WORKSHOP SYNOPSIS

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

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

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.

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 1–2 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.

To guide these discussions, the workshop focused on four key 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.

Workshop format

This multi-stakeholder workshop consisted of a series of plenary sessions (see programme), featuring presentations and panel discussions. In addition, there were four parallel breakout discussions, guided by questions prepared by CIRS. These aimed to generate actionable recommendations to address policy barriers to the meaningful use of PE and PED in regulatory and HTA decision making.

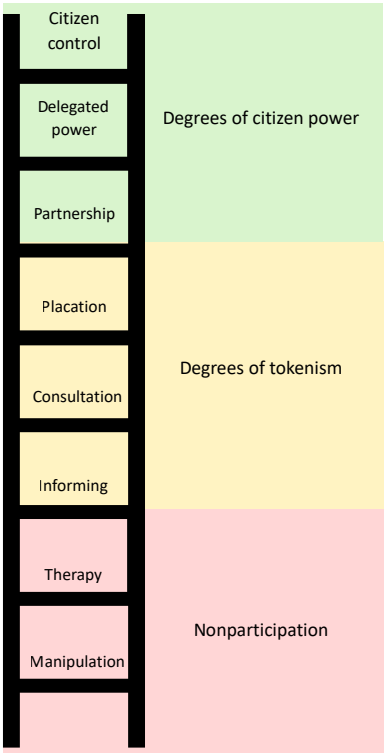
Definitions – adapted from the US Food and Drug Administration (FDA)

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.

Key points from plenary sessions

Embedding patient involvement into early development to enable downstream decision making

Arnstein's Ladder of Participation



The workshop began with an introduction to meaningful PE, which 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. 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 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 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

Session 1: Embedding PE into early development	Session 2: Patient input into regulatory/HTA decision processes
Chair: Niklas Hedberg, Chief Pharmacist, TLV, Sweden	**Chair: Niklas Hedberg,** Chief Pharmacist, TLV, Sweden
François Houÿez, Director of Treatment Information and Access, EURORDIS – Rare Diseases Europe **Dr Martina Garau**, Director, Office of Health Economics (OHE), UK **Kate Trenam**, Head of Patient Engagement and Advocacy - Neurodegeneration / Neuroinflammation, UCB, UK **Julian Beach**, Executive Director, Healthcare Quality and Access, Medicines and Healthcare Products Regulatory Agency (MHRA), UK	**Hayley Chapman,** Senior Program Director, The Synergist **Andrew Mitchell**, Honorary Professor, The Australian National University, Australia **Dr Juan García Burgos,** Head of Public and Stakeholders Engagement Department, European Medicines Agency (EMA) **Dr Pauline McGuire,** Principal Pharmacist, Scottish Medicines Consortium, UK **Dr Wim Goettsch,** Special Advisor HTA, ZIN, The Netherlands **Robyn von Malzahn**, Global Head, Patience Centered Outcomes, GlaxoSmithKline, UK **Dr Alice Biggane**, Associate Director – Outcomes Innovation and Research, Pfizer, UK
Session 3: Visibility of patient input in regulatory/HTA decisions	**Session 4: Breakout discussions**
Chair: Amelia Hursey, Strategic Director, Parkinson's Europe	**Chair: Prof John Skerritt,** Enterprise Professor in Health Research, University of Melbourne, Australia
Mencia de Lemus, Co-Chair Treatment Committee, SMA Europe **Dr Neil McAuslane**, Scientific Director, CIRS **Dr Juan García Burgos**, Head of Public and Stakeholders Engagement Department, EMA **Laura Norburn**, Senior Operations Manager - People and Communities Team, National Institute for Health and Care Excellence (NICE), UK **Robyn Bent**, Director of the Patient Focused Drug Development Program, Center for Drug Evaluation and Research, Food and Drug Administration (FDA), USA **Michelle Gibbens**, Director, Engagement, Strategic Relationships and Initiatives Business Unit, Canada's Drug Agency **Amaia Clemente**, Regulatory Science & Policy Associate Director Europe, Sanofi, Spain **Marina Richardson**, Associate Director, HTA Methods and Health Economics, Institute for Clinical and Economic Review (ICER), USA	**A) Creating supportive policies for meaningful PE and PED** **Chair: Dr Nick Crabb,** Chief Scientific Officer, NICE, UK **Rapporteur: Sunera Awan,** Head UK&I Regulatory Affairs, Bayer **B) Showing the impact of PE and PED in public-facing regulatory and HTA documents** **B1 Chair: Kelly Robinson,** Director General, Pharmaceutical Drugs Directorate, Health Canada **B1 Rapporteur: Jessica Abel,** Director, PED Policy & Best Practices, Patient-Centered Outcomes Research, AbbVie, USA **B2 Chair: Prof Hans-Georg Eichler,** Consulting Physician, Association of Austrian Social Insurance Institutions **B2 Rapporteur: Dr Siobhan Connor-Ahmad,** Principal Scientist, Patient-Centred Outcomes Research, Roche, UK **C) Aligning patient involvement across regulatory and HTA agencies** **Chair: Dr Anke-Peggy Holtorf,** Founder and Managing Director, Health Outcome Strategies, Switzerland **Rapporteur: Giorgia Rauso,** Associate Director, International Patient Advocacy, Regeneron, Italy
Session 5: Panel discussion on patient organisation support	**Session 6: Future thinking for PE and PED**
Chair: Prof John Skerritt, Enterprise Professor in Health Research, University of Melbourne, Australia	**Chair: Dr Brian O'Rourke,** Chair, CIRS HTA Steering Committee
Josephine Mosset, Policy Officer, Cancer Patients Europe **Dr Siobhan Connor-Ahmad,** Principal Scientist, Patient-Centred Outcomes Research, Roche, UK **Dr Fokaline Vroom,** Medicines Evaluation Board, The Netherlands **Dr Anja Schiel,** Senior Adviser; Lead Methodologist in Regulatory and Pharmacoeconomic Statistics, Norwegian Medical Products Agency (NOMA), Norway	**Dr Thomas Butt,** Executive Director, Health Economics and Outcomes Research, Biomarin, UK **Dr Brett Hauber,** Patient Preference Evidence Integration Lead, Pfizer, USA **Dr June Cha**, Director, Policy, Milken Institute, USA **Alastair Kent**, Chair of Trustees, Gene People, UK **Dr Juan García Burgos**, Head of Public and Stakeholders Engagement Department, EMA **Michelle Gibbens**, Director, Engagement, Strategic Relationships and Initiatives Business Unit, Canada's Drug Agency **Michael Ermisch**, Head, AMNOG G-BA department, GKV-Spitzenverband, Germany **Gonzalo Linares**, Global Head, R&D Patient Advocacy, Johnson & Johnson, Switzerland

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.

Workshop organised by

Dr Magda Bujar, Associate Director, Regulatory Programme and Strategic Partnerships, CIRS

Gill Hepton, Administrator, CIRS

Dr Neil McAuslane, Scientific Director, CIRS

Dr Jenny Sharpe, Communications Manager, CIRS

Anna Somuyiwa, Head, CIRS

Dr Tina Wang, Associate Director, HTA Programme and Strategic Partnerships, CIRS

Synopsis prepared by

Dr Jenny Sharpe, Communications Manager, CIRS

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Keep in touch

Centre for Innovation in Regulatory Science (CIRS)

70 St Mary Axe, London EC3A 8BE, UK

Email: cirs@cirsci.org

Website: www.cirsci.org