper understanding of unmet needs, improves product usability, aligns with regulatory expectations, and supports more informed benefit-risk decisions.
- **Challenges:** Integration into development processes, ensuring diverse representation, and measuring impact remain key hurdles.

## Patient involvement at the MHRA

**Julian Beach,** Interim Executive Director, Healthcare Quality and Access, Medicines and Healthcare products Regulatory Agency (MHRA), UK

Patient engagement in regulatory decision making involves understanding that there is no single patient voice, as each individual's reality is different. The challenge is determining how to segment and ensure the right patient input is obtained. Each assessment must consider the potential value of involving patient views, the timelines, the resources available, and the harm that could be caused by inadequately incorporating patient voice (both to public health and public trust).

**Methods of patient involvement**

MHRA accepts patient submissions on licensing applications, allowing patient groups to provide information for consideration. The agency has lay members on its Commission on Human Medicines and invites external experts and patient representatives to committee meetings. Written submissions can sometimes be easier for patients than speaking in meetings with academics, clinicians, regulators etc. It's important to remember that sharing a health experience is not cost free to individuals and can be emotive and distressing.

**Situations requiring patient input**

The following are some broad examples of circumstances in which MHRA would be likely to seek to incorporate the views of patients in the assessment of such products:

- **Lack of available data** – an application for a conditional marketing authorisation, for example, or where the intended patient population has a rare disease and there is little available research data.
- **Finely balanced benefit/risk decision** – to obtain patient/caregiver views on the level of benefit/risk that might be acceptable, plus other relevant factors such as the method of administration and frequency of treatment, or the type of screening and monitoring that might be required for safety reasons.
- **High level of unmet need** – to ensure that patients/caregivers have had an opportunity to input their views where the application concerns a treatment, or a condition, that has been subject to public and stakeholder interest due to, for example, there currently being a high level of unmet need or the innovative nature of the product.
- **Requirement for contextual information** – to help the assessment team understand the lived experience of patients with the relevant healthcare condition, and/or their caregivers, to provide the context within which the product would be used.

**Case study: Casgevy for sickle cell disease**

MHRA has worked with patient organisations on the approval of Casgevy, a gene therapy for sickle cell disease. Patient involvement in the approval process focused on sharing experiences of living with sickle cell and the impact on quality of life. This provided the agency with a better understanding of what it means to live with the condition and the importance of having effective treatment options.

While MHRA did engage with patient groups for Casgevy, earlier engagement would have been beneficial. The agency is now focusing on engaging early in the process, as this can change development pathways. MHRA is involving patients not only in decisions but also in the creation of guidance, such as for rare therapies, working alongside industry partners and academia to reflect the breadth of views across different drug classes.

**Conclusion**

Patient involvement is crucial throughout the regulatory process, from the creation of guidance to decision making. MHRA is working to ensure that patients are involved early in the process and that their perspectives are incorporated into guidance and decisions. By listening to patient voices and conducting appropriate consultations, the agency aims to provide better overall outcomes for patients.

## Why is it important to get this right?

The consequences of not adequately incorporating patient views, where appropriate, in assessments could include:

- **Patient outcomes are ignored** – the assessment does not take account of what matters most to patients.
- **Reputational damage** if a decision is viewed unfavourably by the affected population.
- Certain populations are **disadvantaged** – decisions fail to consider the consequences for certain populations.
- Patients may bring **new perspectives** that support innovation and assist problem solving.

## Session 2: Patient input into regulatory and HTA agencies decision processes – What are current practices and how is the landscape changing?

### Patient engagement and patient experience data in regulatory review and HTA: Where are we today?

**Hayley Chapman,** Executive Director - Operations, Patient Focused Medicines Development (PFMD), The Synergist

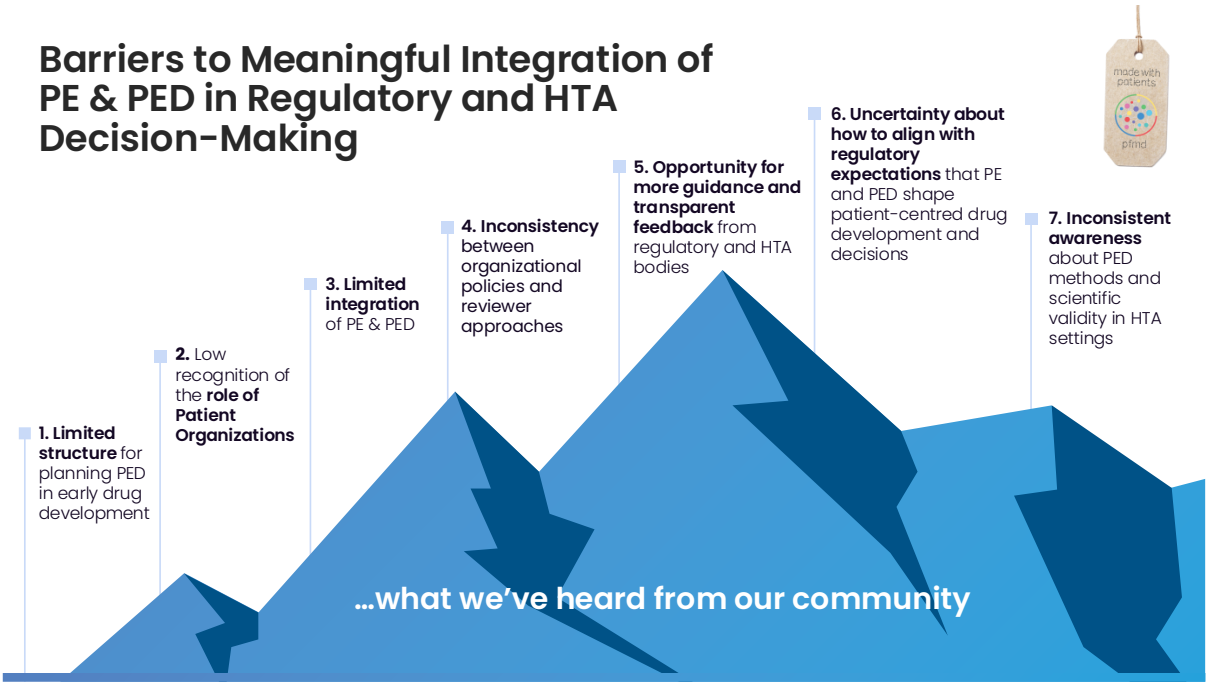
**Global trends in PE and PED use**

Regulators and HTA agencies globally are placing greater emphasis on patient engagement (PE) and patient experience data (PED) and strengthening their use in decision making. This trend is reinforced by the World Health Organization resolution on the importance of social participation. Beyond regulatory and HTA bodies, the investment community is now paying attention to PE and PED to help de-risk their decisions. Patient organisations have a key role in centralising PED, moving beyond anecdotal intelligence to demonstrating the real impact of disease, building credibility and driving policy change.

A recent landscape study coordinated by PFMD revealed that 75% of resources containing information on PE and PED published by regulators and HTA agencies worldwide focused on PE, while 46% focused on PED. Notably, nearly 30% of resources combined both PE and PED, which represents significant progress from a previous study when there were no references to this combination.

**Challenges in PE and PED**

Despite progress in the PE/PED field, challenges remain (see below), including limited integration of PE and PED, lack of guidance and transparent feedback from regulatory/HTA agencies, and uncertainty on how to align with regulatory expectations.



**PFMD PE & PED Project**

Through the PFMD PE & PED Project, tools and resources are being co-created to address challenges in a pre-competitive, disease-agnostic space. Ongoing initiatives include:

- A PE and PED integrated Navigator to align PE activities with the design, generation, analysis, and use of PED.
- An HTA-specific PED Navigator to highlight most meaningful areas of impact for the patient community.
- A framework to structure early inclusion of PED in regulatory and HTA discussions, aligning expectations and increasing communication.

**Conclusion**

PE and PED are strategic, not symbolic, to all stakeholders – they inform strategic planning and resource allocation. Despite growing focus and strengthened use of PE and PED, challenges persist. PFMD works collaboratively with companies, the patient community, regulators, HTA bodies, and other stakeholders to address these challenges and improve the integration of the patient voice in evidence-based decision-making processes. Integrating PE into the design, interpretation and use of PED programmes would help to maximise the value of patient input in regulatory and HTA decisions, with the ultimate aim to improve patient health outcomes.

## Agency case studies - How are agencies engaging patients directly/indirectly during the review and assessment process?

## Use of patient submission summaries – Patient input through a formal written submission during HTA assessment

**Prof Andrew Mitchell,** Honorary Professor, The Australian National University, and Member of the Evaluation Sub-Committee of the Medical Services Advisory Committee (MSAC), Australia

**Public engagement in Australia**

The Australian healthcare system uses the term "consumer" to refer to people with lived experience of a health issue, including patients, families, carers, friends, and members of the general public. The HTA system is designed for public engagement, which includes consumers, healthcare providers, competitors to the applicant, and other organisations.

The purpose of public engagement is to ensure more holistic deliberations by considering diverse perspectives and provide procedural fairness by ensuring all affected stakeholders have an effective voice in deliberations. It helps HTA committees to better understand the benefits and disadvantages of the proposed health technology and how it may affect the lives of people with the condition, relevant clinical practice and relevant parts of the healthcare system.

**Input and output mechanisms**

Written submissions via the Office of Health Technology Assessment Consultation Hub are the primary method of public input on an HTA application in Australia. However, there are other mechanisms for public engagement:

- Option to provide audio or visual input (under 10 minutes)
- Consumer expert members on all HTA committees and subcommittees
- Stakeholder forums including patient and public input
- Patient support group hearings - Pharmaceutical Benefits Advisory Committee (PBAC) only
- Targeted approaches to organisations and individuals - Medical Services Advisory Committee (MSAC) only.

Outputs to the public includes publishing agendas of applications to upcoming HTA committee meetings (to trigger public input) and public summary documents (PSDs). Information that influenced decision making is presented in PSDs under specific subheadings. The PSDs for MSAC have a plain language summary to help explain the HTA committee outcomes to the public.

**Public engagement processes**

PBAC and MSAC have different processes for public engagement:

- PBAC publishes agendas as soon as possible after receiving applications, allowing a limited period for public input that runs parallel to the assessment process.
- MSAC has a prior process called PICO (Population, Intervention, Comparator, Outcomes) setting, where public input is sought on how to define the question for public funding.

In both cases, public input is provided to the applicant before the meeting to ensure procedural fairness. Public engagement work is supported by the HTA Consumer Evidence and Engagement Unit.

**Management of public input**

Personal and third-party information is redacted by the secretariat to comply with privacy principles. Input from groups or organisations is provided in full to HTA committees and applicants after redactions, while input from individuals, forums, or hearings is provided as a collated summary. Public input may also be provided to codependent HTA committees, assessment groups and government officials implementing HTA outcomes.

**Conclusion**

The Australian approach to public engagement in HTA is dynamic and continuously evolving. The system aims to balance the need for comprehensive input with the requirement for timely deliberations. By providing multiple channels for engagement and ensuring transparency in how public input influences decisions, Australia has developed a fit-for-purpose solution that supports meaningful participation while maintaining efficient assessment processes.

## Public engagement by Australian HTA

- Public = ***patients***, ***patient groups***, other consumers, carers, healthcare providers, competitors, organisations
- Main input from the public on an HTA application is ***in writing*** via the Office of Health Technology Assessment - Citizen Space Consultation Hub, ***and also***
  - *option* of an audio or video format of ≤10 minutes' duration via email or post
  - by consumer expert members of the HTA committee and its sub-committees in meetings
  - at a stakeholder forum *if initiated* by the HTA committee independent of an application
  - in a patient support group hearing *if initiated* by the HTA committee (PBAC)
  - in response to a targeted approach *if initiated* by the HTA committee secretariat (MSAC)
- Main output to the public on HTA applications
  - published agendas of applications to upcoming HTA committee meetings to trigger public input in response
  - Public Summary Documents to explain HTA committee outcomes
- All supported by the Consumer Evidence and Engagement Unit of AG DHDA
- Applies to all HTA applications

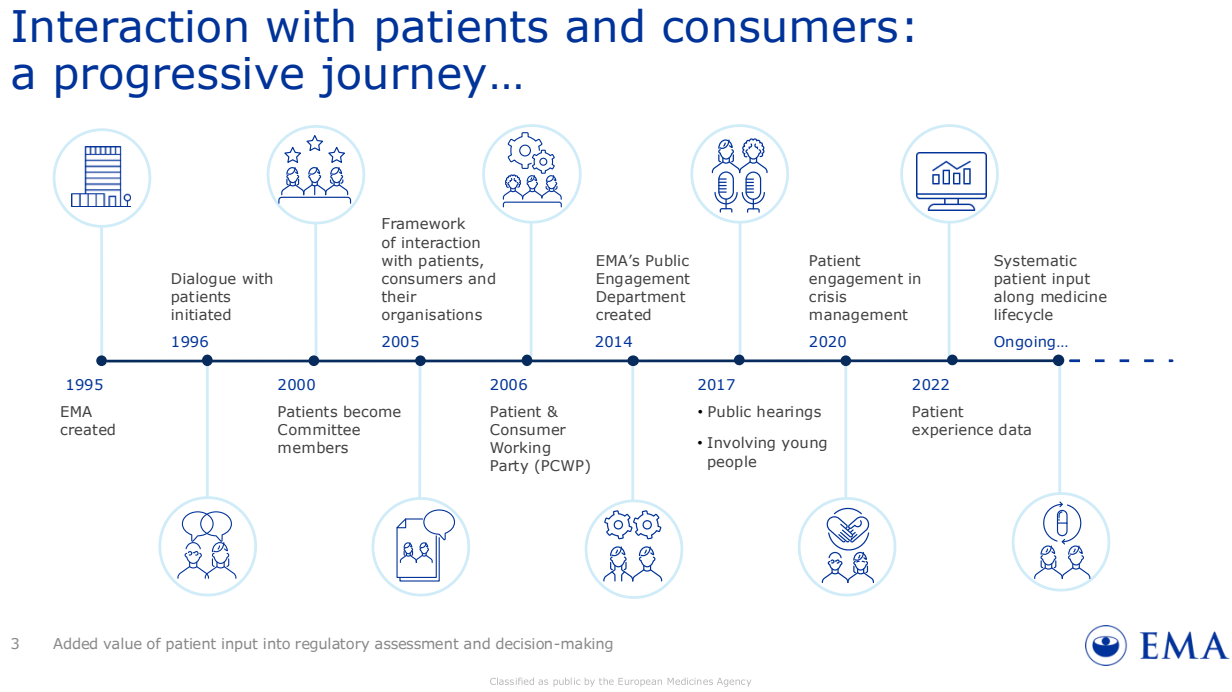
3

## Added value of patient input in regulatory assessment and decision making

**Juan García Burgos,** Head of Public and Stakeholders Engagement Department, European Medicines Agency (EMA)

**Evolution of patient engagement at EMA**

EMA has incorporated patient engagement as an integral part of its work since its establishment in 1995. Over the past 30 years, there has been a significant evolution in how patients are involved and the value they contribute (see below). Today, there is no longer any questioning or reluctance to involve patients, and their value is not contested. The main limitation is resources, both for the agency and for patients who have limited capacity to contribute to the many activities.




**Framework of engagement**

EMA's interaction with patients is organised at the EU level through patient and consumer organisations. Central to this network is the Patients' and Consumers' Working Party, which serves as a forum for discussion with patients and their representatives and monitors the implementation of engagement and patient participation in the agency.

To overcome limitations of reaching only organisations, EMA also interacts with individuals who may not be affiliated with an organisation. This duality provides the right balance and a more efficient system. EMA has developed various methodologies to facilitate interaction, ranging from sophisticated instruments like public hearings to simple interventions such as written consultations or telephone calls.

**Levels of patient participation**

Patients participate in EMA activities at three levels:

- Representing patients in general (e.g., as members of the management board with voting capacity)

- Representing their organisations (e.g., in the Patients' and Consumers' Working Party)
- Representing themselves as individuals, providing expertise and experience on various activities including scientific advice and scientific advisory / ad hoc expert groups.

Analysis of patient participation in EMA committees such as the Committee for Orphan Medicinal Products and the Paediatric Committee shows that patients have performed at the same level as other members, serving as rapporteurs and holding positions such as Vice-Chair. However, their contribution is different in nature and not necessarily scientific. They provide unique value through their perspective and experience of the disease, insights that other members cannot provide.

**Patient involvement in scientific advice**

Patients are involved throughout the product lifecycle, from pre-submission to post-authorisation. An example of this is scientific advice, where their input is particularly valuable. Patients may contribute to the selection of population, selection of endpoints, quality of life aspects, feasibility of studies, and standard of care and comparator choice. Analysis shows that patient input in EMA scientific advice resulted in further reflection by the Scientific Advice Working Party in 52% of cases and led to modifications in development plans in 20% of cases (Murphy et al. 2022). Importantly, for the vast majority of cases where patient input did not change the final advice, their agreement with the proposed development plan was considered equally valuable.

**Methods of incorporating patient views**

Although the Committee for Medicinal Products for Human Use (CHMP) does not yet have patient members, EMA has developed methods to incorporate patient views, such as early dialogue with relevant patient organisations, including patients as experts in benefit-risk discussions, and stakeholder meetings.

Public hearings have been held by the Pharmacovigilance Risk Assessment Committee (PRAC)

Public hearings have proven instrumental in safety reviews, as demonstrated in cases such as valproate (an anti-epileptic with risks during pregnancy) and quinolones/fluoroquinolones (antibiotics with rare but severe side effects). These hearings provided valuable insights that would have been missed in a normal process.

**Conclusion**

EMA is fully committed to patient engagement as an integral part of the regulatory system. The focus now is on how to implement it most efficiently, also in the context of EU Joint Scientific Consultation and Joint Clinical Assessment. Patient experience data is seen as an important complement to patient engagement, providing comprehensive information about patients' views on medicines. Patient involvement not only helps to bridge the gap between clinical trial data and real-world data, but also builds trust, as society and patients are more likely to trust decisions made by regulators when patients are involved.

## Giving patients and clinicians a stronger voice in decision making: The Scottish Medicines Consortium (SMC) Patient and Clinician Engagement (PACE) process

**Pauline McGuire,** Principal Pharmacist, Scottish Medicines Consortium (SMC)

**Background**

The Scottish Medicines Consortium (SMC) provides advice to NHS Scotland about the value of newly licensed medicines. SMC conducts HTA of new medicines through a two-stage review process; first there is a clinical and economic assessment by the New Drugs Committee with clinical expert input. If the case is considered reasonable at this stage, then the New Drugs Committee recommends that SMC accepts the medicine for use. However, if the case is not considered reasonable, there is a further assessment and a final decision made by the SMC Committee, which takes a broader view including patient and wider public input.

For all medicine assessments, clinical experts and patient groups are invited to submit written input. In addition, patient group partners attend SMC Committee meetings, which are held almost entirely in public. SMC's patient and public involvement activities are supported by a dedicated Public Involvement Team.

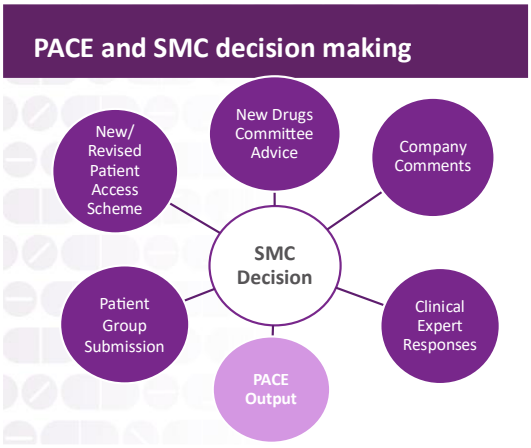
**Development of the PACE process**

In 2013, there was an increasing number of medicines for advanced cancer and rare conditions that were not recommended by SMC, mainly due to high estimated cost-benefit ratios. A Scottish Parliament Health and Sport Committee inquiry identified a strong view from patients and clinicians that existing cost-effectiveness thresholds were not always appropriate for end-of-life medicines or medicines to treat rare diseases. SMC was directed to apply a different approach to evaluation of these medicines to increase access.

**The PACE process**

The Patient and Clinician Engagement (PACE) process was implemented in 2014 specifically for rare and end-of-life conditions. These are defined as orphan-designated medicines or those treating an equivalent-sized population, and conditions that would lead to death within three years (usually advanced cancer). Approximately half of the submissions to SMC are eligible for a PACE meeting if not recommended at the New Drugs Committee stage.

The PACE process involves a one-hour virtual meeting that brings together patients, carers and clinicians to discuss the added value of the medicine. They describe clinical issues, severity of the condition, unmet need, impact on quality of life, ability to continue work or education, symptoms, treatment trade-offs, convenience, and independence. The meetings often reveal information about the impact on family and carers that wouldn't normally be captured.




The output of a PACE meeting is a statement summarising the discussion, which is included in the SMC Committee meeting papers alongside other documentation (see figure). The PACE output is a major factor in the SMC Committee decision. Feedback indicates that patients highly value the opportunity to participate in PACE meetings, finding them a comfortable forum to share their views.

**Case study: Belzutifan**

Belzutifan is an oral medication for von Hippel-Lindau (VHL) disease, a rare life-limiting disorder that causes the formation of tumours and cysts in different parts of the body. The New Drugs Committee noted promising response rates to belzutifan treatment but considered the economic case not sufficiently robust. A PACE meeting was convened, where participants shared that VHL is a devastating disorder causing debilitating symptoms that greatly impact daily living and the mental health of patients and families. As a hereditary condition, it can affect many family members, increasing the impact.

The PACE meeting highlighted that there were no licensed treatments available, with surgery being the primary option, resulting in numerous surgeries throughout a patient's life. Belzutifan was presented as a first-in-class medicine that could control VHL disease and potentially reduce the need for risky and invasive surgeries. After considering all evidence, including the PACE output, SMC accepted belzutifan for use by NHS Scotland.

**Conclusion**

The PACE process was introduced to increase access in Scotland to medicines specifically for rare and end-of-life conditions. It provides an opportunity for the SMC Committee to hear insights not fully captured in conventional assessment and keeps the focus on added value from patient, carer, and clinician perspectives. The process has been well-received by stakeholders and demonstrates the value of structured patient and clinician engagement in decision making.

## Patient involvement in the Dutch national HTA process

**Prof Wim Goettsch,** Special Advisor HTA, Zorginstituut Nederland (ZIN), and Professor of HTA of Pharmaceuticals, Utrecht University, The Netherlands

**Background**

Zorginstituut Nederland (ZIN) is the Dutch HTA body, an independent governmental organisation that functions both as a referee and an advisor. It is clearly defined within Dutch healthcare legislation and part of its work involves the healthcare coverage programme, which clarifies which treatments are sufficiently effective to include in the basic healthcare package.

**Patient involvement in the HTA process**

The ZIN HTA process has four main stages: selection, assessment, appraisal and the policy decision. Patients are involved throughout the HTA process in various ways (see below).

During selection and horizon scanning, ZIN holds roundtables on topics such as orphan diseases and oncology, discussing upcoming treatments with clinicians and patients two to three years before market authorisation is expected. In the assessment phase, patients are involved in scoping (defining the PICO - Population, Intervention, Comparator, Outcomes) and the consultation process. One member of the appraisal committee is a representative from a patient organisation.

For pharmaceuticals, patient involvement is highly institutionalised because the process has been established for many years. For other technologies like medical devices, patient involvement may be more ad hoc, depending on the dossier and timing.

### Who and how are patients involved?

Netherlands Patients Federation

nfk Nederlandse Federatie van Kankerpatiënten organisaties

WHO
- Patient representatives
- Patient experts

HOW
- Experience (e.g. scoping, appraisal)
- Input (e.g. scoping, consultation)
- Informing (e.g. consultation, policy decision)
- Committee member

Utrecht University Pharmacoepidemiology and Clinical Pharmacology

**Experiences with patient engagement**

ZIN's experiences with patient engagement reveal several insights:

- Experiences can vary between patient organisations and disease areas:
  - For larger disease areas, it is often easier to find patient experts.
  - Some patient organisations have well-developed expertise in HTA and health economics, while others (particularly for rare diseases) do not.
- Patient feedback indicates that they value having input on relevant outcome measures in both the scoping/assessment and appraisal phases.
- Patients sometimes find the process formal and opaque ("a black box").
- Short timeframes for consultation responses (sometimes just five days) can be challenging.
- Patients want more clarity on how their comments are incorporated into assessments, appraisals, and final recommendations.

**Conclusion**

Patient involvement is highly institutionalised in the different phases of the Dutch HTA process but remains a work in progress. Feedback from patients on their involvement has highlighted challenges, such as short timeframes for consultation responses, and lack of clarity in how their comments are incorporated into assessments, appraisals, and final recommendations. ZIN is looking at the EU HTA Regulation as an opportunity to identify aspects where patient involvement can be enhanced at the national level.

## Company case study: An example of meaningful patient involvement for regulatory decision making

**Robyn von Maltzahn,** Global Head, Patience Centered Outcomes, GlaxoSmithKline (GSK), UK

**Background**

Primary biliary cholangitis (PBC) is a chronic, progressive, autoimmune, rare liver disease that predominantly affects women. It is characterised by gradual destruction of the intrahepatic bile ducts, leading to accumulation of bile acids in the liver and systemic circulation. This increase in systemic bile acids is believed to contribute to cholestatic pruritus (itch), which is highly common in PBC patients. This internal itch can occur anywhere in the body, is unlikely to be relieved by scratching, and has been described as debilitating, affecting many aspects of quality of life.

**Integrating patient input into development**

Patients with PBC often describe the itch as one of the most debilitating and life-limiting symptoms, impacting sleep, concentration, and mental health daily. Being aware of this through patient interviews and discussions, GSK embedded the patient voice early in outcome selection, trial design, and evidence generation. Patient-reported outcomes (PROs) played a prominent role in assessing itch, sleep, and associated health-related quality of life in the clinical programme.

**PRO measures**

GSK's endpoint strategy required both creating a measure to assess itch and adjusting and validating an existing health-related quality of life measure. Early and frequent engagement with regulators in developing a fit-for-purpose PRO was key to endpoint selection and development.

Itch, like pain, nausea, or fatigue, is a symptom that can only be reported by patients themselves. Thus, PRO measures were collected via eDiaries and were essential endpoints throughout all phases of development. Due to the prominent and unprecedented use of PROs in this indication, significant input from patients was needed for endpoint selection, trial design, and communications. Qualitative and quantitative work was conducted through interviews and psychometric validation studies to support the validation of a fit-for-purpose PRO, which was key from a regulatory perspective.

**Use of patient experience data**

In addition to collecting symptom and quality of life data in every trial, additional efforts were made to collect patient experience data (PED) to understand the true burden of the disease and the unmet medical need associated with cholestatic pruritus. These efforts included qualitative interviews with patients both in and outside the trial setting, and patient feedback on the phase 3 protocol and trial design to understand the practical realities from a participant perspective.

PED was extensively used in regulatory submissions, with the intention of including a statement of reduction of itch in the labelling claim, as well as potential label language around reduction of sleep interference. A separate PRO dossier was included to support the validity and reliability of the PROs used.

Global regulatory submissions have been made to the US, EU, UK, and Canada, with additional countries to follow. PED, in the form of PRO data, is hoped to obtain labels around improvement in itch, reduction in sleep, and

maintenance of itch improvement. It has also been included in the orphan drug designation maintenance report (see below).

## Patient data in regulatory submission

**m.2.5 and m.2.7.3 (efficacy)**

- Quotes to highlight the serious, debilitating nature of cholestatic itch and its burden
- Patient-reported data underpinned all key efficacy endpoints

**m.2.7.4 (safety/tolerability)**

- Patient-reported tolerability data to better understand patient experience GI -related side effects, including diarrhoea

**Orphan Drug Maintenance Report (EU):**

- Patients interviewed regarding their experience with product and other previously-tried therapies for itch with a deep dive into patient preferences – this qualitative data served as one line of evidence (among others) to support maintenance of orphan drug designation in EU

6 01 December 2025 GSK

**Conclusion**

While it is too early to give a final verdict on the impact of PED in the regulatory submissions, this programme demonstrates how embedding the patient voice early in outcome selection, trial design, and evidence generation can drive clinical relevance, regulatory credibility, and meaningful patient impact. Patient input has been central to the development process, including understanding the condition, selecting and developing endpoints, and ensuring a sustainable trial programme. The programme underscores the need for transparency around how and when PED, including PROs, are factored into regulatory decisions. This transparency, paired with closer collaboration between regulators, sponsors and patients, will be essential to embedding the patient voice as a reliable driver of regulatory evaluation and ensuring therapies continue to deliver outcomes that matter most to patients.

## Company case study: Reflections and considerations from an HTA submission in alopecia areata

**Dr Alice Biggane,** Associate Director, Outcomes Innovation and Research, Access and Value, Pfizer, UK

**Background**

Alopecia areata is not just about losing hair; it involves a significant psychosocial burden for patients. Historically, the healthcare field has sometimes been dismissive or unaware of the impact, and until recently, treatment options were often limited. The heterogeneity between patients in baseline characteristics increases measurement challenges, particularly for health-related quality of life (HRQoL) and patient experience data (PED). Hair loss is associated with HRQoL, but the impact is not always linear, so capturing HRQoL is often challenging in this population.

**Quantifying HRQoL for NICE**

The National Institute for Health and Care Excellence (NICE) uses cost-effectiveness analyses to understand the value of new treatments, which typically include the impact the treatment has on HRQoL. This is often assessed through preference-weighted data known as utility values. NICE advocates for the use of EQ-5D, a generic preference-based measure. For the alopecia areata submission, utility estimates were recognised as a key driver of the economic model and 'value for money' assessment. Other PED types also played an important role in providing context and insights in the submission.

**Addressing EQ-5D limitations**

Early on, it was recognised that there would be challenges in showing the full impact of HRQoL based on the EQ-5D alone, with the assumption that trial-based utilities would not capture the full impact. NICE provides guidance on what to do when the EQ-5D is not available or suitable through a hierarchy of preferred methods, which served as a handrail for Pfizer's alternative approach.

**Development of an alternative approach**

Conceptual models were developed through literature reviews and interviews with patient advocacy groups, patients, and clinicians. These models identified the interplay of quality of life impacts across different domains and were used to determine whether measures like the EQ-5D accurately captured the affected areas.

A vignette/time trade-off study was developed to generate alternative utility data. Health state scenarios (vignettes) were created through interviews with patients, carers, and clinicians, aligning with the economic model. The vignettes were evaluated by a representative sample of the UK general population using a time trade-off method, where, for each vignette, participants indicated how much of their lifespan they would be willing to trade for perfect health. This resulted in bespoke values for the vignette-based health states that differed significantly from the trial data.

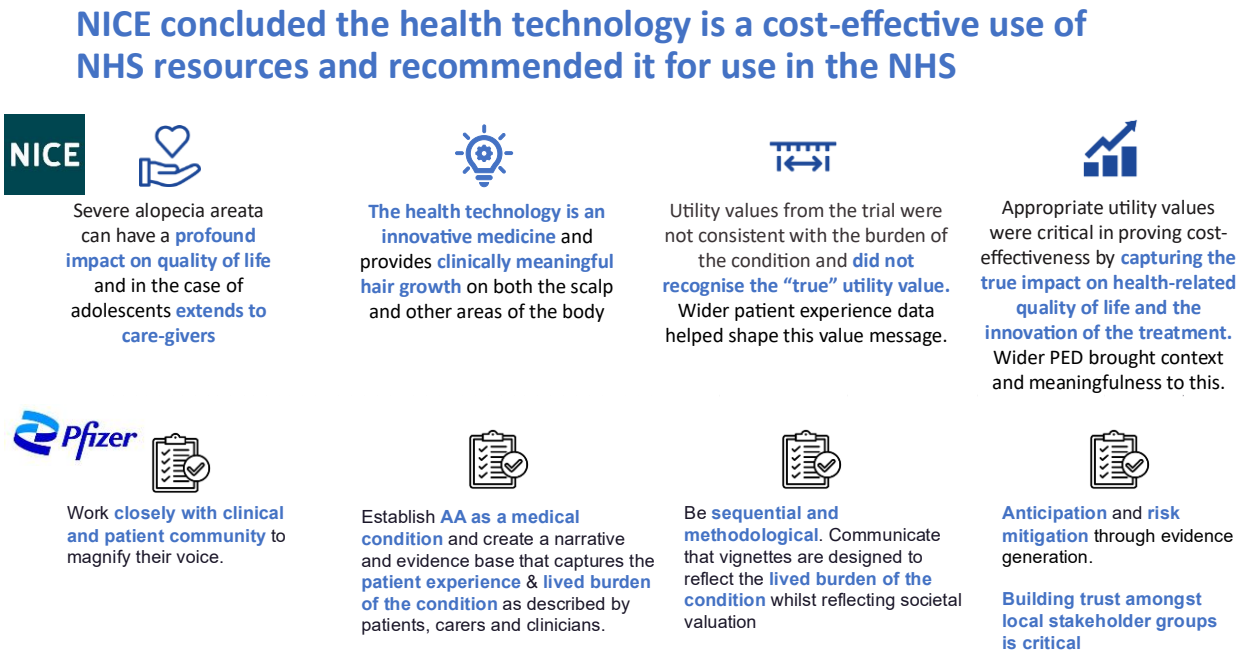
**Validating the alternative approach**

To demonstrate that the vignette utility estimates were better than those from trials, several approaches were used to support the submission:

- Reinforced the validity of the vignette's utility estimates
  - Referred back to qualitative evidence including literature, interview studies and disease-specific patient-reported outcome measures.
  - Spoke with patient advocacy groups and clinicians to understand and reinforce their lived experience.
- Followed the hierarchy of evidence as outlined by NICE
  - Repeated the vignette/time trade-off study in a patient population with alopecia areata (with similar results).
  - Conducted a conceptual overlap analysis of alopecia areata with a proxy condition with similar HQoL burden (atopic dermatitis).
  - Conducted psychometric analysis of the trial EQ-5D data, which confirmed high ceiling effects and showed that the EQ-5D lacked sensitivity, responsiveness, and patient-relevant domains.

**Conclusion**

The alternative approach to quantifying HRQoL was successful, with NICE concluding that the health technology was cost-effective and recommending it for use. Key lessons learned included working closely with clinical and patient communities to magnify their voice and understand their story, generating multiple streams of evidence to establish alopecia areata as a medical rather than cosmetic condition, and capturing the lived burden of patients. By being sequential and methodological in the approach and anticipating and mitigating risks early through evidence generation, the submission was successful while building trust with stakeholders like patient advocacy groups and healthcare professionals.



# Session 3: Visibility of patient input in regulatory and HTA decision making - How should the impact be measured, what should be documented, and why?

## Visibility of patient input - What is needed and why?

**Mencia de Lemus,** Co-Chair Treatment Committee, SMA Europe, Spain

**Why does visibility matter?**

Visibility of patient inclusion in decision-making processes strengthens legitimacy, builds trust, reinforces credibility of institutions, and increases meaningful input for future processes. However, patient organisations struggle to find individuals willing to participate in these processes, partly because patients often don't understand the value they bring to regulatory discussions.

**The value of patient input**

Patients are the real experts when discussing the burden of living with a disease and how that weighs against the burden of receiving treatment. In environments with high uncertainty, such as in rare diseases or advanced therapy medicinal products (ATMPs), patient input can have significant weight. If patients and patient organisations were more aware of their potential positive influence, more effort may be made to ensure meaningful input into regulatory and HTA processes.

**Legitimacy of patient input**

While patients have a legitimate interest in inputting into decisions that affect their lives, not all patient input is usable in regulatory and HTA processes. Not every patient can contribute effectively due to issues of capacity and education. Representatives must be able to speak for their broader community rather than just their personal experience.

Conflict of interest policies can be problematic, particularly in rare disease contexts where patient communities are small. Patient organisations often operate with scarce resources, making it difficult to train and educate representatives adequately.

**Trust issues**

Trust from decision makers towards patient input remains an issue. Regulatory and HTA agencies have significant responsibilities and typically rely on solid scientific evidence. Taking them out of this comfort zone to consider patient experience data requires systematic approaches to build confidence. Several approaches could improve the situation:

- Raising assessors' awareness by exposing them more to patients and patient data
- Systematising patient input into regulatory processes and dossiers
- Creating specific roles for channelling patient input within committees
- Increasing resources to ensure patients are highly trained
- Reinforcing the channelling of information to the right people at the right time.

Trust from patients about the impact of patient involvement in regulatory and HTA processes is also an issue. Patients need to understand the rationale for their input and the process and phase in which they're inputting. To support transparency and trust, feedback should be provided to patients about how their input was used.

**Supporting transparency**

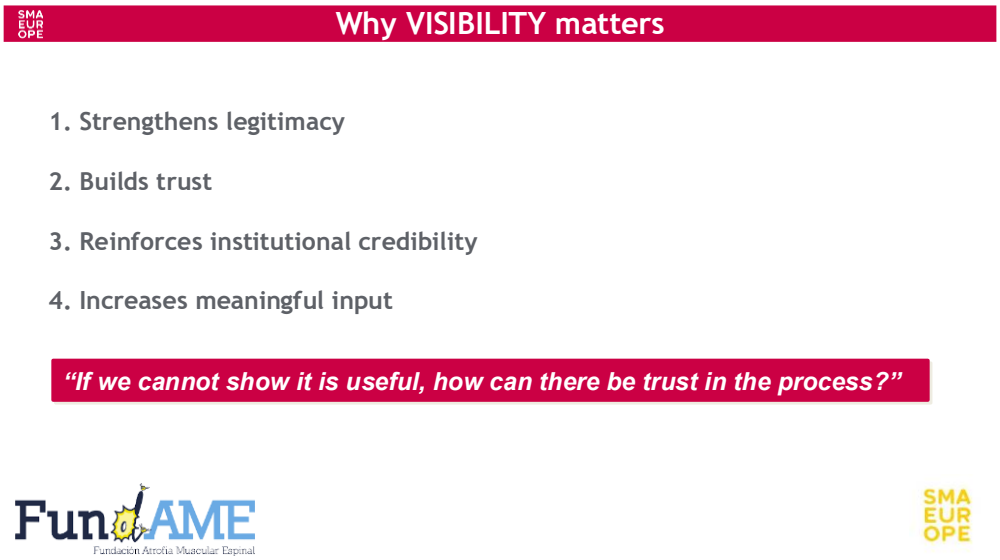
Regulators and HTA agencies can increase legitimacy by showing how patient perspectives have been considered or integrated. Tools that could improve transparency include sharing meeting minutes, providing rapporteur feedback to patients, having liaison persons who communicate with patient organisations, and officially communicating the value of patient input obtained, both publicly and directly to patients.

**Measuring impact**

Measuring the impact of patient involvement is challenging but important. Examples of quantitative measures include number of procedures with patient input, number of submissions received with patient engagement or patient experience data, type of patient input received, and number of decisions or reports in which patient input is explicitly quoted. Narrative examples of impactful interactions could be qualitative measures.

**Conclusion**

Visibility of patient input matters because it strengthens the legitimacy of using patients as a source, builds trust in the process, reinforces institutional credibility and increases participation and meaningful input for future. Transparency on the use of patient input in regulatory and HTA decision making could be enhanced through several means, including direct feedback to patients, sharing meeting minutes and introducing patient organisation liaison roles. Measuring the impact of patient input in decision making is challenging but important, requiring both quantitative and qualitative approaches.



## CIRS agency survey on the impact, measures, challenges and future direction for patient engagement and patient experience data in regulatory and HTA decisions

**Dr Neil McAuslane,** Scientific Director, CIRS

**Background**

CIRS conducted a survey of regulatory and HTA agencies to better understand their perspectives and practices for patient engagement (PE) and patient experience data (PED). The survey focused on agency-level activities related to PE and PED, metrics for measuring impact, communication/documentation, current challenges, and future thinking in this area.

The survey was completed by 13 regulatory agencies and 18 HTA agencies across Latin America, North America, Asia, and Europe. The survey was structured in three parts: current PE/PED landscape and activities (which all agencies could answer), experiences of using PE or PED during assessment or decision processes, and future thinking.

**PE/PED landscape and activities**

For both regulatory and HTA agencies, patient advocates are actively encouraged to comment on agency guidance and participate in patient committees or meetings with patient groups. Most HTA agencies gather patient input through formal written submissions during the assessment, and some accept submissions with PED directly from patients. Training for patient advocates appears to be more common in HTA agencies than in regulatory agencies.

The survey results implied that HTA agencies have broader engagement in PE/PED activities than regulatory agencies. HTA agencies also seemed more likely to have a formal approach (i.e. have specific guidelines, processes and procedures) to PE/PED, suggesting a stronger integration of patient input in HTA processes.

When asked about the general PE landscape with their jurisdiction, both regulatory and HTA agencies indicated that PE processes are in place and being used by patient advocates. Patient advocates and organisations tended to be active and well organised, regardless of disease area.

**Impact measurement**

When asked how often the patient voice had a major impact on final decisions (i.e. altered outcomes), most agencies (both regulatory and HTA) responded with "not at all", "very few assessments," or “Don’t know”. Only two HTA agencies reported impact in at least half of assessments.

The most common impact metric being measured by agencies is the availability of medicines to patients, though this metric doesn't necessarily correlate with whether PE/PED was utilised in the assessment. Fewer agencies measure patient satisfaction with new medicines, the degree to which PE helped in reviews or assessments, or the quality of patient evidence. About a third of the agencies did not have any impact metrics for PE/PED.

**Communication and feedback**

The main communication channel for publicly communicating PE/PED activities is through public assessment reports. Only about 30% of agencies provide direct feedback to patients on the use of their input/evidence. Feedback mechanisms vary, including holding public deliberations, providing impact letters to patients, or informing patients, carers and patient organisations by email how their inputs were used by decision makers.

**Challenges**

The top challenges facing agencies undertaking PE or receiving patient evidence are concerns about biases, representativeness, and the quality and rigour of evidence. Ten themes of challenges were identified: governance and legal gaps; capacity and resources; methodology and evidence; representation and equity; awareness and culture; ethics and trust; data structure and quality; regulatory/HTA integration; timing and strategy; and technology adoption. While generally consistent across both sectors, regulatory agencies tended to focus more on structural and institutional barriers such as legal mandates and governance, while HTA agencies highlighted more practical and operational challenges.

**Future outlook**

Despite challenges, most agencies believe that PE and PED will have greater influence on assessments and appraisals over the next five years. When asked what needs to be in place to support global evolution of PE and PED, most agencies highlighted the need for guidelines to ensure quality, rigour, and representativeness of data, along with infrastructure and resources to support patient groups.

**Conclusion**

The CIRS survey suggested that HTA agencies engage more broadly in PE/PED activities and are more likely to have a formal approach to PE/PED than regulatory agencies, implying a stronger integration of patient input in HTA processes. Most agencies (both regulatory and HTA) do not have impact measures or direct feedback to patients on the use of PE/PED in their deliberations and decisions. While agencies are challenged by concerns about biases and representativeness, they believe that patient data will have greater influence on their work over the next five years. Guidelines on ensuring quality, rigour, and representativeness of data, along with infrastructure and resources to support patient groups, are needed going forward.

## Summary – for the cohort of HTA and Regulatory agencies participating in this survey

General HTA – Regulatory PE/PED Landscape

- **HTA agencies show broader patient engagement** than regulatory agencies across most activities, indicating a stronger integration of patient input in HTA processes
- **HTA agencies more likely to have a formal approach** for undertaking PE/PED in the assessment/appraisal process of new medicines **however Majority of agencies do not measure or have impact measures** on how often did the patient voice (either through PE or PED) have a major impact on the final decision

Current measures and communication and challenges

- **Majority of agencies do not have direct feedback to patients** on the use of PE or PED evidence or other input provided by patients on the deliberations and final decision – **main communication channel is through the public assessment report.**
- **Concern about biases and representativeness** of the patient was the **top two challenges** in undertaking PE or receiving patient evidence?

Future – looking forward

- **Over next five years** - For the majority of agencies - **PED/PE Will have a greater/stronger influence then it currently has on the assessment/appraisal**
- What agencies believe needs to be in place to support the global evolution of PE and PED
  - **Guidelines on how to ensure quality, rigour and representativeness of PE and PED**
  - **Infrastructure/resources to support patient groups and patients with PE activities and collecting robust PED**

## How are patient engagement and patient experience data used in the regulatory evaluation and decision-making process?

**Juan García Burgos,** Head of Public and Stakeholders Engagement Department, European Medicines Agency

**Definition of patient experience data**

The European Medicines Agency (EMA) has released for public consultation a reflection paper providing the view of EU regulators on how patient experience data (PED) should be collected and used in the assessment and regulation of medicines. A key aspect of this document is establishing a clear definition of patient experience data (PED), as there has been inconsistency in understanding this concept.

The proposed definition describes PED as any kind of data reflecting patient experience without input or interpretation by third parties, including health status, functional status, symptoms, disease scores, treatment preferences, quality of life, and side effects. This data can be generated, collected, or submitted by different stakeholders, including pharmaceutical companies, and can be either quantitative (such as patient-reported outcomes or patient preference studies) or qualitative (including information from patient engagement activities).

**Progress and challenges**

For many years, the EU regulatory network has prioritised ensuring that evidence generated for new medicines reflects patients' views. However, progress has been slow due to challenges including the lack of reliable and validated methodologies to collect PED. There is a need to encourage more developers and companies to generate and submit this data.

The initiative to address these challenges began in 2022 with a multi-stakeholder workshop, which identified a gap in EU regulatory guidance. Industry representatives expressed reluctance to invest in generating PED without clear regulatory expectations. In response, an expert group on PED was created in 2023, with significant interest from experts across different committees and working parties.

**PED reflection paper**

The reflection paper sets out the EMA's approach to PED, stressing the importance of systematic consideration of PED by all stakeholders and encouraging developers to generate it. It proposes a framework for clarification rather than specific methodological guidance, acknowledging that regulatory experience in this area is limited and that each medicine's development plan is unique.

The paper describes the value of PED throughout the medicine lifecycle—in preclinical, clinical, and post-authorisation phases. It distinguishes different types of PED (patient-reported outcomes (PROs), patient-preference studies, and information from patient engagement) and sources (clinical trials, real-world evidence, and potentially social media).

**Scientific advice and method qualification**

A key message in the reflection paper is the encouragement for companies to seek scientific advice early in the development process. The EMA would like consideration of PED to be systematic in every scientific advice procedure, even if not every application requires such data. The qualification of novel methodologies, such as new PROs, is another avenue for support.

**Transparency of PED**

To address transparency concerns, the EMA has updated its assessment report template to include a specific section on PED. This will systematically record what kind of PED has been submitted, how it has been evaluated, and what value it has for the application, including any limitations in quality.

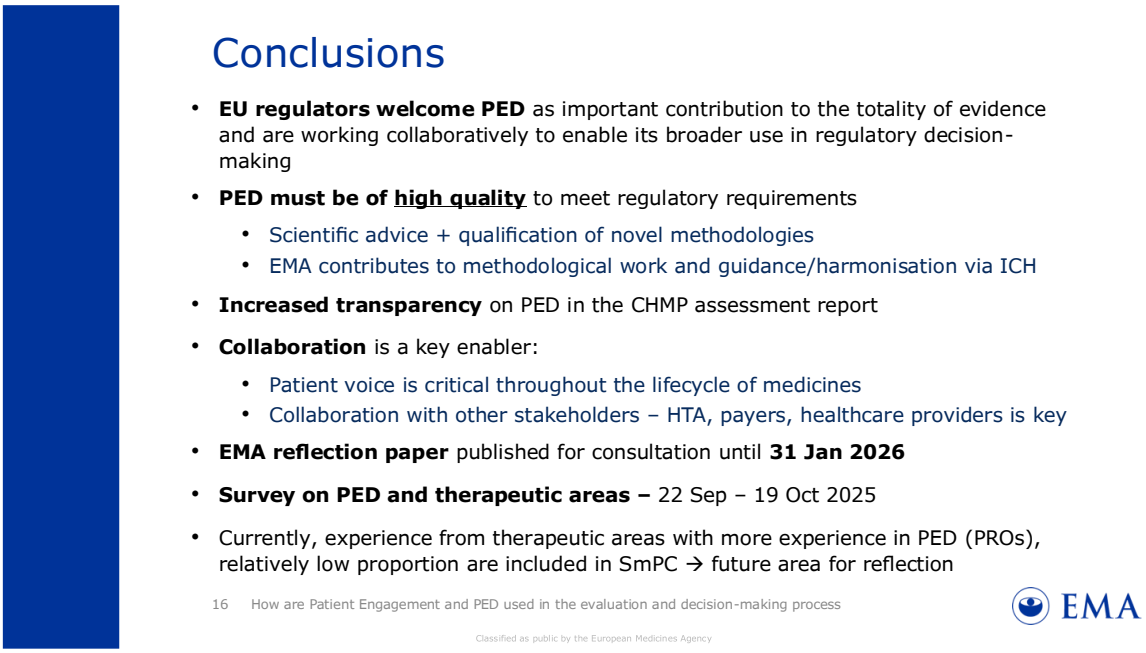
Further consideration is needed on how PED might be reflected in product information. A study of PROs in oncology indications granted a marketing authorisation between 2017 and 2020 found that 78.1% included PRO data in confirmatory trials, with 17% of cases resulting in inclusion in the product labelling. A significant challenge is that submitted PED often lacks quality, making it difficult to use effectively in decision making. Early discussion at the scientific advice stage is crucial to ensure data quality.

**Survey on PED**

The EMA is conducting a survey on how different stakeholders value and use PED across therapeutic areas, seeking input from patients, healthcare professionals, regulators, and industry separately. This will help to identify potential gaps and unmet needs to strengthen the use of PED. Analysis of the survey will be conducted in parallel to finalisation of the reflection paper.

**Conclusion**

The EMA aims to give a strong signal that it considers PED an important contribution to the totality of evidence and wants to support its use in regulatory decision making. However, the data must be of high quality, and scientific advice and qualification are the best avenues to address quality concerns. Transparency improvements through changes to assessment reports will help monitor how PED is used in the EU. Collaboration with patient groups, industry, HTA bodies, and clinical decision makers is essential, and the ongoing survey will help illustrate how to improve in the future.



## How is patient engagement and patient experience data used in the HTA evaluation and decision-making process?

**Laura Norburn,** Senior Operations Manager, People and Communities Team, National Institute for Health and Care Excellence (NICE)

**Patient involvement in guidance development**

Involving patients in guidance development serves four key purposes: adding new evidence and information, challenging existing evidence, adding qualitative context to quantitative data, and challenging professional assumptions. A notable example involved a treatment for myelofibrosis, where patient experts revealed that itching and fatigue were the most significant impacts on quality of life, rather than spleen size reduction, which was the focus of clinical measurements. This highlighted how patient input can identify outcomes that matter most to patients but might be overlooked in clinical trials.

**Sources of patient evidence in HTA**

NICE uses multiple sources of patient evidence in its HTAs:

- Company submissions including clinical and cost-effectiveness data
- Stakeholder submissions from professional organisations, patient/carer organisations, and patient/clinical experts
- Evidence assessment group critiques
- Consultation comments on draft guidance and scopes.

Throughout the HTA process, which typically takes about 18 months, there are numerous opportunities for patient involvement, from topic selection and scoping to committee meetings, consultations, and appeals. NICE manages approximately 100 medicine assessments and 40 HTAs simultaneously.

**Patient evidence at committee meetings**

NICE's independent advisory committees include academics, industry members, NHS representatives, and two lay members. These lay members have a specific role in examining patient evidence and presenting it at committee meetings. A survey of committee members revealed that patient evidence helps them capture disease aspects not reflected in models, validate whether models reflect lived experience, understand impacts on carers, identify difficulties in treatment administration, and appreciate effects on daily life.

**Case studies of patient involvement**

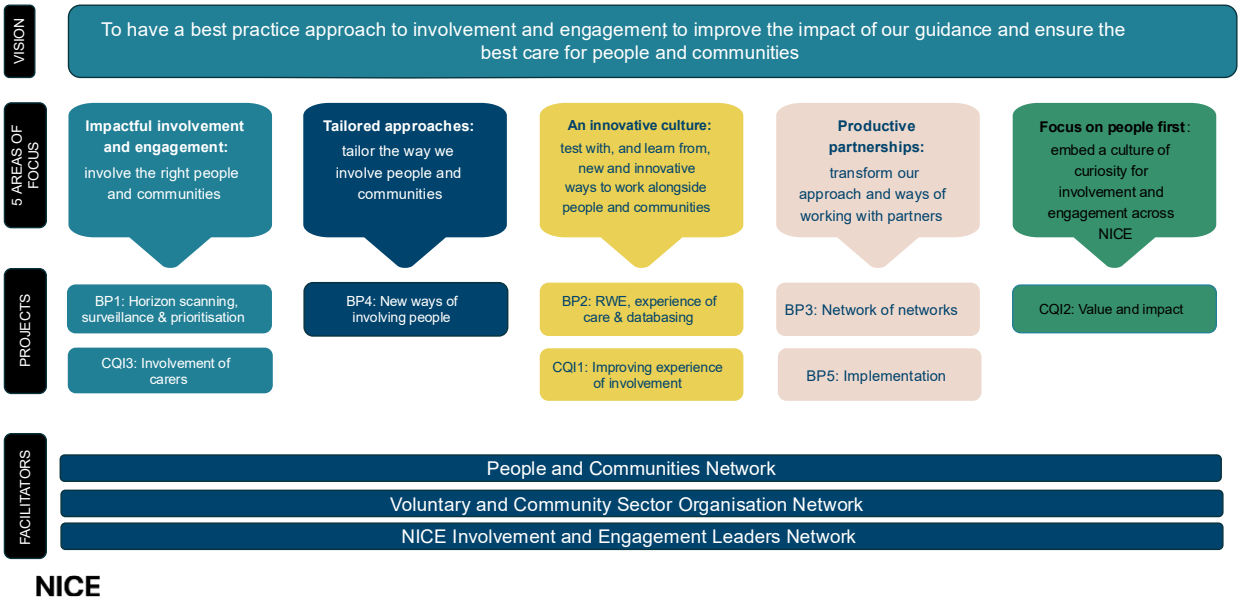
Three examples demonstrate the impact of patient involvement in NICE HTA processes:

- Digital therapies for tic disorders and Tourette's: Tourette's Action conducted a survey with over 1,500 responses, providing powerful insights into the lived experience of the condition, including pain, stigma, isolation, and emotional health impacts.
- Treatment for HIV-1: Strong feedback from the community emphasised that people with HIV-1 didn't see themselves as patients but as a community. Their input highlighted concerns about stigma related to daily tablets, which influenced the assessment of a bi-monthly injection alternative.

- Treatment for visual impairment in Leber's hereditary optic neuropathy: A patient organisation survey at the consultation stage provided crucial evidence about carer needs, giving the committee more confidence in their recommendation.

**Future directions**

NICE's strategy for involvement and engagement focuses on impactful involvement and engagement, tailored approaches, innovative culture, productive partnerships, and focusing on people first (see below). NICE has started work to understand the impact of involvement, engagement, and patient evidence, considering whether impact means changing recommendations, shaping recommendations, building understanding, or influencing organisational work.




**Conclusion**

The impact of patient evidence exists on a continuum: where there is good quality evidence with no gaps, patient information provides context and confirms assumptions; where evidence is limited, such as in rare diseases, patient evidence can have more impact by filling gaps and explaining what's missing.

NICE supports its patient engagement work through three networks: a People and Communities Network of 250 individuals with lived experience, a Community Organisation Network of 250 patient organisations, and an internal Involvement and Engagement Leaders Network to build capacity within the organisation. These networks help shape NICE's ambitions and ensure patient perspectives are integrated throughout its work.

## FDA's use of patient experience data

**Robyn Bent,** Director, Patient Focused Drug Development Program, Center for Drug Evaluation and Research (CDER), US Food and Drug Administration (FDA),

**Patient-Focused Drug Development**

Patient-focused drug development (PFDD) is a systematic approach to ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. The FDA has several PFDD efforts, including the PFDD Methodologic Guidance Series. Patient experience data (PED) encompasses many different types, some of which can be used as endpoints in clinical trials, while others can be hypothesis-generating.

**FDA assessment of PED use**

The FDA published a report in 2021 assessing how PED was used in regulatory decision making. The report examined 1169 review documents, including 176 New Molecular Entity (NME) New Drug Applications (NDAs) and Biologics License Application (BLAs) received between 12th June 2017 and 12th June 2020, and approved by 5th February 2021. Of the 176 NME reviews, 68% mentioned PED, 66% referred to PED submitted as part of the application, and 7% referred to PED from other sources such as natural history studies or meetings with patients.

The report also analysed how PED was referenced in review documents, categorising four approaches:

- Summary of data (98% of NME reviews that referenced PED) - FDA presents or describes PED without further interpretation or analysis.
- Interpretation of data (48%) - FDA provides comments or conclusions about the relevance of PED to the review.
- Factoring into the decision (16%) - FDA explicitly cites PED in the benefit-risk framework or discussions of factors contributing to a regulatory recommendation.
- Analysis of data (15%) - FDA provides its own analysis of PED differently than originally presented by the applicant.

A follow-up report is expected around June 2026, with expectations that some of these numbers should increase.

**PED table**

The FDA includes a PED table in all reviews, completed by reviewers to convey what type of PED was submitted and considered. The table is divided into sections for PED submitted as part of the application, such as clinical outcome assessment (COA) data, qualitative studies, natural history studies and patient preference studies, and PED incorporated by reviewers from other sources, including input from participating in meetings with patient stakeholders, PFDD meeting reports and observational survey studies.

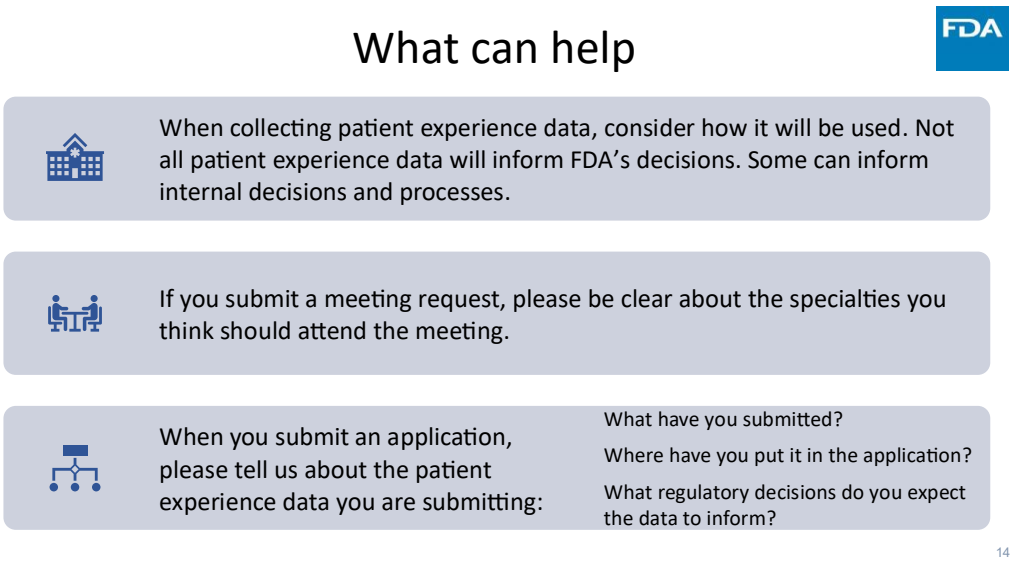
**Recommendation for applicants**

Since 2019, the FDA has asked applicants to clearly indicate what PED they are submitting and where it is located in the application through the electronic Common Technical Document (eCTD) Technical Specifications Document. However, only about 20% of applications include this information.

The FDA needs to know what is being submitted, where it is located in the application, and how the applicant thinks it will inform FDA's regulatory decision making. The FDA is working on making these requests more explicit.

**Conclusion**

Different types of PED can have different utilities in the review process. However, not all PED needs to be submitted to FDA, particularly if it informs internal company decisions and processes. Much PED is received and discussed well before the review stage. When submitting meeting requests, it's helpful to be clear about the specialties needed in the meeting. When submitting applications, applicants should tell the story of the PED so the FDA can understand what was collected, where it is located, and how it will be informative to the review process.



## Patient engagement in Canada's Drug Agency Pharmaceutical Reimbursement Reviews

**Michelle Gibbens,** Director, Engagement, Canada's Drug Agency

**Pharmaceutical reimbursement review process**

When an evidence package is submitted by an industry sponsor to Canada's Drug Agency (CDA-AMC), it is first assessed by an internal review team. At this stage, a call for input goes out to invite submissions from patient and clinician groups. This input is made available to both the internal review team during their assessment and to the expert committee during their deliberations. Following this, the expert committee deliberates within a recommendation framework.

**Integrating patient perspectives into reviews**

There are three main ways that patient perspectives are incorporated into reimbursement reviews:

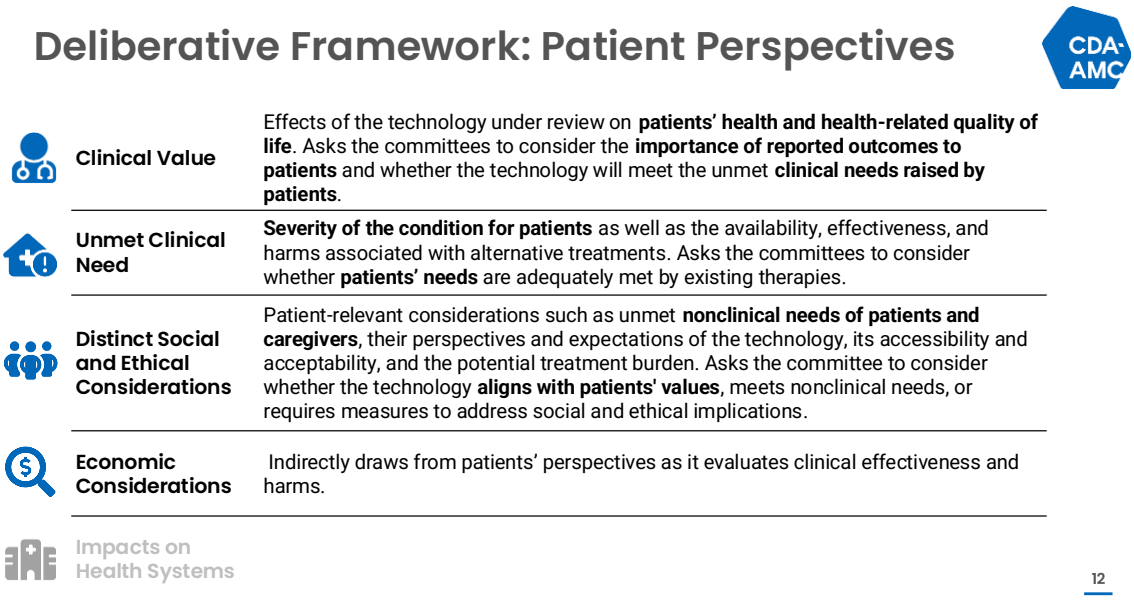
- **Patient group input:** Written input provided by patient organisations or informal groups, focusing on providing multiple patient perspectives to give a broader overview of experiences, values, and treatment goals. A written template guides this input submission.
- **Persons with lived experience presentations:** A newer approach that uses more inclusive language to encompass those who consider themselves patients, those living with health conditions who may not identify as patients, and family members or caregivers. These presentations occur at the beginning of the expert committee's deliberative meeting, allowing individuals to share personal insights aligned with the committee's deliberative framework, followed by committee interaction and questions.
- **Patient members on expert committees:** Similar to other processes, patient members serve as equal committee members, not representing specific diseases but providing a disease-agnostic perspective. They help bring forward patient input received through other channels and ensure it is considered throughout the deliberative process.

**Visibility of patient involvement**

The written patient group input is posted in full (including conflict of interest declarations) on the public website. A summary is included in the review report, with aspects highlighted in the recommendations report. For persons with lived experience presentations, a summary is included in the recommendation report. Presenters can choose to be identified if they so wish. The summary is initially drafted by staff but shared with the presenters to validate that it accurately represents their contribution.

**Deliberative framework**

CDA-AMC recently published the deliberative framework used by expert committees to increase transparency about what is considered in the deliberative process and where patient perspectives can be incorporated. The framework includes five domains: clinical value, unmet clinical need, social and ethical considerations, economic considerations, and impacts on health systems (see below).




**Patient input vs patient evidence**

An important distinction is made between 'patient input' and 'patient evidence.' Patient input is obtained through patient engagement activities and is treated differently than patient evidence that might be part of an industry-submitted package. This distinction is important because patient input is not subjected to critical evidence appraisal but is considered across all deliberative domains to contextualise clinical evidence. Patient experience data submitted as part of industry evidence is subject to critical appraisal and considered as supplemental evidence to establishing clinical effectiveness.

**Conclusion**

CDA-AMC is evolving its patient group input process and focusing on improving how it communicates in recommendations reports. They are working to add clarity that links what was heard from patient input to how it was considered by the committee and how it affected the recommendation. The agency has recently completed a preliminary phase of engagement on evolving the input process and has identified actions to advance changes in the coming months, including immediate implementations and a test-and-learn approach to expand methods for patient input involvement.

## What would companies like to see articulated in public facing documents and why is this important?

**Amaia Clemente,** Regulatory Science and Policy Associate Director EU/AMEE, Sanofi, Spain

**Vision for patient voice in public documents**

Companies want to see patient voice articulated in public-facing documents as an indispensable component of the totality of evidence. The vision encompasses three key elements:

- **Patient engagement activities** that gather involvement and inform patient experience data should be acknowledged in public-facing documents.
- **Patient experience data (PED)** should be relevant in prioritising and defining unmet medical needs, informing drug development and regulatory/HTA decisions, and should be available to healthcare professionals and patients to inform clinical decisions.
- **Patient experience evidence** (high-quality evidence from patient experience) should be pivotal for drug approval, pricing, and reimbursement, and should be included in labels, HTA dossiers, product information, and clinical decision making.

**Changing role of patients**

The role of patients has evolved significantly. Patients are now healthcare decision makers who are informed, not merely subjects of research but participants who inform research. Patients are experts in their disease (especially for chronic conditions), advisors in research, sources of research questions, funders or drivers of research, and contributors with their data and samples.

**Visibility challenges and recommendations**

Visibility ensures the relevance of patient engagement efforts. According to a European Federation of Pharmaceutical Industries and Associations (EFPIA) survey, the main hurdles in using PED are related to visibility:

- Sometimes perceived as "nice to have" or cosmetic
- Submission templates may not consider a separate section for PED
- Lack of transparency on how regulators use PED
- Challenges in quantifying impact to secure internal company support and budget
- Uncertainty about how PED is perceived and valued by regulators.

The EFPIA position paper on transparency of patient evidence made several recommendations to the European Medicines Agency (EMA), including integrating PED into regulatory documents e.g. assessment reports, developing guidance on patient preference studies, and promoting development of methodologically sound clinical outcome assessments in drug development suitable for inclusion in the product information.

**Label limitations**

In the EU, there is no specific section for PED in the label. Questions remain about the level of quality needed for PED to be included in labels and whether there should be an informative section for data that is not considered 'hard evidence.'

Language in labels should be reader-friendly, with explicit mentions that certain data represents patient voice and detailed explanations rather than broad concepts like 'quality of life.' In the EU, the Summary of Product Characteristics (SmPC) is targeted to healthcare professionals, not structured to acknowledge patient engagement and evidence separately.

PED that does not make it into the label should be communicated in other ways, such as in assessment reports, publications, informative documents for healthcare professionals, and patient-facing documents.

**Conclusion**

Progress has been made with the Patient-Focused Drug Development programme at the US Food and Drug Administration (FDA), EMA PED position paper, updated EMA assessment report template, and recognition of PED in EU network strategy and proposed pharmaceutical legislation. However, improvements are needed regarding transparency on PED in labels and public-facing documents, to ensure relevance in clinical decisions.

Companies call for guidance on requirements for including PED in labels, methodological guidance to collect PED, international convergence and common criteria, and transparency about the relevance of submitted PED.

Some challenges persist in the complex ecosystem of regulatory and HTA decision making, including prevention of undue promotion. However, industry needs predictability on the use of PED, and all stakeholders need visibility of the relevance of patient's voice, in its diverse levels of evidence provided.

## We need Visibility on real impact of PE and PED

- ✓ Acknowledgement of PE & measurement of its value

  Patients have been heard, but have their insight had a role? Which one?
- ✓ Description of PED & origin (applicant/other)
- ✓ How the HA/HTA considered it and to what extent
- ✓ Rationale on why not considered
- ✓ How often patient voice influenced/changed final decision

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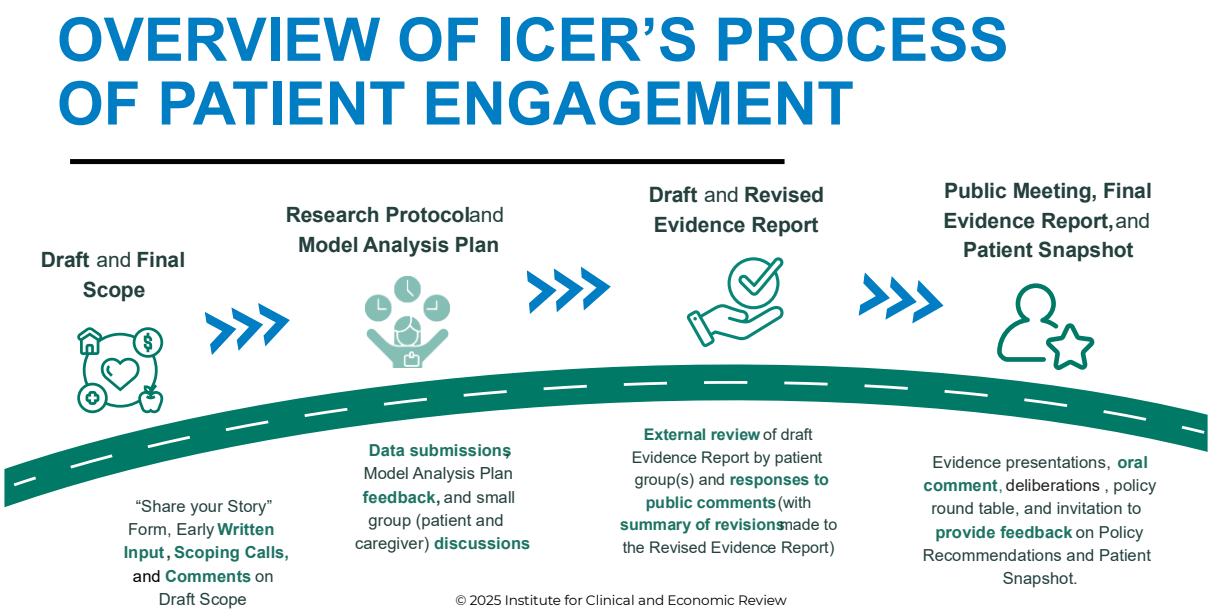
## What should be communicated about patient engagement and patient experience data, and in what way?

**Dr Marina Richardson,** Associate Director, HTA Methods and Health Economics, Institute for Clinical and Economic Review (ICER), USA

**ICER's process of patient engagement**

The Institute for Clinical and Economic Review (ICER) in the US prioritises topics for review based on a set of pre-specified criteria rather than receiving evidence submissions from sponsors. ICER selects about 7-8 high-impact topics annually and conducts the clinical assessments and cost-effectiveness analyses internally and with academic collaborators.

ICER's review process includes producing draft and final scopes, developing research protocols and model analysis plans, creating draft and revised evidence reports, holding transparent public meetings, and producing final evidence reports and subsidiary documents like Patient Snapshots. Throughout this process, ICER engages with patients in various ways (see below).




**Case study of patient engagement impact**

While reviewing brensocatib for non-cystic fibrosis bronchiectasis, ICER received 80 individual responses through its 'Share Your Story' form (a record amount). Thematic analysis revealed consistent themes about unmet need, burden of airway clearance, and impact of exacerbations. This gave the review team confidence in assessing clinical significance of treatment effects and helped inform gaps in available evidence. Patient community insights were included in the evidence report and public meeting slides.

**Challenges and opportunities for standardised communications**

Variation in processes and resources is a key challenge to standardising communications about patient engagement. The type and timing of engagement vary depending on the review and organisation, and resource requirements differ between HTA bodies as well as patient organisations.

In addition, the measurement of impact is complex as it must be considered from multiple perspectives (patient community, HTA organisation, decision makers, life sciences community). It is difficult to quantify certain types of impact, such as the building of trust between stakeholders.

Despite challenges, there are opportunities for establishing standardised principles for communication about patient engagement. These should focus on transparency (explanation of the process and how engagement was carried out), dialogue (giving opportunity for understanding), and communication (clear, accessible and continuous).

**Conclusion**

While standardisation of communications about patient engagement may be challenging due to variations in processes across organisations, there are opportunities to establish common principles focused on transparency, dialogue, and clear communication. When considering how patient engagement impacts decision making, it's important to consider multiple perspectives, including those of the patient community, HTA organisations, decision makers, and the life sciences community. Case studies demonstrate that patient engagement can significantly influence assessments and subsequent decision making when properly integrated into the review process.

## Panel discussion: What should be communicated about patient engagement and patient experience data, and in what way?

Several themes arose from the panel discussion featuring speakers from Session 3:

**Visibility and communication**

- Need for transparency in how insights from patient engagement (PE) and patient experience data (PED) are used.
- Importance of making research accessible beyond paywalled journals.
- Stakeholders should establish long-term relationships with patients and provide them with feedback.

**Harmonisation and collaboration**

- Desire for global consensus on PED processes.
- Need for PED principles that can work across different contexts.
- Opportunities for collaboration on PE and PED should be explored.

**Impact of PE and PED**

- Impact is multi-faceted; not just about the impact in decision making, but also:
  - Impact on evidence interpretation
  - Impact on participants' experience
  - Building trust (both an impact and condition for effective engagement)
  - Contribution to the evidence base.
- Case studies of successful and unsuccessful PE/PED should be shared to facilitate cross-stakeholder learning.

**Complementary data sources**

- Risk of separating PE from other data types.
- All PED should be informed by PE.
- All sources of PED are complementary; they each serve a different purpose but contribute to a comprehensive understanding.

## Session 4: Breakout discussions

Workshop participants were assigned to a breakout group and provided with a background document developed by CIRS, containing information and questions for discussion. The Chairs and Rapporteurs of each breakout were asked to facilitate and document the discussion, respectively. The Rapporteurs then fed back to all workshop participants in the main plenary session.

### Breakout A: Creating supportive policies for meaningful PE and PED

**Chair: Dr Nick Crabb,** Chief Scientific Officer, NICE, UK

**Rapporteur: Sunera Awan,** Head of Regulatory Affairs UK&I, Bayer, UK

**Background**

Despite growing interest in patient engagement (PE) and patient experience data (PED), their integration into regulatory and HTA frameworks remains inconsistent and underdeveloped. While some regulators and HTA bodies have issued guidance, the policy environment lacks clarity, incentives, and infrastructure to support systematic generation, submission, and use of PED.

This breakout group comprised of representatives from the pharmaceutical industry, regulatory and HTA agencies, and patient organisations explored what is needed in the policy space to unlock the full potential of PE and PED in development in order to aid regulatory and HTA decision making.

**Current regulatory and HTA expectations for PE/PED**

The group discussed how PE/PED activities are not mandatory for all health authorities, and there are cultural differences in how much engagement patients want. HTA agencies and regulators have different requirements, and the HTA community is currently leading in the PE/PED field. While science is global, HTA is local, and patient experience can influence that. However, if every authority demands different types of PED, efficiency may decrease and costs increase.

Historically, regulatory advice during clinical development is prioritised over patient organisations' advice, as it is key to obtaining a marketing authorisation. This can lead to PED becoming exploratory rather than being central to development. While regulators' mindsets are changing to become more accepting of PED, it may not influence a regulatory benefit-risk decision as much as an HTA outcome.

**Barriers to industry generating and submitting PE/PED**

A significant barrier to industry generating PE/PED is demonstrating return on investment to key stakeholders internally. There is also a mindset shift needed within companies to break down silos and encourage sharing of PE/PED insights between different functions. There may be a role for regulatory teams to bridge the gap between clinical and HTA teams.

If regulators and HTA agencies were more transparent about how they use PE/PED, this would help demonstrate value within industry. The meaning of PE and PED to agencies is evolving, with greater recognition of what patient organisations bring to the table. However, there needs to be more awareness of overlapping PE/PED that can be used by both regulators and HTA agencies. Investigation into PE/PED at a disease level rather than an asset level would also help to reduce duplication.

**Policy actions to promote consistent and meaningful PE/PED**

The group agreed that more guidance on PE/PED generation and use would be beneficial, though it can be difficult for HTA agencies to mandate and give guidance compared to regulators. Overarching principles agreed on by all stakeholders may work better in the HTA context. There also needs to be greater understanding of the benefit that PED/PE has on the full value chain of medicine development ("starting with the end in mind").

There is no overarching forum for patient organisations that could assist with coordinating PE and PED requests from companies and other stakeholders. Establishing such a forum could help to reduce duplication of PE/PED and preserve patient organisation resources.

**Supporting patient organisations to contribute robust PE/PED**

Patient organisations face capacity and capability challenges when it comes to generating robust PE/PED. There may be opportunities for industry to provide funding support, training and education to patient organisations.

Patient organisations should come together to try to reduce duplication in generating PE/PED, while companies explore the potential for sharing PE/PED data between themselves. In addition, companies should engage early with patient organisations, seeing them as collaborative partners throughout development.

There is currently a gap in terms of what constitutes robust PE/PED that meets regulatory and HTA standards. Regulatory and HTA agencies should issue guidance and key principles to address this.

**Recommendations for further work and research**

- Regulators, HTA agencies and patient organisations should engage early on PE and PED, using clear criteria to decide relevance. They should work together to establish guiding principles, a best practice repository, and a global patient organisation forum to coordinate PE/PED requests.
- All stakeholders should focus PE and PED efforts at the disease level, not just the product level, to align endpoints with both patient and regulatory/HTA needs. Consideration should be given to creating a disease PICO framework.
- Companies should promote clear internal messaging on how PE and PED support clinical development and regulatory/HTA interactions, helping teams understand the value and application of PE and PED.
- Companies, regulators and HTA agencies should collaborate with patient organisations as equal partners, engaging them early with transparent communication, education, and training.

**Other issues for further discussion**

There were several issues that the group raised but did not have time to address:

- Communication of PE/PED – Companies need to make it easy for regulators and HTA agencies to find and understand submitted PE/PED, including why it was submitted.
- Mandating evidence from PE/PED - This is very difficult in practice.
- Early engagement – How to incentivise companies to engage early with patient organisations.
- Diversity in patient perspectives - Not everyone wants to be seen as a patient, and patient organisations may have different demands.
- Target Product Profile (TPP) and Target Value Proposition (TVP) - How to ensure they include PED/PE.

## Breakout B: Showing the impact of PE and PED in public documents

*Please note that two groups tackled this topic in parallel and are reported on separately.*

**Background**

Various regulatory and HTA agencies have established mechanisms to enable both PE as well as the collection and use of PED. However, the visibility of PE and PED and their impact in decision making is inconsistent across public-facing documents, such as regulatory summaries and HTA assessment reports. There is a need to identify how best to articulate how the information has been used as well as its impact on the final decision.

Current reporting practices often lack transparency about how patient input was considered, whether it influenced decisions, or why it may have been disregarded. This creates a risk of "tick-box" engagement, where patient involvement is acknowledged but not meaningfully integrated. Without clear feedback mechanisms, patients and contributors are left uncertain about the value of their input.

These breakout groups comprised of representatives from the pharmaceutical industry, regulatory and HTA agencies, and patient organisations explored what is needed to improve the visibility and reported impact of PE/PED in public documents, and how feedback to patients can be strengthened to foster trust and accountability.

### Group B1 discussion summary

**Chair: Dr Kelly Robinson,** Director General, Pharmaceutical Drugs Directorate, Health Canada

**Rapporteur: Jessica Abel,** Director, PED Policy & Best Practices, Patient-Centred Outcomes Research, AbbVie, USA

**Current visibility of PE/PED in public documents**

The group distinguished between regulatory and HTA agency approaches to reporting PE and PED. Regulator practices vary; for example, Health Canada has no specific reporting on PE or PED but mentions in summary decision documents if these influenced decisions. The European Medicine Agency (EMA)'s new Committee for Medicinal Products for Human Use (CHMP) report template includes a PED table in a separate section, which is published as part of the European public assessment report (EPAR). The US Food and Drug Administration (FDA) similarly publishes a PED table in reviews for all submissions. For HTA bodies, most begin with basic input and include recommendations documents that mention types of patient input received and whether decisions aligned with patient feedback. For example, the French HTA agency HAS, has been known to link written contributions from patients to its final decisions.

Important distinctions were noted between PE and PED (patient input versus patient evidence), between regulatory and HTA expectations, and between sponsor-generated PED versus insights submitted directly by patient groups. Sponsors need to understand PED evidentiary needs of both regulatory and HTA stakeholders early in development. Clear and harmonised regulatory/HTA guidance and opportunities for early and continued engagement would help to support this.

The group identified various gaps in the reporting of PE/PED in public regulatory and HTA documents. These are usually aimed at a professional audience, with no patient-friendly language. There's a need for consistent recognition of patient contributions in language accessible to patients. Outcomes of expert committees including patients are not typically articulated, and there's an opportunity to involve patients in joint scientific advice to inform evidence generation and decision making.

**Feedback to patients**

The group distinguished between formal public-facing feedback from agencies and targeted feedback sent directly to sponsors or patient groups. Building relationships with patient organisations is important to ensure their input is meaningfully incorporated throughout the development lifecycle. Establishing feedback loops to acknowledge receipt of patient input and communicate its meaningfulness is critical. There should also be clear communication to patients and patient organisations on their duties and responsibilities, helping to set expectations and build trust.

Patient input should be recognised on the same level as other stakeholders, with clear articulation of how contributions added value and were used in decision making. More transparency is needed on how PED is used to inform decision making and what input regulators and HTA bodies receive directly from patients.

**Tools and standards to improve reporting**

The group agreed that there is a need to harmonise terminology for PE and PED as well as reporting tools, particularly across HTA agencies. The PED table used by FDA and EMA could be expanded to other regulators and potentially HTA agencies. Assessment reports should include a distinct section on PED as well as discussing PED in relevant sections where it informed decision making. In addition, agencies should consider developing a high-level document explaining what PED is and where it is included in assessment reports.

Patient-centred communications on how insights were considered and their impact on decisions are important. Electronic tools such as the electronic product information (ePI) could help to facilitate this. Standardising and harmonising tools for reporting patient input to regulatory and HTA agencies would be beneficial, considering core questions useful to both types of bodies, as well as specific regulatory/HTA questions. Regional templates may need to be considered to account for potential regional differences.

**Metrics to assess impact of PE/PED**

The group suggested measuring the presence of PE/PED in the regulatory label, including the rationale for why PED was or was not included. Stakeholder surveys could help to identify case studies illustrating effective use of PED. CIRS or other interested groups could survey regulators and industry on where they think PED has an impact to identify trends and areas of highest impact. A survey of payers would also aid understanding of whether PED are helpful for payer decision making.

**Recommendations for further work and research**

- Conduct research on how to better articulate results from PE/PED to patients to inform their decision making e.g. enhanced patient leaflets.
- Evaluate existing guidance on PED generation and identify gaps where further harmonisation is needed across agencies.
- Describe situations where PED are particularly useful e.g. case studies.
- Harmonise definitions of PE and PED, as well as where evidence is generated from to inform decision making – what does high quality PED look like?
- Train and support patient organisations to generate robust PED to inform regulatory and HTA decisions.
- Evaluate the impact of PED in different HTA systems e.g. utilities vs relative clinical benefit systems.
- Improve communication of patient input in pharmacovigilance.

## Group B2 discussion summary

**Chair: Prof Hans-Georg Eichler,** Consulting Physician, Association of Austrian Social Insurance Institutions

**Rapporteur: Dr Siobhan Connor-Ahmad,** Principal Scientist, Patient-Centred Outcomes Research, Roche, UK

Group B2 approached the task in a visual way, capturing PE and PED activities on a continuum of impact and visibility. Quadrant charts - where the top right quadrant represents high impact and high visibility - were created for different stakeholder perspectives (see p58).

### Industry perspective of PE/PED impact and visibility

From an industry perspective, no PE/PED activities were rated as high impact and visibility. Patient preference studies inside or parallel to clinical trials, endpoint selection, and PRO selection were considered to have moderate impact and visibility, while patient advisory group activities were rated low. Qualitative research around individual patient experiences was rated as potentially high impact but with zero visibility due to anonymisation.

### Patient perspective of PE/PED impact and visibility

Patient organisations (PO) were seen to be efficient and effective in PE/PED activities, with most having moderate to high impact. High impact but low visibility activities included identifying individual patients for engagement activities, such as sitting on a regulatory/HTA/payer committee, and PO-informed drug development. High visibility with moderate impact included PO-initiated surveys and PO attendance and input into appraisals.

### Regulator perspective of PE/PED impact and visibility

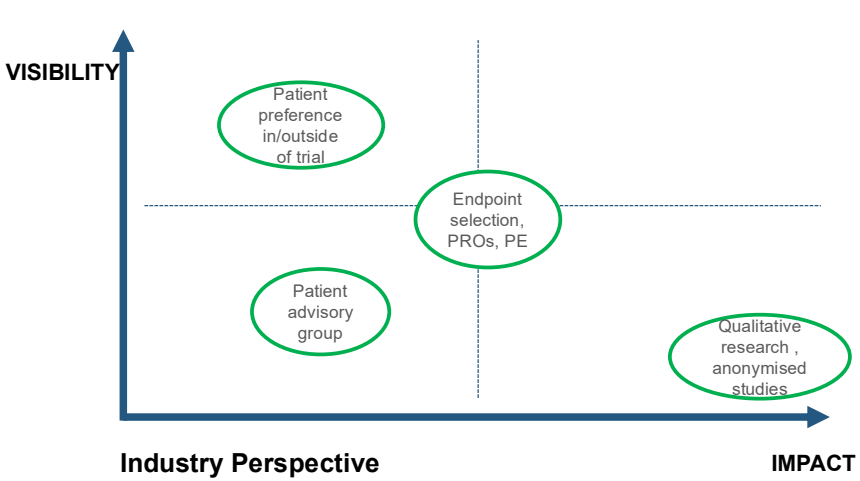
Regulators were similarly described as efficient and effective, with several high impact, high visibility PE/PED activities including PE early in advice, PE in decision making and anecdotal unstructured PE. Scenarios where patients disagree with a drug's withdrawal because they feel living with the condition is worse than the risk of side effects were placed in the top-top quadrant.

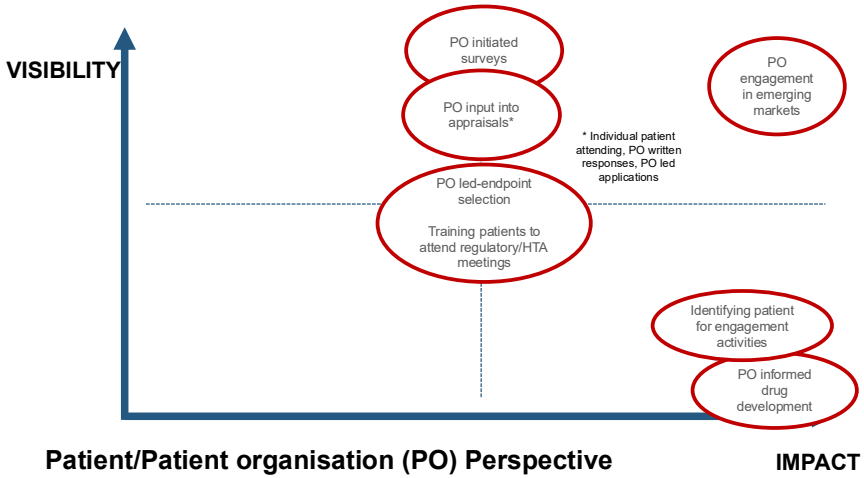
### HTA perspective of PE/PED impact and visibility

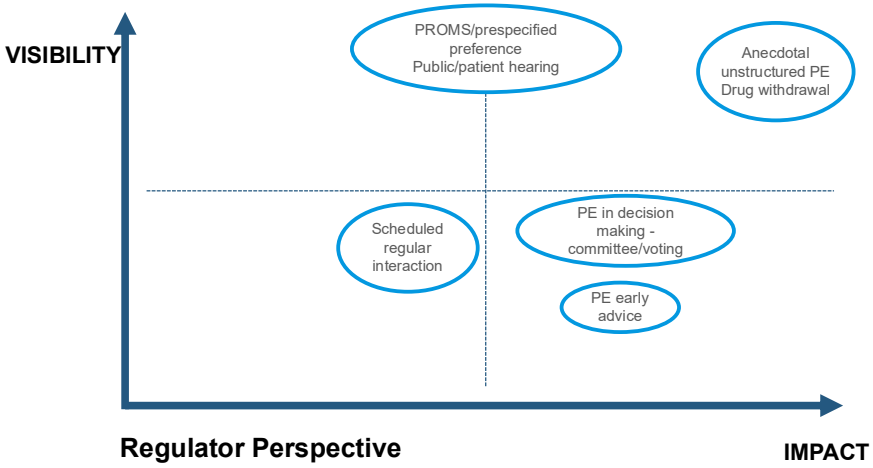
For HTA agencies, PE/PED activities were scattered across all quadrants. High visibility but low impact activities included observers attending meetings, though this to facilitate transparency rather than drive impact. Horizon scanning was rated as high impact but low visibility, in contrast to scoping which was rated low-low. High visibility, high impact was achieved when patient representatives were voting members of decision-making processes.

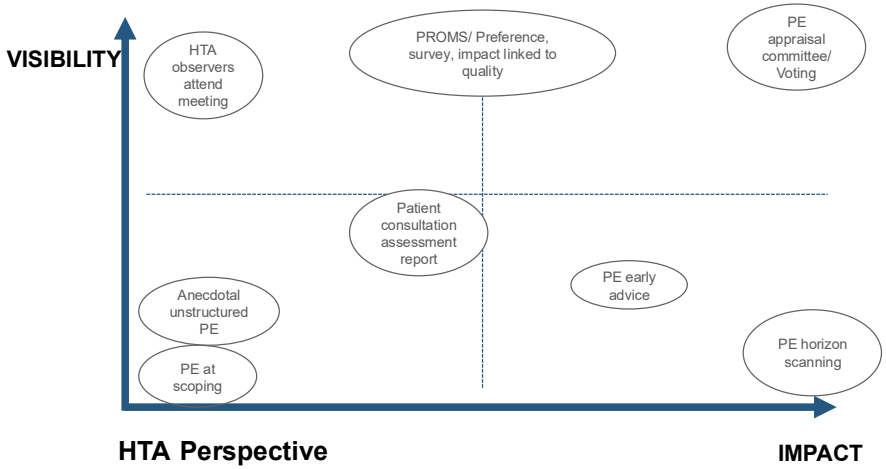
### Recommendations for further work and research

- Increase transparency of PED and PED activities that have high impact but low visibility by publishing evidence in peer-reviewed journals.
- Increase quality of PE and PED activities that have low impact but high visibility by introducing standards for engagement and evidence.
- Standardise a checklist for PE and PED information for regulatory and HTA agencies, linked to Daniels & Sabin's framework of 'accountability for reasonableness'. CIRS could perhaps lead this work.
- Regulatory and HTA agencies should provide clarity on the role of PE and PED information in decision making. The CIRS Quality of Decision-Making Orientation Scheme (QoDoS) checklist could perhaps be leveraged.


VISIBILITY
Patient preference in/outside of trial
Endpoint selection, PROs, PE
Patient advisory group
Qualitative research , anonymised studies
Industry Perspective
IMPACT


VISIBILITY
PO initiated surveys
PO input into appraisals*
* Individual patient attending, PO written responses, PO led applications
PO engagement in emerging markets
PO led-endpoint selection
Training patients to attend regulatory/HTA meetings
Identifying patient for engagement activities
PO informed drug development
Patient/Patient organisation (PO) Perspective
IMPACT


VISIBILITY
PROMS/prespecified preference Public/patient hearing
Anecdotal unstructured PE Drug withdrawal
Scheduled regular interaction
PE in decision making - committee/voting
PE early advice
Regulator Perspective
IMPACT


VISIBILITY
HTA observers attend meeting
PROMS/ Preference, survey, impact linked to quality
PE appraisal committee/ Voting
Patient consultation assessment report
PE early advice
Anecdotal unstructured PE
PE at scoping
PE horizon scanning
HTA Perspective
IMPACT

## Breakout C: Aligning patient involvement across regulatory and HTA agencies to reduce duplication

**Chair: Dr Anke-Peggy Holtorf,** Founder and Managing Director, Health Outcome Strategies

**Rapporteur: Giorgia Rauso,** Associate Director, International Patient Advocacy, Regeneron, Italy

**Background**

Although PE and PED are increasingly recognised as important sources of information for decision making, their use across regulatory and HTA processes remains fragmented. Regulatory and HTA agencies have differing remits and often operate with different timelines, evidentiary standards, and models of participation, potentially leading to duplication, inefficiencies, and missed opportunities to leverage patient insights.

Efforts such as parallel scientific advice, shared data platforms, and aligned expectations offer promising avenues for greater coherence. Yet, consensus on how and when to involve patients—and how to make their input accessible across both domains and beyond single assessments —is still evolving.

This breakout group comprised of representatives from the pharmaceutical industry, regulatory and HTA agencies, and patient organisations explored how stakeholders can work together to create a more integrated, efficient, and impactful approach to patient involvement across regulatory and HTA processes.

**Objectives of involving patients in regulatory and HTA processes**

The group identified commonalities in how regulatory and HTA bodies benefit from PE and PED (see table below). Differences emerged in that regulatory agencies focus on risk-benefit assessment, while HTA bodies focus more on justice, democracy, and incremental health benefit assessment. These differences stem from the different questions each process addresses: regulatory asks if an intervention does more good than harm; HTA examines if benefits are useful, appropriate, and affordable; and coverage decisions consider system sustainability.

| | REGULATORY & HTA AGENCIES | REGULATORY ONLY | HTA ONLY |
|---|---|---|---|
| **PE** | • Bringing different points of view to the table (patient vs physician)<br>• Clarifying unmet needs<br>• Covering all the 'gaps' (design/interpretation)<br>• Building trust in the system through participation | • Explaining the context for improved benefit-risk assessment | • Justice/democracy / health equity<br>• Understanding relevance / value in local healthcare context<br>• Patient acceptance due to understanding the process and decision criteria |

| | | | |
|---|---|---|---|
| **PED** | • Improving representativeness<br>• Supporting data scientific robustness<br>• Post-commercialisation monitoring | • Balancing risks and benefits | • Supporting value assessment<br>• Assessing incremental health benefit |

**Similarities and differences in stakeholder approaches**

The group created a second table showing how different stakeholders approach PE and PED, such as patient-reported outcomes (PRO), patient-reported evidence (PRE) and patient preferences (PP) (see below). Patient organisations seek access to effective care, industry seeks commercialisation (making treatment that is used), regulatory agencies assess risk and benefit, and HTA bodies conduct value assessments.

The conclusion of the discussion was that PE and PED can be leveraged by both regulatory and HTA assessment models. For PE, the two assessments can be combined to a certain point, though value/cost and justice/democracy discussions should remain separate. For PED, both bodies can use the same data while asking different questions.

| | **PATIENT ORGANISATIONS** | **INDUSTRY** | **REGULATORY AGENCIES** | **HTA BODIES** |
|---|---|---|---|---|
| **FORMS OF PARTICIPATION** | | | | |
| INDIVIDUAL TESTIMONY/WRITTEN SUBMISSION | X | X | X | X |
| ROUNDTABLES | X | X | X | X |
| PARTICIPATION IN REGULATORY/HTA COMMITTEES (FIRSTHAND EXPERIENCE) | X | X | X | X |
| INTERVIEWS | X | X | X | X |
| FOCUS GROUPS | X | X | X | X |
| **TYPES OF PED (PRO, PRE, PP, other)** | | | | |
| INTERVIEWS | X | X | X | X |
| SURVEYS | X | X | X | X |
| PED in SUBMISSIONS DOSSIER | | X | | X |
| PED SPONTANEOUS SUBMISSIONS | X | X | X | X |
| ALL TYPES, AS LONG AS HIGH QUALITY AND REPRESENTATIVE | X | X | X | X |
| FINANCIAL BURDEN OF TREATMENT | X | | | X |

**Barriers to PED use across regulation and HTA**

The group identified several barriers to the use of PED across regulatory and HTA processes:

- Policy: national policies, counter-participatory policies, and conflicts of interest.
- Structural/institutional: historical practices, different timelines, and centralised regulatory versus local HTA processes.
- Methodological: lack of competence in using types of PED, challenges in ensuring representation (individual versus population), and privacy issues.
- Mindsets: recognition that one size cannot fit all, and the disconnect between expectations and actual measurement/use of PED.

**Mechanisms for better collaboration**

When asked to identify mechanisms that could support better collaboration and trust between regulatory and HTA agencies, the group felt that both stakeholders do trust each other, but there may instead be a trust issue with data from PE/PED. Understanding current gaps by bringing regulators and HTA agencies together and arranging pilots would be beneficial.

**Considerations for cross-stakeholder collaboration**

The group highlighted the importance of defining clear perimeters between regulatory and HTA agencies to avoid duplications relating to PE and PED. Patient organisations could help to co-design these perimeters. Other steps needed for a cross-stakeholder collaborative model included harmonised methodological guidance, clear PED expectations, improved coordination between regulatory and HTA assessments, and highly incentivised or mandatory early dialogue.

**Recommendations for further work and research**

- Track how regulatory and HTA agency perspectives evolve over time with increased PE and PED usage. CIRS could perhaps delve deeper into the results of its agency survey and repeat this in future.
- Gather patient perspectives on current gaps in infrastructure and policy in regulatory and HTA processes. Academics and patient organisations could work together on this study.
- Seek recommendations from patient advocacy groups and patient experts on how submissions to regulatory and HTA agencies can be made more efficient. CIRS could perhaps lead this work.
- Organise roundtable discussions across stakeholders to avoid duplication in PE/PED.
- Align on definitions of PE and PED.

## Session 5: Challenges and support for patients and patient organisations

### Panel discussion: What is needed to address the challenges patients face and ensure support for patient organisations in their advocacy roles both at a company and agency level?

**Patient perspective: Josephine Mosset,** Senior Policy Officer, Cancer Patients Europe, Belgium

- Public funding for patient groups is declining, creating issues around conflict of interest (COI) and private financing. Rising administrative burdens and stricter COI rules, while well-intentioned, can exclude patient voices.
- The European Medicines Agency (EMA) has a flexible, context-specific approach to COI, while the EU HTA Regulation's more rigid framework leaves little room for case-by-case assessments.
- There should be greater focus on transparency and managing COI for patient representatives rather than automatic exclusion, including:
    - Context-sensitive COI frameworks
    - Better understanding of what constitutes a COI
    - Scoring risk assessment tools to help patient organisations navigate these issues.

**Company perspective: Dr Siobhan Connor-Ahmad,** Principal Scientist, Patient-Centred Outcomes Research, Roche, UK

- Insights from patients and patient groups broadly fit into two categories:
    - Operational information to inform trial design and data quality
    - Disease-specific information (typically patient experience data) that is included in the regulatory and/or HTA dossier.
- There is a concern that anecdotal insights from a handful of patients can influence decision making, but this is not realistic since substantial financial investments into clinical trial designs and filling packages are more robust when based on robust insights and not anecdotal insights.
- Industry follows methodologically rigorous approaches to gathering patient input in accordance with guidelines, and any undue influence in the process represents a risk for industry.

**Regulatory perspective: Dr Fokaline Vroom,** Pharmacovigilance Assessor, Medicines Evaluation Board, The Netherlands

- To optimise the value of patient input, engagement between companies and agencies needs to begin early through scientific advice.
- Confidentiality agreements, while essential for legal reasons, can prevent individuals from gathering wider – and potentially richer – input from their patient communities.

- Transparency works in multiple directions – not only should regulatory agencies be transparent about how they incorporate patient input, but industry should also be transparent about what patient engagement has already occurred during development.

**HTA agency perspective: Dr Anja Schiel**, Senior Adviser; Lead Methodologist in Regulatory and Pharmaeconomic Statistics, Norwegian Medical Products Agency, Norway

- Considering patient involvement across the drug development continuum, there is a difference between the 'lived experience space' (early development) and 'voice space' (later regulatory/HTA processes). If mistakes are made in the expensive lived experience space, the value of patient voice later is diminished.
- While training is important to support patients through regulatory and HTA processes, there are concerns about turning patients into regulatory/HTA experts that are not representative of the population. The goal should be to access real patients' lived experiences rather than creating experts.
- It is important to remember that costs come in many forms beyond just money – including time, education, and potentially lives if patients participate in unsuccessful trials.

## Session 6: Future thinking on patient engagement and patient experience data

### Innovative methodologies: Harnessing real-world data and patient-reported outcomes in evidence generation to inform HTA decision making

**Dr Thomas Butt,** Executive Director, Health Economics and Outcomes Research, Biomarin, UK

The impact that patient-generated real-world data (RWD) has on informing HTA decision making depends on the research question and timing. Pre-launch efficacy and safety data will not come from RWD, and post-launch epidemiology and disease burden should already be established. Where RWD can be particularly valuable is in helping with the interpretation of outcomes and contextualising results.

From an HTA perspective, comparative effectiveness against standard of care is crucial. RWD can help demonstrate the effect of treatments across multiple domains of value, showing the full value of a product beyond a single measure.

**Challenges in interpreting patient evidence**

In rare diseases, there are significant challenges in interpreting patient evidence, particularly patient-reported outcomes (PROs). These include:

- Lack of validated PRO measures
- Short development timelines that don't allow for proper tool development and validation
- Single-arm trials with no comparative data
- Lack of external datasets for comparison.

For example, if quality of life data for a treatment shows a slow decline over time, this might suggest no added benefit. However, when compared with external control data showing rapid decline in functional outcomes, and combined with impactful caregiver testimony, the true value of the treatment may become apparent. Unfortunately, some HTA bodies with less flexible rules might still conclude there was no benefit because the full impact wasn't captured in the data.

**Innovative approaches to evidence interpretation**

Engagement with the patient community has helped operationalise a concept called "disease-free mind." Patients identified interconnected concepts about what the disease and treatment meant to them, categorising outcomes into areas such as event risk, injection schedule, joint pain, physical activity, travel, employment and education, and impacts on family and caregivers. These outcomes were plotted on a radar diagram against standard of care, providing a more comprehensive view of the treatment benefits. Feedback from patients and clinicians indicated that this approach better communicated the benefits of the product by showing all benefits on one page in a comparative way.

**Ensuring representativeness and robustness**

Learnings from a European preference study highlighted the importance of ensuring representative and robust patient data. The study produced unexpected results when patients appeared to prefer worse efficacy, contradicting findings from the same study conducted in the US.

Investigation revealed that the survey link had been shared on a public Facebook group, resulting in responses from people who were completing it quickly for compensation. Additionally, cultural differences in colour interpretation (green being negative in some cultures) may have affected responses when participants saw green-coded attributes for better efficacy.

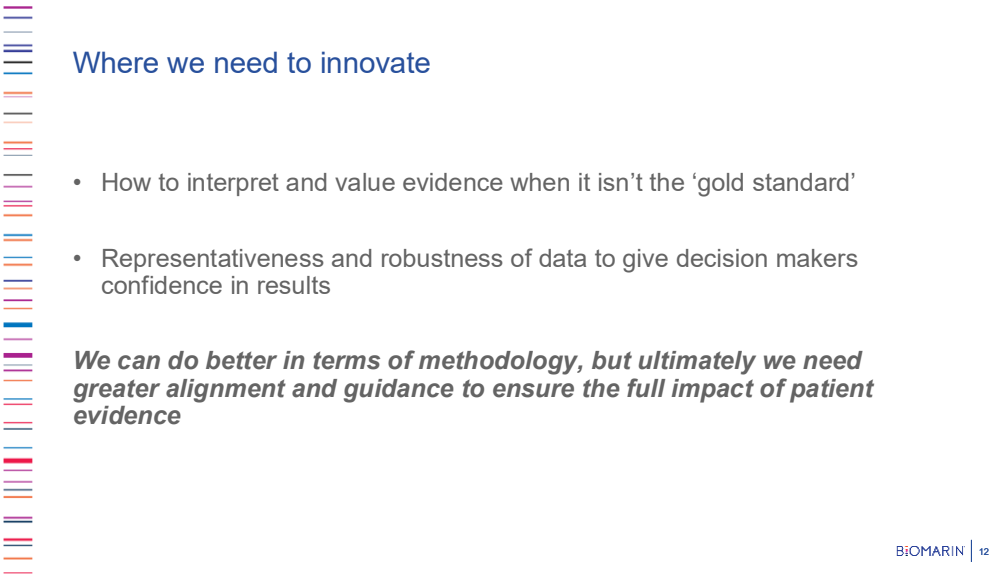
This example demonstrates the potential risks associated with preference surveys and the importance of data validation and cleaning to ensure the validity and representativeness of samples.

**Enhancing patient participation**

Understanding why people participate in research, particularly RWD research where there is no offer of a novel treatment, is crucial. Motivations may include altruism, sense of community, getting results in aggregate, and compensation. In the US, some research providers offer secure access to a curated version of patients' medical records as part of participation, which appears to enhance uptake rates by providing additional value to participants.

**Conclusion**

To improve the use of patient-generated RWD, two key areas need attention: improving the interpretation of evidence that isn't the 'gold standard' and ensuring representativeness and robustness of data to give decision makers confidence in the results. While methodologies can be iteratively improved, ultimately guidance and alignment are needed on how this evidence will be interpreted and considered, and what the full impact of this evidence will be when submitted to agencies.



## Patient preference data – Designing robust patient-centred evidence frameworks

**Dr Brett Hauber,** Patient Preference Evidence Integration Lead, Pfizer, USA

**Outcomes from IMI PREFER**

The Innovative Medicines Initiative (IMI) Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle (PREFER) project was a five-year public-private partnership that ended in 2021, producing recommendations for good practice in conducting patient preference studies and a framework for conducting such studies. The project was not meant to be prescriptive but aimed to stimulate discussion, publication, and increase collaboration.

A primary output from PREFER was a qualification opinion from the European Medicines Agency (EMA), providing EMA's view on the use of patient preference information and potential methods for patient preference elicitation. Since then, the PREFER Expert Network, a voluntary group of people involved in the original project, has been working on projects to advance and promote preference research.

**Use of preference data in regulatory decisions**

While there are examples of successful use of patient preference studies in informing regulatory decisions, nearly all have been in the medical device space. A recent example informing a drug review was a study on esketamine, where patient preference data was presented at an advisory committee meeting and had an impact, though this impact was never formally documented.

A significant breakthrough came with a patient preference study for alopecia areata submitted to the EMA. The preference study was mentioned 13 times in the EMA assessment report, demonstrating three important points:

- The value of the benefit to patients (important for a condition that might be dismissed as cosmetic).
- A quantitative benefit-risk analysis showing the probability that benefits outweighed risks from the patient perspective.
- Evidence that patients would prefer a higher dose over a lower one.

**HTA considerations**

For HTA bodies, the use of preference data is different as they face budget allocation decisions among multiple therapies. NICE has stated that patient preferences should not be put directly into economic models but can help in cases where:

- There are non-health benefits not captured in the economic model
- Multiple treatments are already available and patient perspective on fit is needed
- There is a heterogeneous patient population where preferences might differ based on characteristics.

A review identified only four clear successes where patient preference data was explicitly included in HTA submissions: one from patient group Myeloma UK to NICE, and three to PBAC in Australia, which has a willingness-to-pay approach in its HTA methodology.

**Future directions**

Several developments are underway to advance the use of patient preference information:

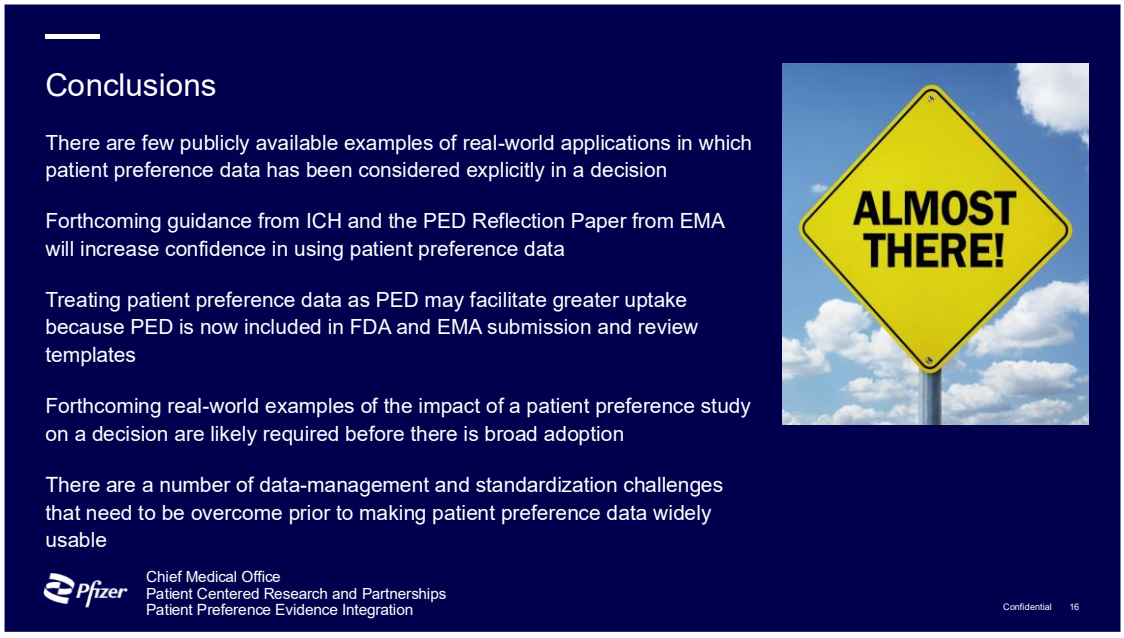
- Learnings continue to come from US FDA and EMA public statements, advisory committees, and scientific advice meetings.
- The EMA reflection paper on patient experience data (PED) was recently published.
- The ICH E22 Expert Working Group has published draft guidance on patient preference studies, which is open for public consultation until April 2026.
- A potential new Innovative Health Initiative (IHI) project focused on using clinical outcome assessments, patient preferences, and digital health technology measures together.

**Strategic approaches**

A key strategic shift has been moving away from treating patient preference information as a standalone element in regulatory and HTA assessment. Instead, patient preference information is considered as a type of PED. The 21st Century Cures Act in the US explicitly included patient preference data as part of PED, providing a legislative mandate for the consideration of patient preferences. In addition, the EMA has defined PED, including patient preference information, it its recent reflection paper. FDA's technical conformance guidance for the Module 5 of the Common Technical Document provides direction on how to submit PED to the agency. Finally, both EMA and FDA review processes include directions for reviewers to complete a PED table. In response, companies are now beginning to partner across different groups generating PED, creating communities of practice rather than separate silos.

**Conclusion**

While significant progress has been made, there is still work to be done before patient preference information is fully integrated into regulatory and HTA decision-making processes. Few examples exist of how these studies have influenced real-world decisions, though there are promising signs that more may be forthcoming. Addressing data management and standardisation issues and treating patient preference data as part of broader PED rather than as a separate entity is important for advancing the field.



## Panel discussion: Future thinking on PE and PED - How should these evolve?

**Patient perspective: Alastair Kent,** Chair of Trustees, Gene People, UK

While early engagement with patient organisations is consistently emphasised as important, it's crucial to remember that the patient is the only involuntary partner in this process. There can also be significant psychological pressure on patient representatives who feel responsibility to effect change on behalf of their community.

**Barriers to patient participation**

Several factors can make patients reluctant to participate in engagement activities:

- Lack of confidence that they have something useful to say.
- Complex management regimes for their condition that they are reluctant to disrupt.
- Travel challenges, including exhaustion and recovery time needed.
- Uncertainty about whether support systems will transfer to new locations.
- Need to take time off work or pay for carers.
- For clinical trials, concerns about travel, accommodation costs, and healthcare access.
- Limited personal time that they may prefer to spend on other activities.

For patient organisations, participation in engagement activities competes with helping their members with immediate needs such as respite care, education, health care plans, and peer-to-peer support. They need to consider what other activities might need to be sacrificed to participate. Additionally, for rare diseases, this may be the first time they've been asked to participate in such activities, so they need clear information about what is involved, the expected commitment, and how they can deliver it sustainably.

**Solutions and recommendations**

Several approaches could improve patient engagement:

- Increase capacity and competence of small patient organisations through training and support.
- Provide support similar to the 'McKenzie friend' concept in the UK legal system – someone who sits beside the patient representative, helping them represent themselves.
- Be clear about expectations from patient organisations, recognising their varying levels of resource and maturity.
- De-risk the process through co-creation and shared decision-making tools.
- Provide sustainable funding – perhaps through a percentage levy on drug prices to create a trust fund, or an element in fees paid to medicines agencies.
- Improve data sharing globally to reduce duplication and improve efficiency.

**Conclusion**

Patient organisations, particularly those representing rare diseases, face significant challenges in engaging with development, regulatory and HTA processes. While there are exciting possibilities for developing disease-modifying interventions, the opportunity for small patient organisations to engage is limited by practical, financial, and psychological factors. Addressing these challenges through support, clear expectations, co-creation, sustainable funding, and better knowledge sharing would enable more effective patient engagement and ultimately improve outcomes for patients and families.

## Panel discussion: Future thinking on PE and PED - How should these evolve?

**Company perspective: Gonzalo Linares,** Global Head, R&D Patient Advocacy, Johnson & Johnson, Switzerland

**Current status of patient engagement**

While the pharmaceutical industry is moving forward with patient engagement and advocacy, it has not yet reached its full potential.

A recent study presented at the Chief Patient Officer Summit revealed that 80-90% of stakeholders, including patient organisations and pharmaceutical companies, agreed that engagement with patients and patient leaders in R&D is essential. However, only 25% of pharmaceutical companies were actually using these patient insights from patient leaders.

Johnson & Johnson created a dedicated patient advocacy team in 2024 to engage with patients and patient leaders in a more strategic, consistent way throughout the product lifecycle. This approach is not happening across the pharmaceutical industry overall, though there are good examples and practices. The challenge for large companies is doing this at scale, with hundreds of clinical trials running simultaneously.

**Guidance and best practices**

The increasing guidance from regulators and HTA bodies is welcomed. Industry needs to hear more about best practices, what works well, and what doesn't. Continuing dialogue is essential to learn what matters to different stakeholders.

There has been a shift from the approach of doing things "all with patients for patients" to being more selective, recognising the limited resources of patients and patient leaders. It's important to be mindful that patients and patient leaders usually don't have day jobs working with regulators, HTA bodies, and companies.

**Challenges and opportunities**

Pharmaceutical companies face two key challenges in improving patient engagement:

- **Culture transformation:** Many companies are not yet fully committed to involving and engaging with patients in a systematic way. This cultural shift has real impact on conversations and decisions.
- **Process integration:** Unless patient engagement is embedded in the processes and governance of companies, efforts will not be successful. Without this integration, patient engagement initiatives risk being overlooked.

**Conclusion**

There is clear alignment on why patient engagement matters, but more work is needed on how to implement it effectively. The focus should be on determining what matters to different stakeholders and bringing patient engagement and patient experience data to reality. Continued collaboration and sharing of best practices will help move the industry forward in this important area.

## Panel discussion: Future thinking on PE and PED - How should these evolve?

**Regulatory perspective: Juan García Burgos,** Head of Public and Stakeholders Engagement Department, European Medicines Agency

**Cultural change needed**

While stakeholders have aligned on principles of patient engagement (PE), despite years of discussion, real-life implementation has been limited. Medicine regulation is a relatively young discipline in Europe, perhaps 60-70 years old, and has been built without patient involvement. The system was designed to meet requirements assumed to be what patients need, rather than incorporating patients from the beginning.

Implementing PE represents a cultural change that challenges the system itself. The EMA's experience developing the reflection paper on patient experience data (PED) demonstrated this challenge—it took two years of discussions among experts from different disciplines to align everyone and ensure a common understanding. This process of changing culture and aligning minds takes significant time and should not be underestimated.

**Implementation strategies**

To facilitate implementation of PE and use of PED, changes to EMA's processes are underway:

- Templates for assessment reports have been modified
- The electronic Common Technical Document (eCTD) is incorporating a section for PED
- Plans to change the template for scientific advice to incorporate specific questions on PED.

**Future opportunities**

The changing landscape of medicines development and regulation presents opportunities for greater patient involvement. The introduction of real-world evidence and data from various sources, including social media, currently poses methodological challenges but may eventually facilitate change and increase the use of PED.

**Conclusion**

While there is strong agreement on the importance of PE, implementing meaningful change requires time, cultural shifts, and process modifications. The EMA is taking steps to facilitate this transformation through documentation changes and the reflection paper, but stakeholders should be realistic about the pace of progress while continuing to work toward greater patient involvement in regulatory processes.

## Panel discussion: Future thinking on PE and PED - How should these evolve?

**HTA agency perspective: Michelle Gibbens,** Director, Engagement, Strategic Relationships and Initiatives Business Unit, Canada's Drug Agency (CDA-AMC)

**Importance of language**

Common terminology is key when discussing patient engagement (PE) and patient experience data (PED). There has been evolution in understanding what is meant by PE and PED, and the connections between the two. PE should be viewed from a long-term relationship partnership focus, with PED collection and use happening within that context.

**Purpose-driven engagement**

A key consideration is determining when different types of engagement are needed. When is engagement sufficient to ensure patient perspectives are considered appropriately, and when is data needed to make a meaningful difference? More work is needed to think through these questions.

It is also important to consider what engagement requires from patients, and the challenges they face when participating. This raises questions about how to make engagement more patient-centric and less duplicative across organisations.

**Systems perspective**

Taking a systems perspective means thinking about shared objectives—providing access to high-value medicines for patients who need them—rather than focusing on individual organisational needs. This approach requires greater efficiency and collaboration.

Pre-market disease-level information could potentially be used by multiple stakeholders, minimising demands on patients and patient organisations. While this may seem daunting, it's important to explore opportunities for regulators and HTA agencies to collaborat

**Evolving through learning**

Rather than just relying on case studies of what has happened in the past, there's value in trying new approaches through pilots or test-and-learn initiatives. This experimental approach can help all stakeholders evolve their practices with patients at the centre.

**Conclusion**

A more patient-centric, systems-oriented approach to engagement is needed, focusing on shared objectives rather than individual organisational requirements. Language matters in how these discussions are framed, and there are opportunities to collaborate across stakeholders to reduce burden on patients while ensuring their perspectives are meaningfully incorporated. Moving forward will require both learning from past experiences and testing new approaches through pilots and collaborative initiatives.

## Panel discussion: Future thinking on PE and PED - How should these evolve?

**Payer perspective: Dr Michael Ermisch,** Head, AMNOG G-BA Department, GKV-Spitzenverband, Germany

**Patient engagement in German HTA**

The German legal framework clearly specifies how patients must be included in the HTA assessment process. Patients are present during decision making in all of G-BA's HTA procedures. Trained staff recruit patients from organisations with knowledge of or experience with the disease in question, and help them bring their voices into the process.

Patients participate when PICOS (Population, Intervention, Comparator, Outcomes, Study design) are decided and during appraisals. IQWiG, the scientific body conducting assessments, asks patients for input on the disease and on weighing evidence, though not directly on the dossier itself.

From the payer perspective, patient engagement in German HTA is relatively mature. Since the basis of HTA is patient-relevant outcomes, the focus is on determining what outcomes are relevant to patients and how relevant they are.

**Limitations and future directions**

While the current system is considered relatively advanced, there are limitations to what can be done. Patients voting in committees would not fit the German HTA system, where doctors and insurance committees negotiate how the decision framework is set. Additionally, not every patient suggestion will be incorporated, though all will be considered—there is an important distinction between consideration and adoption.

The plans for integrating more patient experience into European Public Assessment Reports (EPARs) will be valuable for German HTA, as these reports are carefully reviewed to understand what has been said about the disease and current treatment in Europe, for comparison with input from German clinicians.

**Methodological requirements**

The principle that "what can be measured should be measured" drives reliance on patient-reported outcomes (PROs). Patient experience data needs to meet methodological standards—it must be sensitive, robust, reproducible, and address potential biases in both the data and its representatives.

It's important to recognise that patients have legitimate agendas that may change depending on the stage of the process. During early scientific advice, patients may focus on relevant outcomes, while during decision making, their focus may shift to ensuring access, particularly in jurisdictions where access is in question.

**Conclusion**

The German HTA system has established clear processes for patient engagement, with patients participating in key decision points for new active substances. While there are limitations to how patient input can be incorporated, the system recognises the importance of patient-relevant outcomes and considers patient perspectives. Future developments, including enhanced patient experience information in EPARs, will provide additional valuable input for decision-making processes. The evolution will continue slowly but surely, with more information becoming available to inform these processes over the next five years.

## Panel discussion: Future thinking on PE and PED - How should these evolve?

**Policy perspective: Dr June Cha,** Head of Policy, Milken Institute, USA

**US payer environment**

The US healthcare system differs significantly from others in terms of the HTA and payer environment. Approximately 50% of insurance is covered by public insurers such as Medicare, Medicaid and Healthcare Marketplace, with the other 50% provided by private and other sources. This creates a fragmented, yet dynamic payer environment. Additionally, payers conduct their own HTA within this complex environment, making it challenging for patients to engage with them.

**Conducting value assessment**

In the US system, discussions tend to focus on price and reimbursement rather than the value of medical products. This may be because payers also conduct HTA, linking directly to cost and economic impact rather than value. A paradigm shift is needed to gain public support for transformative medical products and precision medicine. Patient engagement (PE) and inclusion of patient experience data (PED) in value assessment is critical for sustaining continuous innovation and ensuring public acceptance of new medical products.

**Legal barriers and potential solutions**

In the US, industry is prevented from talking to payers before FDA approval due to complex legislative barriers. The Pharmaceutical Information Exchange Act of 2022 has enabled industry to share some scientific information before FDA approval, but this is limited in scope and engagement.

A potential solution under consideration is to create a structure within the Centers for Medicare & Medicaid Services (CMS) and other payers similar to the FDA Patient-Focused Drug Development programme. This structure would have an appropriate budget and mandate to consider what kind of PED would be meaningful for payers.

**Validation of PED**

For PED to be truly valuable, it needs scientific research and validation from regulators, HTA agencies and payers. This requires designated structures and research frameworks to enhance the inclusion of patient voices in value assessment and coverage determination of medical products.

**Conclusion**

The US healthcare system faces unique challenges in incorporating patient voices into value assessment due to its fragmented payer environment and less than ideal patient involvement in their decision making. Creating dedicated structures and frameworks within payers for PE and developing scientific approaches to PED could help address these challenges. As precision medicine advances, ensuring that the entire healthcare system is ready to appropriately value these innovations becomes increasingly important, with PE playing a critical role in this process.

# Conclusion

The workshop demonstrated clear consensus that patient engagement (PE) and robust patient experience data (PED) are increasingly valued by regulators and HTA agencies, yet their use remains inconsistent and not always visible. Despite progress, stakeholders noted persistent challenges facing PE and PED, including difficulty quantifying their impact, fragmented approaches across jurisdictions, lack of methodological clarity, inconsistent quality and representativeness of data, and limited transparency about how patient input influences final decisions. Nevertheless, the workshop reinforced a shared commitment to strengthening patient voice throughout the medicine lifecycle.

The discussions emphasised the importance of early, purpose-driven PE in ensuring that patient perspectives shape research questions, evidence generated (including PED), and the outcomes assessed. Embedding PE and PED at the disease level rather than solely per asset was suggested to reduce duplication and improve efficiency. Agency and company case studies showed that patient input can be highly impactful when linked directly to decision-making frameworks—whether through PRO and endpoint selection, preference studies, qualitative insights, or structured committee contributions. However, clearer expectations from regulators and HTA agencies are needed to incentivise investment in PED generation and reduce uncertainty about its evidentiary weight.

A recurring theme was the need to improve the visibility and transparency of how PE and PED are used in regulatory and HTA assessments and decisions. Current feedback to patients is often limited, risking perceptions of tokenism. Harmonised terminology, structured reporting tools such as standardised PED tables, clearer rationales for accepting or excluding patient evidence, and more accessible public-facing summaries were seen as key steps toward building trust and demonstrating meaningful use of patient insights by agencies. Enhancing transparency is also essential to support internal decision making within companies, empower patient organisations, and provide assurance to communities that their input matters and is valued.

Looking ahead, participants agreed that progress requires cultural change, stronger infrastructures, and sustained cross-stakeholder collaboration. Increased alignment between regulatory and HTA agencies was viewed as important for reducing duplication, supporting consistent expectations, and easing the burden on patient organisations. Support for patient groups, including capacity-building, sustainable funding, and more flexible conflict-of-interest policies, will be necessary to ensure diverse, representative patient voices can contribute effectively. Ultimately, the workshop concluded that the field must now move from *why* PE and PED matter to *how* to embed them systematically and transparently in evidence generation and decision making.

# List of attendees

*Affiliations are stated as they were at the time of the meeting.*

| Pharmaceutical companies | | |
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CIRS Centre for Innovation in Regulatory Science

**About CIRS**

The Centre for Innovation in Regulatory Science is a neutral, independent UK-based subsidiary of Clarivate plc. Its mission is to maintain a leadership role in identifying and applying scientific principles for the purpose of advancing regulatory and health technology assessment (HTA) policies and processes. CIRS provides an international forum for industry, regulators, HTA bodies and other healthcare stakeholders to meet, debate and develop regulatory and reimbursement policy. It is governed and operated by Clarivate for the sole support of its members' activities. The organisation has its own dedicated management and advisory boards, and its funding is derived from membership dues, related activities, and grants.

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