WORKSHOP SYNOPSIS

Building Capacity for Patient Involvement in Regulatory and HTA Decision Making in the Asia-Pacific: Focus on Rare Diseases and Precision Medicine

4-5 March 2026, Intercontinental Hotel, Kuala Lumpur, Malaysia

CIRS Centre for Innovation in Regulatory Science

DukeNUS Medical School

Centre of Regulatory Excellence

GRAPHIC SUMMARY

Building Capacity for Patient Involvement in Regulatory and HTA Decision Making in the Asia-Pacific: Focus on Rare Diseases and Precision Medicine

Insights from a multi-stakeholder workshop

Across the Asia-Pacific, patient involvement in regulatory and HTA processes is expanding, but approaches vary. Rare diseases and precision medicines amplify evidence uncertainty, highlighting the need for structured, meaningful patient involvement.

Key themes of discussion

Patients as knowledge holders

Patient perspectives help define unmet need, meaningful outcomes and real-world burden, especially when clinical evidence is limited, such as in rare diseases.

Ensuring that engagement is early helps to capture patient insights effectively.

Integrating engagement into evidence

Patient involvement is evolving from ad hoc consultation toward more systematic generation and use of patient experience data.

As this evolves, being clear about the purpose of patient involvement is important for setting expectations and supporting meaningful participation.

Transparency builds trust

Clear communication about how patient input is considered in decisions – including its role, influence and limitations – is central to building trust and sustaining patient involvement.

Building capability across the region

Experience levels, infrastructure and resources vary widely across the Asia-Pacific.

Strengthening patient involvement requires capability building for agencies and patient organisations, alongside regional collaboration to share learning and reduce duplication.

Looking ahead

Continued progress depends on transparency, investment in capability, and collaboration across regulators, HTA bodies, industry and patient organisations in the Asia-Pacific to ensure patient perspectives meaningfully inform decision making.

Background

Integrating patient perspectives into the development, regulation, and health technology assessment (HTA) of new medicines is increasingly vital to ensuring decisions reflect patient needs and values. In October 2025, CIRS convened a multi-stakeholder workshop that emphasised the need for supportive policies, greater transparency in decision making, and alignment between regulatory and HTA bodies to embed patient engagement (PE) and patient experience data (PED) effectively.

While healthcare systems in the Asia-Pacific are rapidly evolving, the use of PE and PED in regulatory and HTA processes remains nascent in many countries. Challenges persist such as limited infrastructure, fragmented data sources, small patient populations, cultural and linguistic barriers, and operational complexity in multi-stakeholder collaboration. Addressing these issues is essential to ensure meaningful PE and PED and equitable access to innovative therapies.

The growth of rare disease and precision medicines has emphasised the need for integrated PE and PED in regulatory and HTA decision making. In such cases, patient and caregiver experiences provide critical context to understand unmet need, acceptability of treatment options, and the real-world implications of disease burden.

Recognising these developments, CIRS, in collaboration with the Duke NUS Centre of Regulatory Excellence (CoRE) in Singapore, convened this workshop to bring together regulators, HTA agencies, patient organisations, academics, and industry representatives from across the Asia-Pacific.

Workshop objectives

- **Identify current practices and challenges** in patient involvement across Asia-Pacific HTA and regulatory systems.
- **Explore strategies for building capacity** among patient organisations and agencies to incorporate PED in regulatory and HTA processes.
- **Examine the role of PE and PED** in the evaluation of **rare diseases and precision medicines**.
- **Develop practical recommendations** to support sustainable and meaningful patient involvement aligned with local health system priorities and economic contexts.

Workshop format

This multi-stakeholder workshop followed a programme of plenary presentations, panel discussions, case studies, and breakout sessions designed to facilitate open dialogue and actionable recommendations (see programme).

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

Patient involvement landscape

The workshop began with an exploration of how PE and PED are shaping regulatory and HTA decision making globally and in the Asia-Pacific region.

Despite progress in co-design initiatives, capacity-building efforts and increasing use of structured PED methods, several challenges remain globally. These include under-investment in research capturing patient perspectives, limited retention and reuse of patient-generated insights, and inadequate documentation of patient involvement and its impact. Meaningful patient involvement – without the risk of tokenism – requires clear goals, methodological rigor and explicit demonstration of how patient input influences decision making.

Focusing on the Asia-Pacific, a systematic literature review of patient involvement in HTA value assessment frameworks revealed a heterogenous landscape. While some jurisdictions have more established, structured approaches, others are at earlier stages of implementation. Across the region, governance-level patient involvement remains limited, and diversity strategies are still emerging. As patient involvement continues to evolve, structured feedback loops and capacity-building mechanisms are needed to support more equitable and consistent practices.

Many regulatory agencies in the Asia Pacific are adopting reliance approaches, expedited pathways, and other regulatory tools to improve timely access to innovative therapies. As these approaches advance, there is growing recognition that patient perspectives should be incorporated into evaluation and approval processes in a more structured and consistent way. For example, in Malaysia, while patient voices are increasingly heard at different levels of the healthcare system, PE and PED have not yet been systematically integrated into regulatory decision making.

Systemic and cultural factors—such as hierarchical communication norms and limited awareness of regulatory and HTA processes —can hinder collaborative engagement with patients in the Asia-Pacific. As well as building methodological expertise, academic institutions may have a role in providing a neutral platform to convene regulators, HTA bodies, patient groups and industry. Patient involvement should be grounded in transparency, clarity of expectations and early relationship-building.

Patient involvement in HTA

This session examined how patient input is being operationalised within HTA agencies across the Asia-Pacific region.

For example, a patient representative from Chinese Taipei shared experience of the jurisdictional HTA framework, which integrates patient input through a patient opinion sharing platform and patient representatives on its reimbursement committee. The establishment of a Cancer Drug Fund and expansion of risk-sharing models signals a strategy to support earlier access to treatments with uncertain evidence while incorporating patient perspectives.

From the **patient perspective**, there needs to be greater recognition that HTA is helping to pay for health outcomes, not just the medicines themselves. HTA can be viewed as a bridge that aligns expectations of different stakeholders through collaboration; it is not about winning for one group but about building a win-win partnership among policy makers, healthcare providers, industry and patients.

From the **industry perspective**, there has been a positive shift from broad data collection to curation of evidence – including co-created patient evidence – demonstrating value. However, formal integration of patient voices into HTA decision making remains limited across the Asia-Pacific. To advance patient-centred HTA processes, there needs to be consideration of the full spectrum of healthcare impacts, from individual patient experiences to broader economic and social benefits.

For the **Philippines' HTA Council**, patient involvement operates across three levels: informative (collecting or receiving information), consultative (e.g. PICO validation and public review of draft assessments) and collaborative (e.g. co-developed capacity-building activities with patient organisations). Patient perspectives are incorporated into the methodological framework through an impact assessment that examines equity considerations, patient rights, patient preferences and service delivery. Current priorities include developing rare disease assessment methods, establishing a dedicated engagement unit and enhancing support for patient training programmes.

The **Malaysian Heath Technology Assessment Section (MaHTAS)** involves patient representatives in prioritisation, expert committees, and the national HTA council. Resource constraints, representativeness gaps and the need for more robust PED collection remain key challenges. Future plans include outreach to under-represented communities and guidance on patient involvement.

For **Singapore's Agency for Care Effectiveness (ACE)**, patients are involved in topic prioritisation, technical evaluations and co-developing educational resources. Over 80% of HTAs between 2022-2024 incorporated patient input, drawn from tailored surveys co-developed with patient organisations. ACE includes patient-submitted lived-experience data in its evaluation reports, publishes plain-language summaries and maintains feedback loops with patients to ensure transparency. Patients can suggest topics for evaluation for inclusion in the Rare Disease Fund, a national charity fund that provides long-term financial support to patients with ultra-rare diseases requiring high-cost treatments.

Australia's Pharmaceutical Benefits Advisory Committee (PBAC) and **Medical Services Advisory Committee (MSAC)** consider patient input from two sources: the application dossier and public consultation processes. Public consultation submissions allow individuals, advocacy organisations and carers to articulate lived-experience evidence, helping committees contextualise clinical and economic data. Following the Australian HTA Policy and Methods Review, an HTA stakeholder engagement framework with a focus on improving consumer and patient engagement is being developed.

Regulatory practices of patient involvement

This session transitioned from HTA to regulatory processes, addressing how regulatory agencies across the region integrate patient insights into their decision making.

From the **patient perspective**, patient experience serves as a powerful data source that, when properly structured and analysed, can significantly influence policy development and regulatory decisions. In contexts where data is limited, populations are small, and uncertainty exists—particularly in rare diseases—patient input is essential. While regulators must ensure early, meaningful patient involvement supported by feedback mechanisms, patient organisations should work on building capacity and diversifying representation.

Industry is increasingly committed to providing high-quality PED to regulatory agencies, which is becoming a growing expectation worldwide. The Asia-Pacific region is well-positioned to harmonise PED approaches by leveraging existing global guidance whilst tailoring frameworks to specific country needs. Through collaboration, data sharing, and mutual trust between stakeholders, the region can ensure patients receive optimal treatment outcomes without necessitating complete reinvention of established methodologies.

The **Japanese Pharmaceuticals and Medical Devices Agency (PMDA)** seeks to achieve four 'firsts', with the first concept being 'Patient first'. Patient involvement at PMDA spans patient participation in advisory committees, structured mechanisms to gather patient views, and use of patient-reported outcomes (PROs) to support evaluation. Key principles for working with patients include common understanding, interactive engagement, capability development and continuity.

For the **Indonesian Food and Drug Authority**, patient involvement is integrated into the regulatory framework through public consultations, clinical trial oversight, marketing authorisation evaluations, and post-marketing pharmacovigilance. Patient risk tolerance and lived experiences are essential components of the regulatory assessment. A key challenge is the lack of patient and public awareness of involvement channels for drug evaluation processes.

Regulatory decision making by the **Australian Therapeutic Goods Administration (TGA)** seeks to incorporate patient and community input at all levels: individual decision making and advisory input, development of broad regulatory policy, community engagement initiatives, and specific sectoral engagement, for example, in relation to women's health. Recognition that patients experience the health system as a whole, regardless of regulatory boundaries, is key. While this can make patient engagement challenging, it can also provide additional value by encouraging regulators to consider the broader impacts of regulatory activities.

Global best practices for PE and PED

This session brought global perspectives on how PE and PED frameworks are evolving internationally.

A landscape study coordinated by **Patient Focused Medicines Development (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. However, inconsistent terminology, incomplete feedback loops, and infrastructure issues remain major barriers.

HTAi's Patient and Citizen Involvement Interest Group is conducting a landscape analysis to document patient involvement practices in HTA in the Asia-Pacific and develop recommendations for strengthening patient involvement in the region. Stakeholder workshops conducted in 2025 highlighted key enablers — such as recognition of patients as experts, transparent processes, early involvement and co-design — and persistent barriers including limited awareness, resource constraints, language and cultural diversity, and hierarchical institutional norms.

The **National Institute for Health and Care Excellence (NICE)** in the UK operationalises patient involvement within its technology evaluations through lay committee members, patient evidence submissions and communication tools such as the Summary of Information for Patients (SIP). Case studies have illustrated how patient insights influence clinical interpretation, quality-of-life assessment and final recommendations, especially where evidence is sparse. To facilitate continuous refinement, NICE sends feedback surveys to patients involved in NICE processes. organisations when guidance is published, patient experts after they have attended committee meetings, and lay members when they leave committees.

The **European Medicines Agency (EMA)** has a longstanding framework for patient involvement, including participation in scientific advice, committee deliberations, guideline development, and public hearings. Overarching principles for engagement include transparency, independence and broad representativeness. Ongoing work includes finalising the PED Reflection Paper and collaboration through the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) on the ICH E22 Guideline on patient preference studies.

During the multi-stakeholder panel discussion, it was highlighted that good PE and PED practice requires both agencies and patient organisations to have adequate capability and resources to engage effectively. Regulatory and HTA agencies should aim to clearly communicate the role and weight of patient contributions using structured and consistent processes. Including patient experience in regulatory labeling would help to validate patient input and facilitate shared physician-patient decision making.

Supporting patients and patient organisations to share their perspectives

On the second day of the workshop, a multi-stakeholder panel reflected on the challenges patients and patient organisations face to provide their perspectives and collaborate with other stakeholders.

Patient organisations have limited resources and are challenged with responding to multiple, uncoordinated engagement requests. Engaging volunteers and building talent pipelines requires a culture of celebrating small wins and developing leadership capabilities. Funding is also essential to support sustainable patient involvement, though careful consideration must be given to funding sources and potential conflicts of interest.

In addition, regulatory and HTA agencies can lack sufficient resources, trained personnel and formal mechanisms for meaningful PE. Establishing structured channels for patient groups to communicate concerns and including patient involvement formally in policies and guidelines is key.

In multinational companies, PE is often concentrated in the US, limiting engagement in Asia and other regions. As PE is usually not a priority compared to other areas (e.g. pricing, AI, supply chain), it can be challenging to obtain internal buy-in to expand PE efforts. However, there may be opportunities to do so in the rare disease area, where global populations are more important.

Shaping the future of PE and PED

The final multi-stakeholder panel of the workshop focused on the evolution of PE and PED in regulatory and HTA decision making across the Asia-Pacific region.

Patients are shifting their expectations from mere “seat at the table” presence to meaningful, demonstrable impact. This evolution relies on agencies taking accountability, being transparent and ensuring traceability on how patient input actually influences decisions. In addition, patients are becoming more digitally competent, data-informed partners, with an intergenerational shift where younger patients focus on quality of life and productivity—not just survival.

Technology is enabling greater access and linking of PED, as demonstrated in Europe with the European Health Data Space and H2O (Health Outcomes Observatory) project. There may be opportunities to pursue similar data-sharing models in Asia, depending on countries’ legislation alignment and adoption of international standards and guidelines. It is important to consider accessibility when designing digital tools for collecting patient data.

Increasing opportunities for joint regulatory-HTA scientific advice across the Asia-Pacific region may help to shape more patient-centred clinical trials and prevent duplicative requests on patient organisations at a later stage. There could also be value in exploring pre-competitive disease-level (rather than product-level) dialogue, involving multiple companies, regulators, HTA agencies and patient organisations simultaneously.

To build sustainable frameworks for PE and PED across the Asia-Pacific, multi-stakeholder collaboration and knowledge sharing is key. When implementing global practices, it is important to recognise the importance of the regional healthcare context.

Recommendations from breakout discussions

Policy enablers to aid integration of PE and PED in decision making

- Establish a multi-stakeholder group to discuss use cases and usability of PED across the Asia-Pacific region.
  - Align with global guidelines, adapting them to the local context.
    - e.g. for regulatory: ICH E22 Guideline, EMA PED Reflection Paper, FDA Patient-Focused Drug Development guidance.
    - e.g. for HTA: global guidance should detail common aspects among markets and differences that should be considered.
  - Agree on important common endpoints across regulatory and HTA.
  - When reliance mechanisms are used for regulatory decision making, consider extrapolation of PED used by the reference agency (local PED may need to be generated to supplement foreign PED, based on local requirements).
- Engage with patient organisations to understand what PED is relevant according to therapeutic area and natural history.
  - e.g. PROs for rare diseases.
- Build capacity for PE/PED within patient organisations, equipping them with terminology and ways of working that increase credibility and facilitate effective dialogue.
  - Capacity-building activities could be funded by industry with measures (e.g. a third party) to reduce conflict of interest.

Assessing and communicating the impact of patient input

- Conduct a workshop to develop a regional consensus/white paper on a streamlined process for reporting impact of patient input.
- Establish a global platform for PE/PED communication and unification.
- CIRS should consider developing a PE/PED reporting template for regional/global adaptation.
- CoRE should consider conducting research on how PED can be adapted for country-specific clinical trial applications and new drug application requirements, while minimising cross-country gaps.

Building capacity and alignment on PE/PED

- Establish a formal multistakeholder coordination mechanism for regional PE collaboration.
  - Short term: Explore the creation of a formal coordination body, providing structured engagement platforms and communication channels.
  - Long term: Introduce formal mandates embedding PE into regulatory and HTA processes.
- Build cross-stakeholder capacity for meaningful PE and PED generation
  - Short term: Launch targeted training programmes and workshops.
  - Long term: Institutionalise capacity-building frameworks within agencies and stakeholder organisations.
- Develop patient data infrastructure to support evidence generation and decision making.
  - Short term: Pilot patient registries and develop standardised data collection frameworks.
  - Long term: Build integrated regional infrastructure for patient data collection, sharing, and analysis.

Workshop programme

Session 1: Why PE/PED are central to regulatory and HTA decision making for new medicines	Session 2: Focus on HTA agencies' decision processes - How are patient insights being integrated?
Chair: Prof John Lim, Executive Director, Centre of Regulatory Excellence (CoRE), Duke-NUS Medical School, Singapore	
Country Welcome: Mdm Wan Noraimi Wan Ibrahim, Director, National Pharmaceutical Regulatory Agency (NPRA), Malaysia, and **Dr Syaqirah Akmal**, Deputy Director, Medical Development Division, Head, Malaysian Heath Technology Assessment Section **Ann Single,** Health Technology Assessment International (HTAi) President, and CEO, Patient Voice Initiative, Australia **Dr Durhane Wong-Rieger,** President, Asia Pacific Alliance of Rare Disease Organisations (APARDO), and CEO, Canadian Organization for Rare Disorders **Dr Yoong Khean Khoo,** Assistant Professor, Duke-NUS Medical School Centre of Regulatory Excellence (CoRE), Singapore **Dr Arshiya Zaheer,** Regional Patient Engagement Lead, MSD, Singapore	**Hwan Ruey (Eric) Liu,** Secretary General, Taiwan Young Patient Association **Logan Caragata,** APAC Policy Head, Roche, Malaysia **Tisha Isabelle de Vergara,** Project Technical Specialist III, HTA Division, Department of Science and Technology, Philippines **Dr Roza Binti Sarimin,** Head of HTA Unit, Malaysian Heath Technology Assessment Section **Shawn Quek,** Senior Specialist (Consumer Engagement and Education), Agency for Care Effectiveness, Singapore **Hon Prof Andrew Mitchell,** Honorary Professor, Department of Health Economics Wellbeing and Society, The Australian National University, Australia
Session 3: Focus on regulatory agencies' decision processes - How are patient insights being integrated?	**Session 4: What best practices and considerations should guide sustainable implementation of PE in an agency?**
Chair: Dr Ratna Devi, Board Member, International Alliance of Patients' Organizations (IAPO)	
Chris Munoz, President, Philippine Alliance of Patient Organizations (PAPO) **Dr Svetlana Yanchuk,** Country President, AstraZeneca, Malaysia **Naoyuki Yasuda,** Special Advisor to the Chief Executive and Associate Executive Director for International Programs, Pharmaceuticals and Medical Devices Agency (PMDA), Japan **Prof Taruna Ikrar,** Chairperson, Indonesian Food and Drug Authority **Prof Anthony Lawler,** Deputy Secretary, Health Products Regulation Group, Australian Government Department of Health and Aged Care	**Hayley Chapman,** Executive Director - Operations, Patient Focused Medicines Development (PFMD), Canada **Fiona Pearce,** Co-Chair, HTAi Patient and Citizen Involvement in HTA Interest Group, and Senior Advisor, Agency for Care Effectiveness (ACE), Singapore **Prof Steffen Thirstrup,** Chief Medical Officer, European Medicines Agency (EMA) **Dr Nick Crabb,** Chief Scientific Officer, National Institute for Health and Care Excellence (NICE), UK **Chris Munoz,** President, Philippine Alliance of Patient Organizations (PAPO) **Dr Miyoung Choi,** Director, Clinical Evidence Research, National Evidence-based Healthcare Collaborating Agency (NECA), South Korea **Prof Anthony Lawler,** Deputy Secretary, Health Products Regulation Group, Australian Government Department of Health and Aged Care **Rob Berlin,** Head, Regulatory Policy, Vertex, USA
Session 5: Breakout discussions	**Session 6: PE/PED and capacity building - How should this evolve and what are the challenges?**
A) Policy enablers for integrating PE and PED in decision making **Chair: Prof Steffen Thirstrup,** Chief Medical Officer, EMA **Rapporteur: Stephanie Chen,** Associate Director, AP Regulatory Policy, Merck, Sharp & Dohme, Singapore **B) Assessing and communicating the impact of patient input** **Chair: Prof Manuel Espinoza,** Associate Professor, Division of Health Economics, Policy and Management, Hong Kong University **Rapporteur: Joanne Tan,** Senior Manager, Regulatory Affairs, APAC, BioMarin, Hong Kong **C) Building capacity and alignment on PE/PED** **Chair: Nidhi Swarup,** Chair, Alliance of Patients' Organizations Singapore (APOS) **Rapporteur: Mugdha Barik,** Senior Program Manager, Dakshayani and Amaravati Health and Education, India	**Chair: Prof John Skerritt,** Enterprise Professor in Health Research, University of Melbourne, Australia **Wee Seng Phua,** Executive Director and Chief Rare Advocate, Rare Disorders Society Singapore **Susumu Kitamura,** Head of GRA Japan, Sanofi, Japan **Dr Iris Conela Tagaro,** Medical Specialist III, Head of Clinical Research Section, Philippines FDA **Dr Phatcharee Chukaew,** Researcher, HITAP, Thailand **Nadiah Hanim,** President, Malaysia Rare Disorder Society **Dr Li-Ying (Grace) Huang,** Senior Director, Division of HTA, Center for Drug Evaluation, Chinese Taipei **Dr Nikki Kitikiti,** Vaccines External Engagement & Policy Lead, Takeda, Singapore **Dr Nick Crabb,** Chief Scientific Officer, NICE, UK **Prof Steffen Thirstrup**, Chief Medical Officer, EMA

About CIRS

The Centre for Innovation in Regulatory Science is a neutral, independent UK-based subsidiary of Clarivate plc. Its mission is to identify and apply scientific principles for the purpose of advancing regulatory and health technology assessment (HTA) policies and processes in developing and facilitating access to pharmaceutical products. 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.

About CoRE

The Centre of Regulatory Excellence (CoRE) was established in 2014 at the Duke-NUS Medical School to advance regulatory systems for health products and health services across the Asia Pacific. Guided by its mission, CoRE contributes to improving patient access to health products while enhancing regional health systems and health security. The centre adopts a multi-pronged approach through three key strategic areas: education and training, applied research, and policy innovation in an Asian context. As a trusted partner with a wide collaborative network comprising major international and regional regulatory authorities, organisations, industry, and academic institutions, CoRE plays a coordinating role and contributes actively to regional capacity building and broader systems strengthening initiatives.

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