WORKSHOP REPORT

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

Executive summary

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, the Centre for Innovation in Regulatory Science (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.

Asia's healthcare systems are rapidly evolving, driven by rising demand for innovative therapies and the need for timely access to high-quality medicines. National regulatory authorities are adopting digitalisation, regulatory reliance, and expedited pathways—such as South Korea's Global Innovative products on Fast Track (GIFT) programme and Japan's PMDA reforms—to modernise processes. However, many regulators still face challenges, including limited infrastructure, workforce capacity, and a lack of formal mandates for PE.

HTA is gaining prominence across Asia-Pacific, with jurisdictions like the Philippines, Singapore, Thailand, Taiwan, and South Korea leading the way. Yet, many others remain in early stages of development. PE in both regulatory and HTA processes remains nascent across the region, with few structured mechanisms to incorporate PED or lived experiences.

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, traditional evidence is often limited, and so patient and caregiver experiences provide critical context to understand unmet need, acceptability of treatment options, and the real-world implications of disease burden.

Regional organisations and initiatives, such as the Asia Pacific Alliance of Rare Disease Organisations (APARDO) and the Southeast Asia Rare Disease Summit, are helping to amplify patient voices and promote collaborative research and policy reform. In 2025 Malaysia launched its National Policy for Rare Diseases and held the South-East Asia Rare Disease Policy Forum, where Malaysia will be the lead to improve diagnosis, treatment and support through the South-East Asia Declaration and Action Plan on Rare Diseases.

The Centre of Regulatory Excellence (CoRE) at Duke-NUS Medical School, Singapore, also has initiatives to support capacity building that equips regulators and HTA bodies to better integrate PED. Through its Coalition to Accelerate Patient Engagement in Asia-Pacific (CAPE), CoRE fosters inclusive dialogue and policy innovation, working closely with regional partners, including those in the rare disease field.

Recognising these developments and challenges, 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 region. The purpose was to explore patient-centric practices, identify gaps, and foster collaborative strategies that strengthen inclusive, transparent, and evidence-based healthcare decision making in the region.

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.

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

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.

Key points from the plenary sessions

Patient involvement landscape

The workshop began with an exploration of how patient engagement (PE) and patient experience data (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 regulatory and HTA 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, patient voices are increasingly heard at different levels of the healthcare system, yet 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 and EU, 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 the 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 patient-centric 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 of activities, reducing duplication.
- 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

Day 1: 4th March 2026

Session 1: Why PE and PED are central to regulatory and HTA decision making	
09:00	**CIRS welcome, housekeeping and logistics of the day**
09:05	**Country welcome** **Mdm Wan Noraimi Wan Ibrahim,** Director, National Pharmaceutical Regulatory Agency, Malaysia & **Dr Syaqirah Akmal,** Deputy Director, Medical Development Division, Head, Malaysian Heath Technology Assessment Section
09:10	**Chair's welcome and introduction** **Prof John Lim,** Executive Director, Centre of Regulatory Excellence (CoRE), Duke-NUS Medical School, Singapore
09:20	**How is PE and PED shaping regulatory and HTA decision making around the world?** **Ann Single,** HTAi President, and CEO of Patient Voice Initiative in Australia
09:35	**PE in Asia: Current practices, challenges, and opportunities in regulatory and HTA decision making for rare and precision disease medicines** **Dr Durhane Wong-Rieger,** President of the Asia Pacific Alliance of Rare Disease Organisations (APARDO) and Canadian Organization for Rare Disorders
09:50	**Panel discussion – PE in the Asia Pacific: Practical insights on challenges and opportunities for precision medicines and patients with rare disease** **In addition to the above speakers – 5 mins commentary from their perspective** **Dr Yoong Khean Khoo,** Assistant Professor, Duke-NUS Medical School and Centre of Regulatory Excellence (CoRE), Singapore **Dr Arshiya Zaheer,** Regional Patient Engagement Lead, MSD, Singapore **Followed by open floor discussion**
10:30	**Break**
Session 2: Focus on HTA agencies' decision process: How HTA agencies integrate patient insights into decision making	
	Patient input into HTA agencies' decision processes: How are HTA agencies leveraging patient experience and industry-generated PED into the assessment and appraisal?
11:00	**Patient perspective – Hwan Ruey (Eric) Liu,** Secretary General, Taiwan Young Patient Association
11:10	**Company perspective – Logan Caragata,** APAC Policy Head, Roche, Malaysia
11:20	**Discussion**
	Case studies - How are agencies currently or planning to engage patients or utilise PED directly/indirectly during the assessment and appraisal process?
11:30	**Tisha Isabelle de Vergara,** Project Technical Specialist III, Policy Planning and Evaluation Unit, HTA Division, Department of Science and Technology, Philippines
11:40	**Dr Roza Binti Sarimin,** Head of HTA Unit, Malaysian Heath Technology Assessment Section

11:50	**Shawn Quek,** Senior Specialist (Consumer Engagement and Education), Agency for Care Effectiveness, Singapore.
12:00	**Hon Prof Andrew Mitchell,** Honorary Professor, Department of Health Economics Wellbeing and Society, The Australian National University, Australia
12:10	**Discussion**
12:30	**Lunch**
Session 3: Focus on regulatory agencies' decision process: How regulatory agencies integrate patient insights into decision making	
13:30	**Chair's introduction – Dr Ratna Devi,** Board Member, International Alliance of Patients' Organizations (IAPO)
13:40	**Patient input into regulatory agencies' decision processes: How are agencies leveraging patient experience and industry-generated evidence in evaluation and approval?** **Patient perspective – Chris Munoz,** President, Philippine Alliance of Patient Organizations (PAPO)
13:50	**Company perspective – Dr Svetlana Yanchuk,** Country President, AstraZeneca, Malaysia
14:00	**Discussion**
14:10	**Case studies: How are regulatory agencies currently or planning to engage patients or utilise PED directly/indirectly during the evaluation and approval process?** **Naoyuki Yasuda,** Special Advisor to the Chief Executive &Associate Executive Director for International Programs, PMDA, Japan
14:20	**Dra. Tri Asti Isnariani,** Director of Drug Registration, Indonesia Food and Drug Administration
14:30	**Prof Anthony Lawler**, Deputy Secretary, Health Products Regulation Group, Australian Government Department of Health and Aged Care
14:40	**Discussion**
14:55	**Break**
Session 4: What best practices and considerations should guide the practical and sustainable implementation of PE in an agency?	
15:30	**PE and PED in regulatory review and HTA: Where are we today? Landscape study** **Hayley Chapman,** Executive Director - Operations, Patient Focused Medicines Development, Canada
15:45	**Global vs Asia-Pacific findings from HTAi 360 studies** **Fiona Pearce,** Co-Chair of the HTAi Patient and Citizen Involvement in HTA Interest Group (PCIG) and Senior Advisor, Agency for Care Effectiveness (ACE), Singapore
16:00	**Patient voice in regulatory evaluations and lifecycle engagement** **Prof Steffen Thirstrup,** Chief Medical Officer, EMA
16:15	**Operationalising PE in HTA submissions** **Dr Nick Crabb,** Chief Scientific Officer, NICE, UK

16:30	**Discussion**
16:45	**Panel discussion** **Agencies need to ensure transparent communication on how patient input/experience data was considered and what impact/value it had on decision making. What does 'Good' look like for HTA and regulatory decisions?** **5-minute perspectives from panellists followed by open floor discussion** **Company perspective – Rob Berlin**, Head, Regulatory Policy, Vertex, USA **Patient perspective - Chris Munoz,** President, Philippine Alliance of Patient Organizations (PAPO) **HTA agency perspective – Dr Miyoung Choi,** Director of Clinical Evidence Research, Director of NECA GRADE Center, National Evidence-based Healthcare Collaborating Agency (NECA), South Korea **Regulatory agency perspective – Prof Anthony Lawler,** Deputy Secretary, Health Products Regulation Group, Australian Government Department of Health and Aged Care
17:45	**Introduction to breakout discussions**
18:00	**End of Day 1**
18:45	**Reception**
19:30	**Dinner**

Day 2: 5th March 2026

Session 5: Breakout discussions	
08:30	**Topic A: Policy enablers for PE/PED: What's needed in the Asia-Pacific vs globally to drive integration into company development and regulatory/HTA decision making?** **Chair: Prof Steffen Thirstrup,** Chief Medical Officer, EMA **Rapporteur: Stephanie Chen,** Associate Director, AP Regulatory Policy, MSD, Singapore **Topic B: Measuring impact: How can agencies assess and communicate the influence of patient input on decisions?** **Chair: Prof Manuel Espinoza,** Associate Professor, Division of Health Economics, Policy and Management, University of Hong Kong **Rapporteur: Joanne Tan,** Senior Manager, Regulatory Affairs, APAC, BioMarin, Hong Kong **Topic C: Building capacity and alignment: How can patient organisations and agencies strengthen capabilities for 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

11:30	**End of roundtable discussions**
12:00	**Lunch**
Session 6: PE and capacity building – How should this evolve and what are some of the challenges?	
13:00	**Chair's introduction – Prof John Skerritt,** Enterprise Professor in Health Research, University of Melbourne, Australia
13:05	Feedback by roundtable rapporteurs and discussion
13:50	**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?** **8 Minute perspective followed by open floor discussion** **Patient perspective - Wee Seng Phua,** Executive Director and Chief Rare Advocate, Rare Disorders Society Singapore **Company perspective – Susumu Kitamura,** Head of GRA Japan, Sanofi, Japan **Regulatory agency perspective** – **Dr Iris Conela Tagaro**, Medical Specialist III, Head of Clinical Research Section, Philippines FDA **HTA agency perspective** - Dr Phatcharee Chukaew, Researcher, Health Intervention and Technology Assessment Program (HITAP), Thailand
14:50	**Break**
15:05	**Forward looking panel discussion – The next five years: Shaping the future of PE and PED in regulatory and HTA decision making** **5 minutes for representative perspectives followed by open floor discussion** **Patient representative – Nadiah Hanim,** President, Malaysia Rare Disorder Society **HTA agency perspective - Dr Li-Ying (Grace) Huang,** Senior Director, Division of Health Technology Assessment, Taiwan Center for Drug Evaluation **Company perspective – Dr Nikki Kitikiti,** Vaccines External Engagement & Policy Lead, Takeda, Singapore **HTA agency perspective - Dr Nick Crabb** Chief Scientific Officer, NICE, UK **Regulatory agency perspective - Prof Steffen Thirstrup,** Chief Medical Officer, EMA
16:05	**Chair's summary**
16:10	**Close of workshop**

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: Why patient engagement and patient experience data are central to regulatory and HTA decision making

How are patient engagement and patient experience data shaping regulatory and HTA decision making around the world?

Ann Single, HTAi President, and CEO of Patient Voice Initiative, Australia

Patients as knowledge holders

Recent developments have provided improved frameworks for understanding patient expertise. The Dumez and L'Espérance classification system identifies six types of patient knowledge based on where patients learn: from themselves (embodied and monitoring knowledge), from the health system (medical and navigation knowledge), and from within communities of shared conditions (relational and cultural knowledge). This approach shifts focus from what clinical knowledge patients lack to what unique knowledge they possess that clinical knowledge cannot provide. This enables better recognition of patients as knowledge holders rather than merely interest holders.

Patient involvement in HTA

Over three decades, HTA bodies have documented substantial learning from patients across multiple domains: outcomes that matter to patients, appropriate comparators, treatment experiences, quality of life realities and unmet needs, trade-offs, care pathways and variation implications, non-health benefits and risks, and subgroups with additional needs/gaps.

Two complementary approaches have emerged: patient participation/engagement and patient-based evidence research. These approaches serve different purposes and require different methodological considerations, with research requiring robust data capture and analysis to meet the requirements of critical assessment, and engagement providing credible input through deliberation and two-way learning. Shared goals and setting should drive the approach to engagement, including identification of knowledge-holders and ways of triangulating input.

Evolution of patient involvement

Several positive developments have strengthened patient involvement over the past decade:

- Clear descriptions of patient knowledge types and research methodologies
- Widespread adoption of patient engagement across the product lifecycle
- Co-design of processes with patients
- Comprehensive training and support for patients and patient organisations
- Methodological advances in patient-reported outcome measures, patient preference studies, and rapid qualitative evidence synthesis.

Areas requiring improvement

Despite progress in patient involvement, there are critical areas needing attention:

- **Relationship building:** Engagement requires relationships, but these are difficult to establish during high-stress, high-impact individual assessments. Greater engagement in organisational settings, process development, and capacity building is needed.
- **Knowledge retention:** Patient knowledge is often learned but not documented or transferred across the product lifecycle, resulting in repeated requests for the same information from patients.
- **Research investment:** Under-investment in robust research into patient perspectives prevents building an enduring evidence base.
- **Representation:** Lack of clarity about who provides input may result in learning primarily from those most similar to researchers, potentially missing opportunities to reduce inequities.
- **Skills development:** Effective engagement requires training not only for patients but also for HTA and regulatory professionals in social science and engagement skills.
- **Process and goals:** Highly skilled engagement requires explicit goals to enable appropriate method selection and evaluation of effectiveness.
- **Impact assessment:** Understanding whether patient input makes a difference is essential for both communities to adjust their practices, reduce the burden of involvement, and maintain engagement.

Change management considerations

The complexity of integrating patient engagement and experience data into established HTA and regulatory systems requires understanding change management principles. Larger, more ambitious, and politically contested change requires relying on social practice perspectives rather than purely mechanical approaches, such as processes and frameworks. Whilst patient engagement has spread quickly as a compelling concept, use of patient experience data or evidence has been slower. Truly integrating both into practice to grow knowledge and impact decision making involves higher risk and complexity.

Conclusion

Meaningful progress in patient engagement and experience data requires moving beyond mandate-driven approaches to embrace thoughtful, respectful debate and critical examination of current practices. The literature suggests that strengthening collaboration through open discussion of doubts, questions, and disagreements will advance patient engagement and patient experience data integration throughout the regulatory and HTA lifecycle. The foundation for change exists, but realising its potential requires addressing systemic challenges and embracing more sophisticated approaches to knowledge integration and relationship building.

Patient engagement in regulatory and HTA decision making in the Asia-Pacific

Comparative maturity analysis with global benchmarks

Dr Durhane Wong-Rieger, President, Asia Pacific Alliance of Rare Disease Organizations, and President and CEO, Canadian Organization for Rare Disorders

Literature review on patient engagement in HTA in the Asia-Pacific

A systematic literature review was conducted to evaluate patient engagement (PE) in HTA processes across the Asia-Pacific region. The literature search focused on studies published between 2018 and 2022 from Australia, China, Japan, Malaysia, New Zealand, the Philippines, South Korea, Singapore, Taiwan, and Thailand.

The assessment framework had 11 parameters grouped into two categories: *how* patients engage (e.g. written submissions, public consultations, committee participation, public hearings, and early dialogue), and *what* they contribute to HTA evaluations (e.g. patient-reported outcomes, societal impacts, and patient preferences).

Findings and regional variations

The study revealed considerable variations across the Asia-Pacific region in PE maturity. Australia emerged as the most developed jurisdiction, benefitting from longstanding HTA processes and early patient involvement initiatives. Taiwan demonstrated remarkable progress, having embraced PE comprehensively and expanded beyond initial limitations. Singapore, despite entering the field relatively recently, showed rapid advancement in implementation.

Jurisdictions were categorised into different developmental stages: most developed (Australia, Taiwan, Singapore), developing (Korea, Philippines, Thailand, New Zealand), and underdeveloped (Japan, China, Malaysia). These classifications reflected not judgements on overall HTA quality but specifically on PE integration.

Key gaps identified across the region were in relation to early engagement, diversity strategies, patient-generated data use, societal weighting, and transparency of impact. In addition, patient participation in governance remained limited across most countries.

International comparison

Comparative analysis with other HTA systems outside of the Asia-Pacific revealed that countries like Scotland, Canada, and Germany have more advanced PE frameworks. Scotland was identified as exemplary in PE practices, whilst Canada offers structured patient input processes and Germany demonstrates effective institutional embedding of PE.

The evolution of PE in HTA typically progresses through five stages: 1) no engagement, 2) consultation, 3) structured submissions, 4) deliberative participation (e.g. committee membership), and 5) governance partnership. Most countries worldwide, even those with advanced PE frameworks, have not achieved stage 5 with full governance participation. The majority of countries in the Asia-Pacific are at stages 2 (consultation) or 3 (structured submissions).

Ongoing challenges

The evaluation of qualitative patient-based evidence presents ongoing difficulties, as most HTA committees and researchers lack training in robust qualitative analysis methods. Threshold clarification beyond traditional cost-effectiveness measures requires development, as well as the integration of preference data.

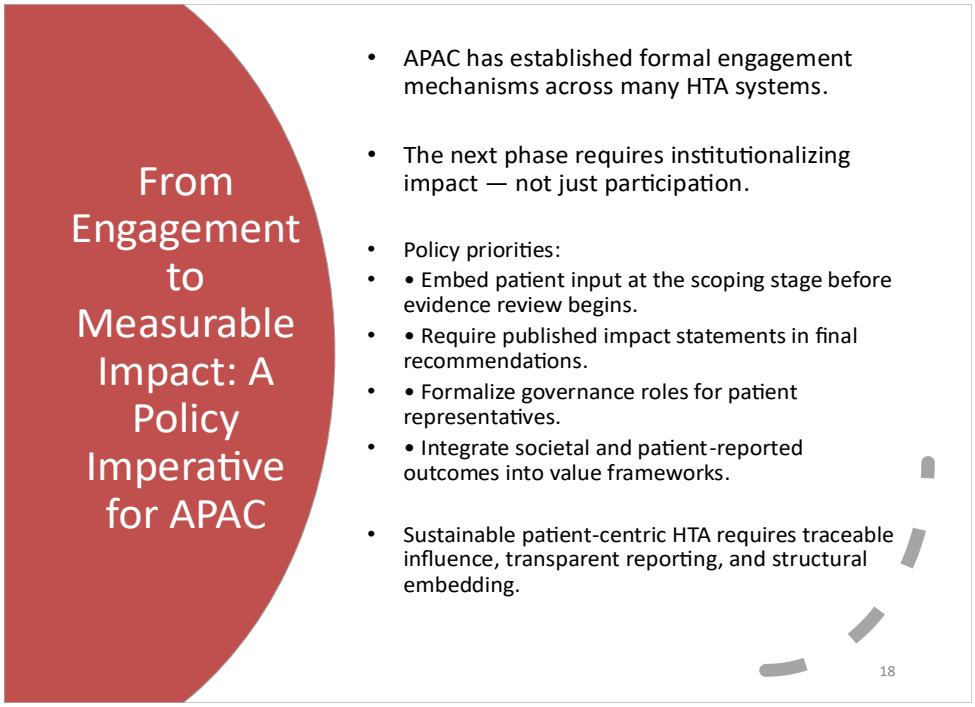
Measuring the impact of PE also presents significant challenges. Traceability of patient input through HTA decision-making processes is essential but often lacking. Reports should clearly demonstrate where patient contributions influenced outcomes, providing transparency and accountability. Formal evidence structures must accommodate diverse patient groups, including smaller organisations with limited experience.

Implications for regulators

Regulators' engagement with patients in the Asia-Pacific region is limited compared to HTA agencies, with minimal evidence of formalised patient participation in regulatory decision making. Regulators should first aim to structurally embed PE in their processes, then formalise governance roles for patients.

Conclusion

The evolution from engagement to measurable impact represents the next frontier in patient participation in HTA. The presence of patients at decision-making tables is insufficient; demonstrable influence on outcomes must be evidenced and measured. Regional variations in PE in HTA suggest that whilst progress has been made, significant opportunities exist for advancement, particularly in governance participation, qualitative evidence integration, and impact accountability.



Panel discussion: Patient engagement in regulatory and HTA decision making in the Asia-Pacific

Ann Single, HTAi President, and CEO of Patient Voice Initiative, Australia

Dr Durhane Wong-Rieger, President, Asia Pacific Alliance of Rare Disease Organizations, and President and CEO, Canadian Organization for Rare Disorders

Dr Yoong Khean Khoo, Assistant Professor, Centre of Regulatory Excellence (CoRE), Duke-NUS Medical School, Singapore

Dr Arshiya Zaheer, Regional Patient Engagement Lead, MSD, Singapore

Several themes arose during the open discussion:

Transparency to avoid tokenism

Patients need clarity on how their input was used and what difference it made to decision making. This transparency supports meaningful patient engagement and reduces the risk of tokenism.

Institutionalising patient engagement

Fundamental challenges for regulatory and HTA systems in the Asia-Pacific include the lack of formal patient engagement mechanisms and the need to institutionalise patient engagement in decision making. Additionally, socio-cultural differences across the region require tailored frameworks rather than one-size-fits-all approaches.

Capacity and capability building

While patients need to have an understanding of regulatory and HTA processes, regulators and HTA agencies also need to understand patient-lived experience and the value of patient data. Agencies should consider using nomenclature that recognises people with lived experience as experts (in accordance with the WHO framework). Addressing this knowledge asymmetry between agencies and patients requires collaborative efforts, which could be supported by academia as a neutral third-party.

Affordability and economic value

There is a need for clarity on what healthcare systems can afford. Government budget constraints must be explicit to support patient participation in trade-off discussions as equal partners, while governments need to articulate what's achievable within resource limitations.

Patient-led evidence on productivity loss, early diagnosis benefits, and societal impact can help build the economic case for the value of a treatment, especially in rare diseases. Engaging with government stakeholders beyond health ministries, such as finance and labour departments, could be beneficial.

Exploring alternative pathways

Rather than simple yes/no decisions, more flexible pathways should be explored, particularly in cases with uncertain outcomes, such as rare diseases. Examples of alternative pathways include compassionate access programmes, coverage with evidence development agreements, and risk-sharing arrangements.

Industry considerations

Industry should integrate the patient voice early and across the product life cycle, building mutual respect and trust. Patient input collected too late and retrofitted retrospectively is unlikely to meaningfully influence decisions.

Companies should consider coming together to create patient engagement platforms, avoiding silos. They should also ensure underrepresented populations are brought to the table in the diverse Asia-Pacific region.

Session 2: Focus on HTA agencies' decision process: How HTA agencies integrate patient insights into decision making

Patient input in HTA decision processes: How are HTA agencies leveraging patient experience and industry-generated PED into the assessment and appraisal?

Patient perspective

Hwan Ruey (Eric) Liu, Secretary General, Taiwan Young Patient Association

The value paradox in HTA

HTA often focuses primarily on costs and technical capabilities whilst neglecting to consult patients about their needs. This approach fails to recognise that value is fundamentally determined by the patient's experience and requirements. Patient perspectives must therefore be sufficiently integrated into value assessments.

Growth in precision medicine

The healthcare landscape faces an unprecedented budget paradox characterised by infinite innovation against finite resources. The growth trajectory of precision medicines demonstrates this challenge. In the early 2000s, typically one to three precision medicines received regulatory approval annually, but between 2019 and 2024, this figure increased to 15-25 approvals per year. By the end of 2024, approximately 400 precision medicines were estimated to exist globally. The proportion of precision medicines in FDA approvals has risen from only 5% in 2005 to 38% in 2024.

This expansion is particularly evident in oncology, a core area of precision medicine. Global oncology drug spending has increased exponentially from $24 billion in 2004 to $220 billion in 2024. Such rapid growth necessitates a fundamental reconsideration of traditional evaluation methodologies, as conventional approaches prove inadequate for assessing the value of these innovative treatments.

Precision medicine funding challenges

Three primary challenges emerge in the era of precision medicine. Firstly, precision medicines target smaller patient populations, compelling pharmaceutical manufacturers to increase unit costs to maintain viability. This creates a fundamental tension between providing life-changing medicines for individual patients and ensuring broader population coverage under national health insurance schemes.

Secondly, patients can face difficulties in advocating for their needs within reimbursement discussions. The question of resource allocation presents an ethical and practical dilemma that patients cannot reasonably be expected to resolve. Patients should not bear the responsibility of determining governmental prioritisation strategies.

Thirdly, data uncertainty poses substantial challenges. Many innovative treatments rely on single-arm phase two trials or accelerated approvals, requiring HTA agencies and national health insurance administrations to make long-term funding decisions based on immature data. However, this uncertainty creates opportunities for patient engagement, as patient perspectives can help define essential needs and values when concrete clinical data remains limited.

Integrated approach to patient access

The Center for Drug Evaluation has developed a comprehensive framework to address these challenges through three key strategies. The first involves transforming patient stories into evidence through a patient opinion sharing platform, where patient groups can contribute perspectives on treatments under review. These opinions are integrated into HTA reports and considered alongside expert assessments in the Pharmaceutical Benefit and Reimbursement Scheme Joint Committee, which includes four patient representatives.

The second strategy employs diversified financial risk-sharing mechanisms, acknowledging the need for creative approaches to accessing innovative treatments whilst maintaining system sustainability. This requires honest dialogue about needs and affordability constraints.

The third strategy established a Cancer Drug Fund, providing additional government funding to accelerate coverage for breakthrough treatments with uncertain evidence. This resource helps bridge the gap between promising innovations and established evidence requirements.

Conclusion

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. Through early dialogue and collaboration, patient needs can be transformed into sustainable solutions that serve all stakeholders whilst maintaining system viability and ensuring equitable access to life-changing treatments.

Patient input in HTA decision processes: How are HTA agencies leveraging patient experience and industry-generated PED into the assessment and appraisal?

Company perspective

Logan Caragata, APAC Policy Head, Roche, Malaysia

Evolution of evidence requirements

The healthcare evidence landscape has transformed significantly over the past 15 years. Previously, industry submissions to HTA agencies were primarily scientifically and clinically driven, with limited discussion about supporting healthcare system objectives or government agendas. The current approach emphasises evidence curation, with companies demonstrating product value in ways that align with broader healthcare system goals. This shift is particularly evident in Malaysia, where formulary listing discussions now centre on how medicines support government healthcare agendas and system-wide benefits.

Meaningful patient engagement

Effective patient engagement to create 'HTA ready' patient evidence requires three critical elements:

- **Early engagement** - must begin at the clinical trial design stage and continue throughout the entire access continuum until patients receive treatment and beyond.
- **Mixed methods** - approaches that combine patient-reported outcomes with qualitative patient preferences, ensuring both clinical and patient perspectives are captured.
- **A 'seat at the table'** - ensuring patient input and lived experience are formally integrated into HTA appraisal processes rather than remaining peripheral considerations.

Regional HTA patient involvement

Analysis of mature markets including Taiwan and Korea reveals that whilst most HTA agencies allow evidence submission and patient comments, and some permit meeting participation, very few integrate patient groups into actual decision-making processes. Taiwan represents one of the most advanced examples in the Asia-Pacific, collecting patient opinions online and including representatives in some Pharmaceutical Benefit Reimbursement Scheme meetings. However, the challenge remains integrating these perspectives into formal decision making rather than treating them as supplementary information.

Societal value integration

The BRAVER Report from the Office of Health Economics demonstrates varying levels of societal value consideration across Asia-Pacific countries. Whilst Thailand and China include societal value in base case analyses, most countries are still developing these capabilities.

Conclusion

Current HTA processes inadequately capture real-world patient experiences and societal impacts. The evolution towards evidence curation and societal value consideration represents progress, but formal integration of patient voices into decision making remains limited across the Asia-Pacific. To advance patient-centred HTA processes, there must be patient involvement from clinical trials through to patient access, formal weighting of the patient voice in decision making, and consideration of broader societal value.

Roche

Recommendations

Future of Patient Input into the 'Access Continuum'

1

From Trial to Living Room

From clinical trials through to full patient access, patient involvement efforts should be designed to be responsive across the 'Access Continuum', to minimize efforts for the patient community and involved bodies and to further drive consistency.

2

Scoring the Voice

Within HTA, move beyond "consulting" patients to formally weighting their input. By integrating a form of Multi-Criteria Decision Analysis (MCDA), we can ensure the patient experience is a non-negotiable, quantified percentage of the final reimbursement score.

3

Adopting Societal Value

HTA must account for broader societal gains–like returning caregivers to the workforce and productivity–to reflect the true ROI of health innovations.

9

How are HTA agencies currently or planning to engage patients and utilise PED during the assessment and appraisal process?

Philippines perspective

Tisha Isabelle de Vergara, Project Technical Specialist III, Policy Planning and Evaluation Unit, HTA Division, Department of Science and Technology, The Philippines

HTA mandates

The Philippines' HTA Division operates through four main mandates. Firstly, it serves as a priority setting mechanism that provides recommendations to the Department of Health (DoH) and the Philippine Health Insurance Corporation (PhilHealth). The process requires market approval from the Philippines' Food and Drug Administration before any health technology can be considered for HTA evaluation. Secondly, there is an HTA Council comprising a core committee with nine voting members, including a citizens' representative from the Philippine Alliance for Patient Organizations (PAPO). The council includes subcommittees organised by different types of health technologies.

The third mandate involves the HTA Office, which regularly engages with various stakeholders including decision makers and patient groups. The fourth mandate ensures adherence to HTA processes and principles, maintaining fairness throughout the entire process from topic nomination to final decision making.

Stakeholder engagement framework

A comprehensive stakeholder engagement guide is currently under development, separate from the main HTA process and methods guide. This framework establishes guidelines for stakeholder identification, roles and responsibilities, and engagement methods. Patient participation operates at three distinct levels: informative, consultative, and collaborative.

The informative level primarily involves submissions of nominations and document collection from stakeholders. The consultative process, which is most commonly employed, involves requesting comments and feedback on assessment documents from various stakeholders throughout different HTA process steps. The collaborative level involves conducting joint assessments, primarily with academia and research institutions, whilst partnering with PAPO for patient-led activities and capacity training programmes.

Stakeholder involvement in HTA processes

The topic nomination and prioritisation process involves the DoH and PhilHealth submitting priority topics for assessment. Patients and civil society organisations can submit their nominations through the DoH and PhilHealth. PICO consultations engage medical societies, patient groups, and programme implementers to validate assessment parameters, including identifying critical outcomes for consideration.

The methodological framework progresses from clinical assessment to economic evaluation when interventions are deemed superior or non-inferior to comparators. This is followed by the Ethical, Legal, Social, and Health Systems Impact (ELSHI) assessment, which examines patient experiences and perspectives across multiple dimensions including equity, geographical inequities in healthcare access, existing local policies affecting access, social preferences and values, and health systems implementation considerations. Data gathering for the ELSHI assessment primarily relies on literature reviews, adapting relevant patient preference studies to the local context.

To facilitate the consideration of qualitative aspects of patient preferences, a social values guide was created in 2022 based on national survey data. This guide identifies 21 social values categorised into specific domains, which are considered during assessments through deliberative and consultative discussions with the HTA Council and relevant stakeholders.

Following the posting of a preliminary recommendation, stakeholders have two weeks to appeal and submit additional evidence that may change the recommendation. The HTA Council decides on the merits of the appeals. The final recommendation is posted publicly on the website and social media pages of the HTA Council, regardless of the decision of the DoH/PhilHealth Board of Directors.

Challenges and limitations

Key challenges for involving patients in HTA in the Philippines include limited HTA capacity, poor health information infrastructure, limited capacity and training opportunities for patients, and limited financing mechanisms to compensate patient input as expert input.

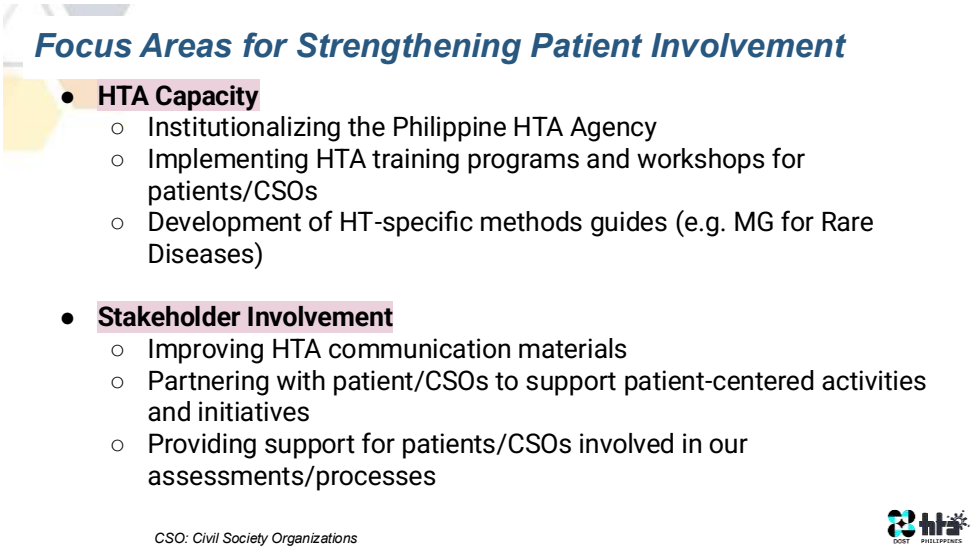
Regarding rare diseases and precision medicines, the Philippines' HTA Division currently lacks experience in assessing rare disease topics, as none have been prioritised since 2019. Whilst some cancer medicines have been evaluated, they were not specifically categorised as precision medicines.

Future initiatives and development

The HTA Division is transitioning to become an HTA agency under the Department of Science and Technology, with plans to establish a dedicated stakeholder engagement unit. Expansion plans include enhanced training programmes and workshops for patients and civil society organisations through PAPO partnerships.

Conclusion

The Philippines' HTA Division demonstrates a commitment to comprehensive patient engagement through its multi-level stakeholder involvement framework. Whilst challenges remain, ongoing initiatives to strengthen institutional capacity, develop specialised methods guides, and improve communication materials indicate a progressive approach to enhancing patient involvement in HTA processes.



How are HTA agencies currently or planning to engage patients and utilise PED during the assessment and appraisal process?

Malaysia perspective

Dr Roza Binti Sarimin, Head of HTA Unit, Malaysian Health Technology Assessment Section (MaHTAS), Ministry of Health, Malaysia

Introduction

The Malaysian healthcare system operates on both public and private sectors. The public healthcare system is government-provided, subsidised through general taxation, and offers comprehensive services from preventive to curative care. In contrast, the private healthcare system relies on private health insurance, out-of-pocket payments, and employer-based insurance, with services concentrated mainly in urban areas.

MaHTAS: The national HTA agency

The Malaysian Health Technology Assessment Section (MaHTAS) serves as the national HTA agency, situated within the Ministry of Health. The agency's scope extends beyond traditional HTA to include clinical practice guidelines, horizon scanning, and functioning as a 'single door' to the health industry. MaHTAS assesses not only drugs but also procedures, devices, systems, public health interventions, and traditional and complementary medicine. Despite its considerable responsibilities, MaHTAS operates with a modest workforce.

Structure and governance of MaHTAS

MaHTAS operates under a structured governance framework with patient involvement at multiple levels. The HTA-Clinical Practical Guidelines (HTA-CPG) Council sits at the top, chaired by the Director General and meet twice annually. Notably, patient representatives serve as one of Council members. Below the HTA-CPG Council sits the Technical Advisory Committee for HTA, and for individual projects, expert committees are established that occasionally include patients depending on the topic.

Value-based healthcare transition

Malaysia is progressing towards value-based healthcare guided by principles of efficiency and patient-centredness. The approach aims to deliver healthcare that is not only clinically effective, economically sound, and aligned with population values and expectations. This transition is supported by healthcare reforms including the Health White Paper for Malaysia, which outlines 15-year strategies to structurally reform the national healthcare system towards greater equitability, sustainability, and resilience.

Patient and public involvement framework

MaHTAS has embraced patient involvement in the HTA process, incorporating and guided by five key elements of patient and public involvement (PPI) as highlighted by HTA International (HTAi): relevance, fairness, equity, legitimacy, and capacity building. Importance of PPI in HTA include enhancing transparency and public trust, whilst preventing the widening of health disparities. MaHTAS utilises a dedicated PPI team to facilitate the execution of strategic engagement initiatives.

Current patient involvement processes and challenges

The HTA process in Malaysia begins with topic identification through requests from stakeholders, including from patient representatives, followed by filtration on appropriateness for assessment, prioritisation using predefined criteria, assessment, appraisal, and approval by the Council before dissemination. MaHTAS facilitates patient involvement at key stages of its assessment process—from initial identification and prioritisation through to participation in expert committees where appropriate, and during appraisal through patient representation in the HTA-CPG Council.

MaHTAS' experience with collecting patient experience data (PED) is limited, with the most recent example focusing on continuous glucose monitoring system for diabetes patients. Balancing the requirement for ethical rigour in patient perspective studies with the need for timely HTA delivery remains a key operational challenge.

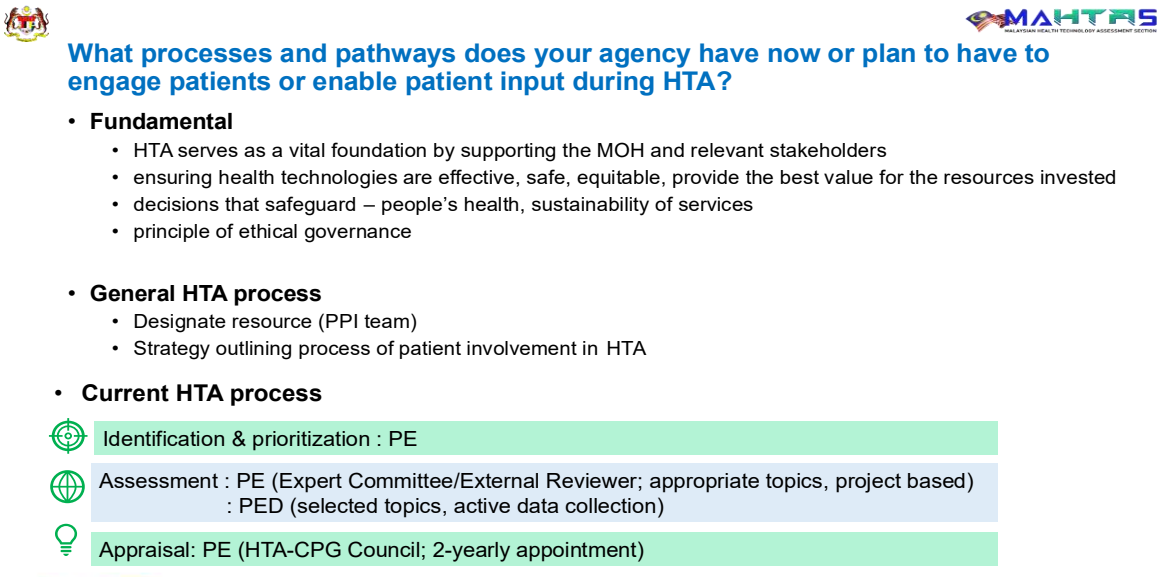
Integrating patient perspectives into HTA faces several challenges include time and resource limitations, ensuring representative patient perspectives, varying quality of input, knowledge gaps between patients and providers, and government concerns about emotional or biased decision making.

Future plans for patient involvement

MaHTAS plans to enhance patient involvement by inviting patients to participate in the prioritisation phase together with the advisory committee. The agency will try to expand patient engagement projects to more topics where appropriate, and obtain PED directly from patients for identified topics through patient survey forms. To align with existing capacity and resources, priority will be given to topics/disciplines/diseases where quality of life is of utmost important, such as oncology, mental health, and rare diseases, as well as those topics with national interest, unmet needs, high impact, and high cost. In addition, MaHTAS plans to finalise patient involvement guidance and explore outreach engagement activities to underrepresented groups.

Conclusion

MaHTAS integrates patient involvement throughout the key stages of the HTA process (see below). While operationalising patient perspectives presents challenges, including time and resource constraints, ensuring representative input and bridging knowledge gaps remain the strategic focus. Moving forward, there are plans to explore the expansion of patient engagement projects to more topics, obtain direct PED via structured surveys, and finalise standardised guidance on patient involvement.



How are agencies currently or planning to engage patients and utilise PED during the assessment and appraisal process?

Singapore perspective

Shawn Quek, Senior Specialist, Consumer Engagement and Education, Agency for Care Effectiveness, Singapore

Patient involvement opportunities

The Agency for Care Effectiveness (ACE) in Singapore established its Consumer Engagement and Education (CEE) team in April 2021 to support patient involvement in ACE's work. There are three main opportunities for patients and carers to be involved:

- Suggesting health technologies (topics) for evaluation
- Providing lived experiences to inform technical evaluations
- Providing feedback on educational resources that the CEE team co-develops with patient organisations.

Patient input in technical evaluations

Since May 2022, patients have been providing lived experiences to inform HTAs for drugs and medical technologies. At the start of each evaluation, the CEE team distributes surveys to patient organisations that can be completed by individual patients and carers, or by the patient organisation as a combined submission of its members' responses. These surveys capture patients' lived experiences with their condition, experiences with current treatments, and expectations for the health technology under evaluation.

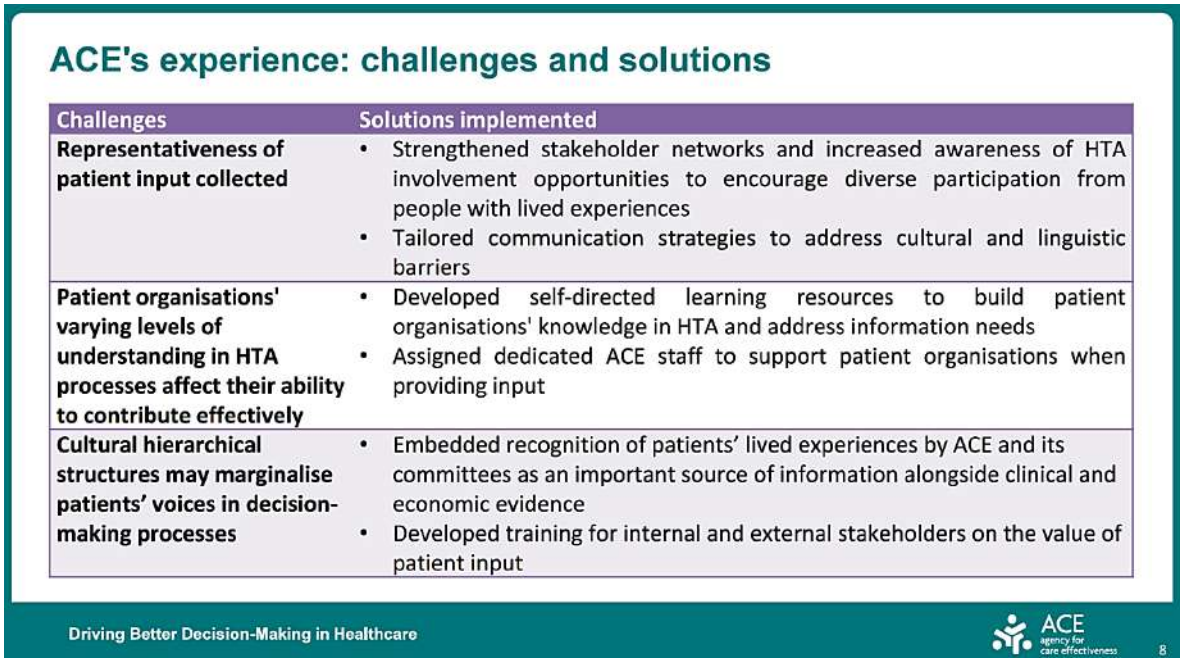
The CEE team incorporates survey responses into ACE's evaluation reports, which are considered by the Ministry of Health Advisory Committee for funding deliberations. When the committee has made its recommendation, ACE publishes a summary of the patient input in its guidance for the health technology, including a plain language summary. In addition, the CEE team provides feedback to patient organisations to ensure that they understand how their responses fit into the HTA process.

Rare Disease Fund

Patients can suggest topics for evaluation for inclusion in the Rare Disease Fund (RDF), a national multi-stakeholder charity fund launched in 2019. This fund provides long-term financial support to patients with ultra-rare genetic diseases who require high-cost treatments. The process involves topic suggestion by stakeholders including patients, carers and patient organisations, followed by topic prioritisation with a clinical expert group, and funding decisions by the RDF committee. Eight rare diseases have been successfully included in the RDF.

Challenges and solutions

ACE has identified key challenges and implemented targeted solutions based on experience (see table below). To address representativeness issues, the agency has strengthened stakeholder networks, increased awareness of involvement opportunities, and tailored communication strategies to overcome cultural and linguistic barriers. Self-directed learning resources have been developed to support patient organisations with varying levels of HTA understanding. To address cultural hierarchical structures that may marginalise patient voices, ACE has developed training materials and embedded recognition of patients' lived experiences as important information sources alongside clinical and economic evidence.



Consumer Panel involvement

ACE's Consumer Panel, comprised of senior leaders from patient organisations in Singapore, has been key to overcoming barriers to patient involvement. With their guidance, solutions have been developed to ensure patient involvement is inclusive, patient-centred, and meaningful. This collaborative approach has culminated in contributions to academic publications, including a chapter in the second edition of *Patient Involvement in Health Technology Assessment*, co-written with a Consumer Panel member.

Impact measurement

ACE's efforts have achieved measurable outcomes, with more than 80% of HTAs from 2022 to 2024 including patients' lived experiences. Over 40 patient organisations were involved over that time, and more than 700 patient and carer testimonials were incorporated into technical evaluations. Outcomes and impact stories are published annually in the Voices to Impact publication, providing ongoing transparency about the patient involvement programme's development and impact.

Conclusion

Singapore's approach to patient involvement in HTA demonstrates a systematic and collaborative model that has achieved meaningful impact in decision-making processes. The emphasis on transparency, impact measurement, and continuous process improvement positions Singapore's experience as a valuable resource for other jurisdictions seeking to enhance patient involvement in HTA processes.

How are agencies currently or planning to engage patients and utilise PED during the assessment and appraisal process?

Australia perspective

Andrew Mitchell, Honorary Professor, The Australian National University

Patient input framework

Australia operates a dual-stream approach to patient input in HTA. The first stream involves embedding patient information directly within dossiers submitted by applicants. This approach emphasises measuring improvements in patient-relevant outcomes, preferably in randomised trials. Formal utility valuation methods using multi-attribute utility instruments are preferred, with Australian weights, if possible, thereby embedding patient input directly into the evidence base rather than treating it as a separate component.

The second stream involves a comprehensive public consultation process that enhances and supplements information provided by the applicant. This consultation primarily operates through written submissions, though video and audio formats are accepted provided they under 10 minutes long. Patients can submit any patient experience data they consider relevant. Importantly, this consultation extends beyond patients to encompass the broadest possible public participation, ensuring comprehensive stakeholder engagement.

Committee processes

The system operates through two main committees: the Medical Services Advisory Committee (MSAC) and Pharmaceutical Benefits Advisory Committee (PBAC). Each committee publishes an agenda showing all the applications to be assessed, allowing the public to prepare their inputs. MSAC employs a longer process involving Population, Intervention, Comparator, Outcome (PICO) determination as well as assessment steps, creating an additional opportunity for patient engagement.

Added value of patient input

Patient input enables HTA committees to better understand how proposed health technologies may affect the lives of people with specific conditions, relevant clinical practice, and parts of the healthcare system. The information may also provide insights into benefits, disadvantages, and experiences of individuals who have received proposed health technologies. Patient-relevant outcomes and expressed values complement clinical trial results in applications, while individual patient insights enable more holistic deliberation processes.

Patients frequently provide valuable insights regarding safety concerns, better patient targeting, and occasionally highlight healthcare system constraints that the HTA committees must consider. The fundamental driver behind public consultation centres on ensuring that all affected stakeholders have, and are seen to have, an effective voice in deliberations, emphasising procedural fairness and natural justice whilst maintaining timely decision-making processes.

Challenges and considerations

The Australian HTA agency faces several challenges with application-based patient input and public consultation, such as issues with capacity, infrastructure, representativeness, quality of input, and timeliness (see below). Public consultation timelines are necessarily constrained, creating ongoing tension between comprehensive patient input and efficient decision making.

Challenges (HTA agency has an indirect role)

	In application (up to health technology sponsor)	Via public consultation (up to respondent)
Capacity	Influence of PED on decision making varies across applications	Acknowledge and manage multiple competing demands on same patients
Infrastructure	Guidance and options are open-ended rather than directive	Explore ways to increase confidence in providing useful information
Representativeness	Assess relevance to local patients and healthcare system	Expect skewing to greatest baseline severity and treatment benefit
Quality of input	Assess risk of bias	Identify and minimise any conflict of interest
Timeliness	May be superseded by competitors	Consultation period necessarily short

Policy developments

Recent developments include the Australian HTA Policy and Methods Review, which produced at least five recommendations related to ensuring person-centred HTA and elevating consumer voices in deliberations, particularly for children and First Nations people. The Australian government subsequently commissioned an implementation advisory group, with ministerial priorities including developing an HTA stakeholder engagement framework focused on improving consumer and patient engagement. Additionally, eight research grants have been awarded through the Medical Research Future Fund to better understand how to incorporate patient data into HTA decision making.

Case study: Spinal muscular atrophy

Looking across 21 HTA committee meetings involving three medicines for spinal muscular atrophy illustrates the complex interplay between patient input and decision making. The progression from initial rejection through stakeholder meetings to eventual recommendations demonstrates the pivotal role of patient input in providing context to the decisions. Patient advocacy, combined with other factors including pricing negotiations, influenced access decisions across disease severity spectrums and different age groups.

Conclusion

Australia's approach to patient input in HTA demonstrates a multi-faceted system that balances comprehensive stakeholder engagement with efficient decision-making processes. The framework ensures that patient perspectives are embedded both within sponsors' evidence submissions and through broad public consultation. While challenges remain regarding timeliness, representativeness, and resource allocation, the system's emphasis on procedural fairness and the demonstrated impact of patient input on complex decisions illustrate the value of systematic patient engagement in HTA processes.

Session 2 discussion summary

The business case for patient involvement

The discussion highlighted that while there is general agreement on the benefits of involving patient voices in development, moving to the next level requires stronger commercial incentives. The potential to achieve premium pricing when including comprehensive patient experience data in clinical packages could serve as a key driver for companies to increase investment in patient involvement initiatives. A fundamental challenge is how to create transparent mechanisms that honestly translate demonstrated value into appropriate pricing, while ensuring patient perspectives are included in these discussions.

Broadening value assessment beyond drug costs

The importance of considering societal value beyond direct drug-to-drug cost-effectiveness comparisons was also emphasised. Research can quantify additional benefits such as healthcare system savings from reduced travel requirements, fewer hospitalisations, or enabling treatment delivery closer to communities. Governments often focus solely on drug acquisition costs while overlooking significant downstream savings from reduced hospitalisations and other healthcare system efficiencies.

A structural challenge exists in how governmental responsibilities are divided. National health insurance systems typically focus on budget allocation within their own portfolios, with limited incentive to consider productivity losses or broader economic impacts. There is growing recognition that ministries responsible for labour, economics, and finance should be engaged in these discussions, as disease impacts extend beyond patients and families to affect entire societies.

The challenge of opportunity costs

A fundamental question emerged about how decision-making processes characterise both the value and losses associated with adopting a health technology. While considerable attention focuses on incorporating patient-reported outcomes and experiences on the value side, less consideration is given to characterising the opportunity costs and losses associated with resource allocation decisions. However, addressing this may put more resource pressure on HTA agencies and risks creating divisions within patient communities by forcing discussions about disease prioritisation. A creative approach is needed to address it constructively without undermining patient community unity.

Measuring value in complex real-world settings

A question was raised about how value measurements account for comorbidities in real-world settings. While industry submissions typically focus on one product for one condition, many patients live with multiple health issues, making it challenging to quantify how a specific product improves health outcomes.

Session 3: Focus on regulatory agencies' decision process: How regulatory agencies integrate patient insights into decision making

How are regulatory agencies leveraging patient experience and industry-generated evidence in evaluation and approval?

Patient perspective

Christopher Munoz, President, Philippine Alliance of Patient Organizations, and Civil Society Representative of the Philippines HTA Council

Regulatory bodies in the Philippines

The Philippine healthcare system comprises three key regulatory entities: the Department of Health (DoH), PhilHealth, and the Food and Drug Administration (FDA) Philippines. PhilHealth and FDA Philippines operate as attached agencies to the DoH, with independent mandates whilst maintaining harmonised policies. The DoH oversees all health services, products, and providers, while PhilHealth serves as the national health insurance body. FDA Philippines functions as the regulatory authority for health products, managing product registration, licensing, establishment oversight, and monitoring and enforcement activities.

Patient-centred clinical trials

The conceptualisation phase, where patients identify the most crucial symptoms requiring treatment, is key to informing the design of clinical trials. This is particularly relevant in rare disease contexts where multiple symptoms are present. It is important to develop patient-friendly protocols, identify meaningful outcomes and provide clarity in informed consent processes.

Patient experience integration in regulatory processes

Regulatory agencies need to know what risks patients are willing to take, acceptable side effects, trade-offs, what is important to patients, lived experiences, unmet clinical needs, treatment challenges, and impact of the disease on daily life. Patient experience complements rather than replaces scientific evidence, contributing valuable insights to clinical and technical data whilst potentially revealing gaps in research findings.

The FDA Philippines brings patient experience into its work through public consultations and stakeholder meetings. Regulatory oversight activities focus on patient incident reporting and complaints.

The DoH integrates patient experience into policy development through public consultations and stakeholder meetings, with more comprehensive patient involvement in clinical practice guidelines and technical working groups. Patient participation in clinical practice guidelines proves essential as care pathways developed without patient input may not align with patient expectations. Technical working groups involve more extensive collaboration in creating administrative orders and strategic plans.

Patient involvement in healthcare benefit processes

PhilHealth incorporates patient experience in developing inpatient and outpatient benefits through stakeholder meetings and public consultations. The benefit development process involves multi-stakeholder collaboration and comprehensive patient journey mapping to ensure continuous care across all disease stages.

Cost estimation represents a crucial component of benefit development, requiring government data repositories for accurate costing libraries combining private and public sector data. Service mapping ensures patient-centred referral systems, whether through one-stop shop facilities in larger hospitals or primary care initiation points.

Enhancing patient influence in regulatory decisions

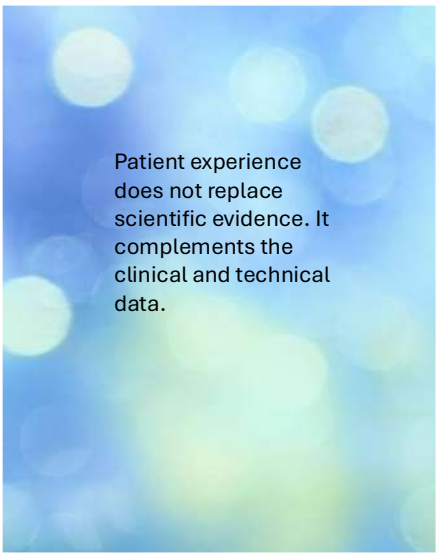
Meaningful patient involvement requires progression from engagement to influence. Engagement is being invited to participate, whilst influence means shaping how decisions are made and interpreted. Patient organisations must shift from testimonial participation to structured, evidence-informed advocacy.

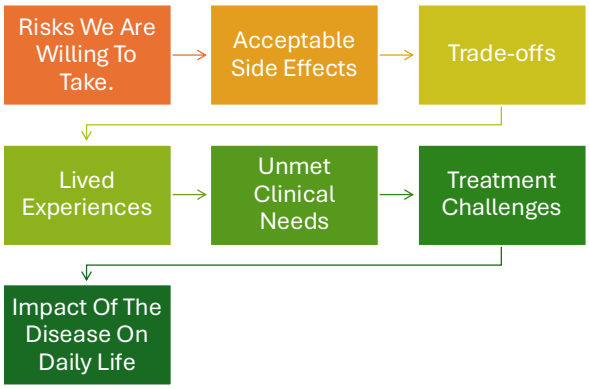
Rare diseases and precision medicine

Regulatory agencies need to improve patient involvement in rare disease and precision medicine evaluations through early engagement, formalised patient input, translating patient perspectives into evidence, ensuring patient-centred care, giving decision-making authority to patients, enhancing transparency, and implementing feedback mechanisms. Patients must have awareness of the regulatory process and ensure diverse representation from across disease stages. Capacity building and financial support are key.

Conclusion

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 becomes indispensable. The ultimate measure of treatment success extends beyond clinical parameters to encompass patients' ability to resume normal daily activities, emphasising the critical importance of incorporating patient perspectives in regulatory frameworks.





How are regulatory agencies leveraging patient experience and industry-generated evidence in evaluation and approval?

Company perspective

Dr Svetlana Yanchuk, Country President, AstraZeneca, Malaysia

Patient-centered development

The pharmaceutical industry is experiencing a fundamental shift where patient-centered drug development has evolved from a desirable practice to an essential licence to operate. This transformation reflects recognition that patients must be viewed as true partners in the co-creation of drug development processes, study designs, and implementation throughout all phases of research.

Global regulatory landscape for patient experience data

Incorporating patient experience data (PED) in regulators' decision making enhances understanding the unmet need, benefit–risk, and real-world impact, making regulatory decisions more clinically relevant and legitimate to patients. The incorporation of PED into regulatory pathways represents a global trend with varying degrees of advancement across different regions. Whilst the US Food and Drug Administration (FDA) and European Medicines Agency (EMA) have established more sophisticated frameworks for patient input integration, the Asia-Pacific region demonstrates promising developments, particularly in countries such as China and Japan.

Rare disease drug development

The implementation of patient-centered approaches proves particularly valuable in rare disease development, where patient populations are limited and individual experiences carry greater significance. AstraZeneca's rare disease division, Alexion, demonstrates comprehensive patient involvement throughout the entire development lifecycle, from pre-development research through to product launch. This includes patient participation in study protocol development, clinical site selection, and ongoing programme oversight.

The development of eculizumab for paroxysmal nocturnal haemoglobinuria is a good example of patient-centered development. Beyond standard clinical protocol implementation, the programme incorporated comprehensive patient feedback through patient-reported outcomes (PROs), continuously monitoring fatigue, symptom burden, and quality of life. Additionally, global registry data provided valuable insights despite the non-randomised nature of the patient population. This collaborative approach resulted in an extended label for eculizumab, demonstrating the tangible benefits of patient-centered methodologies.

Benefits of early involvement

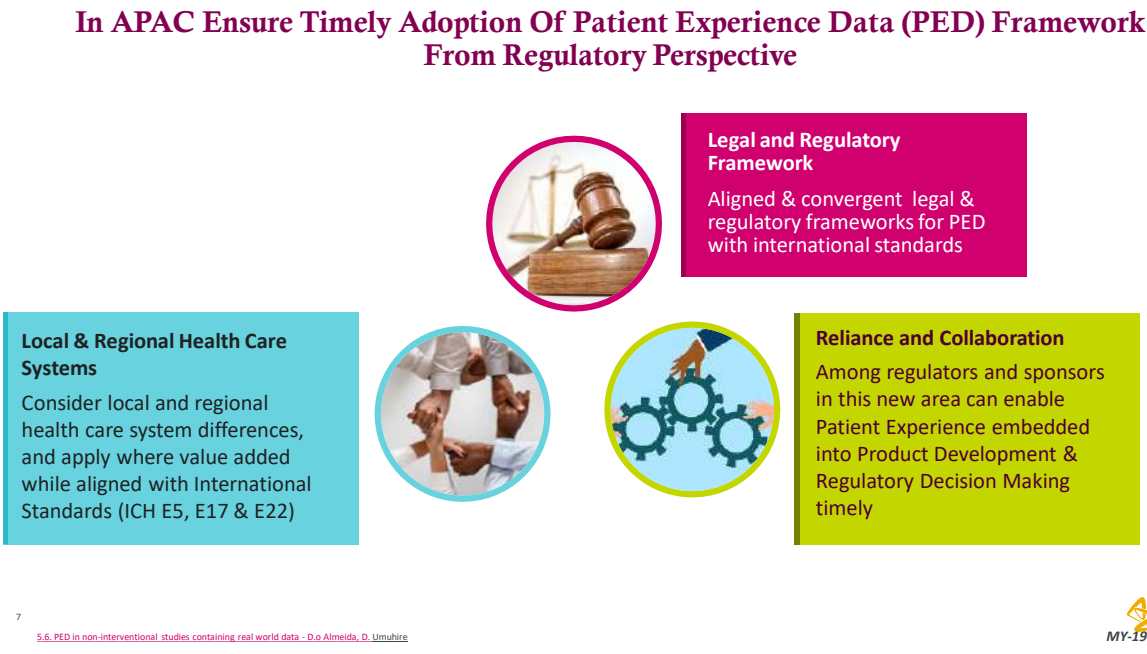
Early patient involvement creates significant advantages for downstream regulatory evaluation, because the same questions addressed during initial patient consultations are likely to be raised by regulatory bodies. The consistency of patient advocacy groups and patient representatives across development and regulatory phases ensures continuity and coherence in the evaluation process.

Addressing complex patient populations

Patient registries, PROs, and PED offer potential solutions for incorporating data from patients with comorbidities, addressing a significant challenge in modern drug development. These approaches provide richer, more comprehensive datasets that better reflect real-world patient experiences and treatment outcomes.

Conclusion

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



How are regulatory agencies engaging patients and utilising patient experience data during the evaluation and approval process?

Japan perspective

Naoyuki Yasuda, Special Advisor to the Chief Executive and Associate Executive Director for International Programs, Pharmaceuticals and Medical Devices Agency (PMDA), Japan

Putting patients first

The Japanese Pharmaceuticals and Medical Devices Agency (PMDA) seeks to achieve four 'firsts', with the first concept being "Patient first". The establishment of a patient engagement discussion group in May 2019 marked the beginning of systematic patient involvement in PMDA's processes. The culmination of these early efforts was the publication of PMDA guidance on patient engagement in September 2021.

Framework for patient engagement

The PMDA guidance comprises five distinct components designed to facilitate meaningful patient participation. The first element involves **direct patient participation in PMDA meetings**, including representation on the Pharmaceutical Affairs Council within the Ministry of Health, Labour and Welfare (MHLW), which serves as the final decision-making body for drug approvals. Additionally, patients participate in the PMDA Management Council, which convenes three times annually and represents one of the agency's most important forums.

The second component establishes dedicated **platforms for hearing patient voices** through exchange meetings with patient groups, citizens' seminars, and regional workshops. These initiatives, whilst conducted primarily in Japanese, create structured opportunities for dialogue between regulatory authorities and patient communities.

The third element focuses on providing **accessible information** to patients about pharmaceutical products. When PMDA approves new medications, the agency prepares materials specifically designed for patient understanding, complementing the technical documentation provided to healthcare professionals. These patient-oriented materials include product overviews, safety information, and effectiveness data presented in accessible formats.

The fourth component promotes **understanding of pharmaceutical regulations** through open information sharing via the PMDA website and direct communication with patient groups. This educational approach aims to enhance patient literacy regarding regulatory processes and decision-making frameworks.

The fifth element involves the use of **patient-reported outcome studies**, though the specific implementation details of this component are still under development. PMDA is collaborating with various stakeholders on the use of patient experience data – including patient preference studies – in PMDA's decision making.

Strategic principles

PMDA has identified four fundamental principles essential for effective collaboration between patient groups and regulatory authorities: coalition-building, interactive engagement, continuous development, and sustained continuity. PMDA is working together with patient groups to promote patient participation in consultation, review, and other steps of the regulatory process, and facilitate patient engagement across all disease areas.

Future initiatives

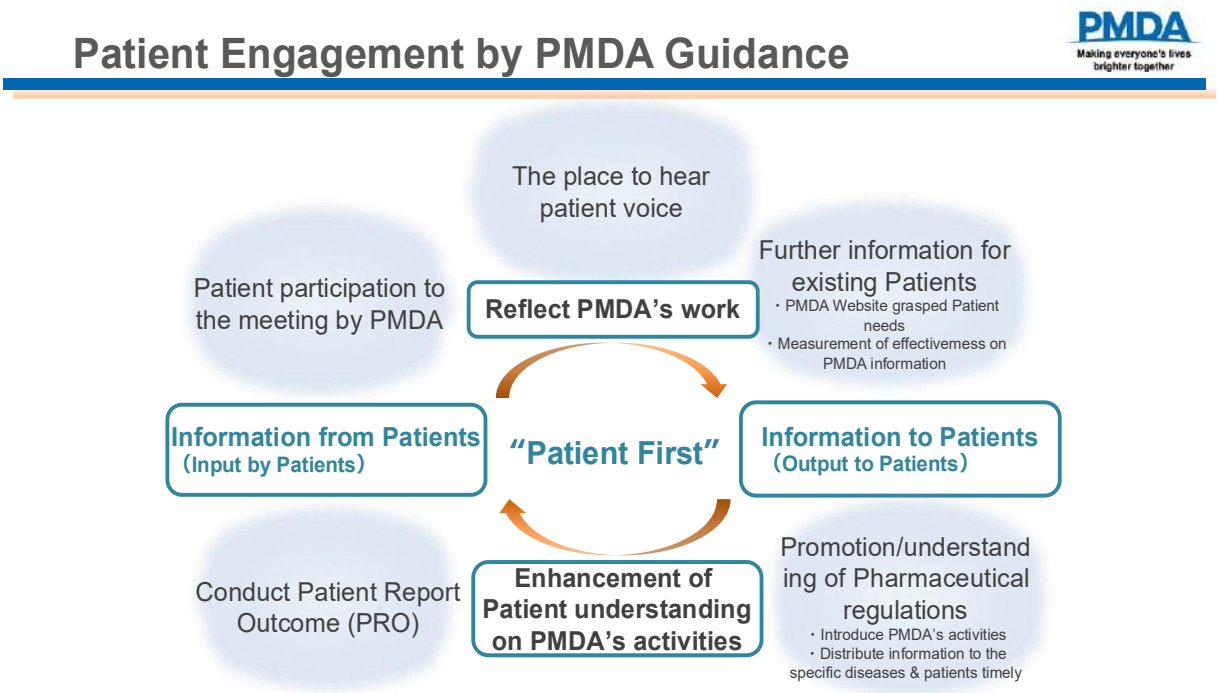
Future initiatives include expanding the breadth of patient participation, implementing more proactive PMDA activities to facilitate patient involvement, creating opportunities for open exchange of views and further participation, developing comprehensive education and training programmes for patients, and establishing systematic approaches for utilising patient voices in regulatory decision making.

International harmonisation

PMDA is actively contributing to international harmonisation efforts through participation in the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH). The development of ICH E22 Guideline for patient preference studies represents a major advancement in global regulatory practice. These guidelines are expected to be finalised by December 2026.

Conclusion

PMDA seeks to achieve four 'firsts', with the primary concept being 'Patient first'. Through structured guidance, multi-faceted engagement strategies, and international collaboration, the agency has established a foundation for meaningful patient participation in pharmaceutical regulation. Key principles for working with patients include collaboration, interactive engagement, continuous development, and continuity.



How are regulatory agencies engaging patients and utilising patient experience data during the evaluation and approval process?

Indonesia perspective

Dra. Tri Asti Isnariani, Director of Drug Registration, Indonesia Food and Drug Administration

Patient engagement framework

Indonesia's Food and Drug Administration (FDA) has adopted and benchmarked itself against the patient engagement framework of the European Medicines Agency (EMA), recognising the transparency and comprehensive patient involvement processes that can be learned from this model. The Indonesian FDA has implemented patient involvement across four major regulatory processes: regulation development, product development, marketing authorisation evaluation, and pharmacovigilance.

Regulation development

Public consultation represents a fundamental component of the process for developing regulations. Consumer agencies and consumer foundations are consistently invited to participate in these consultations. For example, when developing guidelines for cancer medicines, patient group communities are actively engaged, ensuring that patient perspectives inform regulatory guidance documents.

Product development

Patient engagement extends into the product development phase, particularly within clinical trials. Patient engagement facilitates the collection of patient experience data that can subsequently be submitted during the evaluation process. This approach helps to ensures that real-world patient experiences inform regulatory decision making. For example, in the case of a precision medicine with a molecularly targeted indication for breast cancer, patient-reported outcome data informed decisions regarding urgency and clinical consequences.

Marketing authorisation evaluation process

The marketing authorisation evaluation involves expert clinicians who participate in national committee meetings. These forums enable regulators to understand medical needs and identify the urgency of specific medicines. Drug usage experience from other countries is also considered during the evaluation process.

In addition, input is collected from disease-specific patient associations. For example, in the case of edaravone for amyotrophic lateral sclerosis, recommendation letters from patient foundations described real-world patient needs, which supported the evaluation process.

Post-marketing surveillance

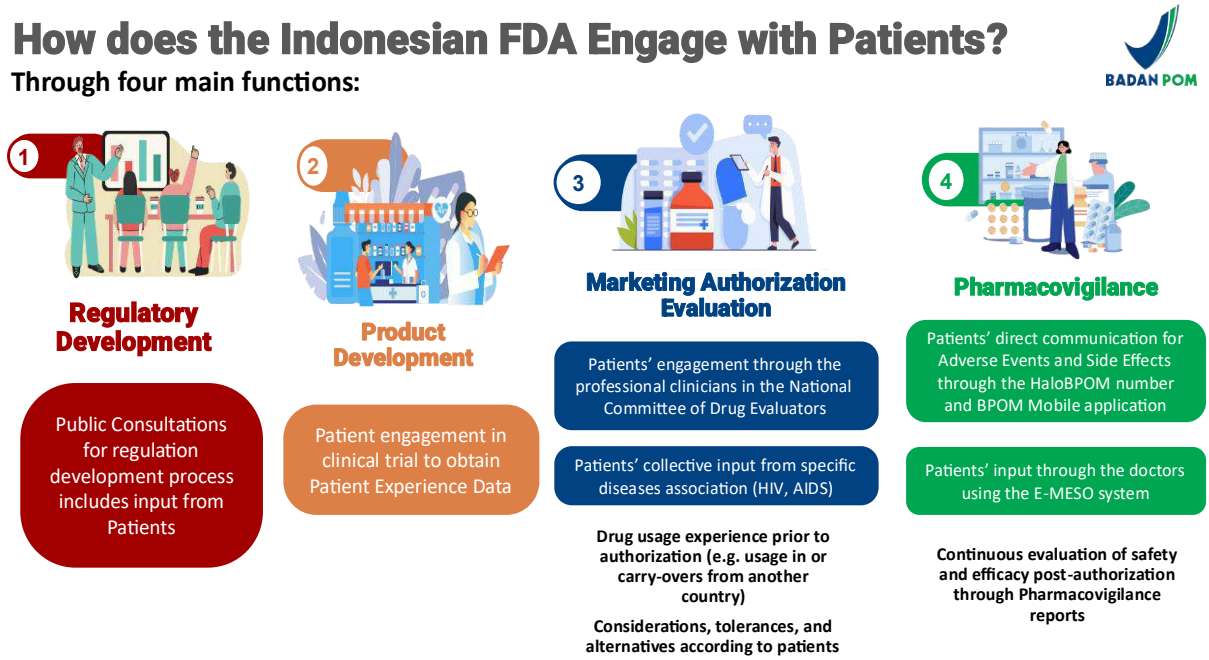
Post-marketing pharmacovigilance incorporates patient engagement through the BPOM Mobile app, which enables consumers to report complaints and symptoms experienced after medicine consumption. The E-MESO system allows doctors to report adverse events on behalf of their patients. It is currently being updated to allow public inputs. Professional supervision may be needed to help patients identify medicine-related adverse events, particularly given the complexity of multiple medicine use including traditional medicines.

Challenges and future plans

A key challenge is limited public awareness of the official channels for input and involvement in drug evaluation processes. Plans include establishing regular meetings with patient organisations and continuing to promote official public services for patient input. Regulatory and legal frameworks are being continuously updated according to international standards and national needs.

Conclusion

Patient involvement is integrated into Indonesia's regulatory framework through public consultations, clinical trial oversight, marketing authorisation evaluations, and post-marketing pharmacovigilance. The Indonesian FDA actively promotes the use of real-world evidence to inform and support regulatory decisions, ensuring that policies and approvals are based on robust data while aligning with international best practices. The agency continuously updates and strengthens its regulatory framework and implementation, leveraging partnerships between academia, industry, and government to foster innovation, knowledge sharing, and effective regulation.



How are regulatory agencies engaging patients and utilising patient experience data during the evaluation and approval process?

Australia perspective

Prof Anthony Lawler, Head, Therapeutic Goods Administration (TGA), Australia

Integration of patient insights

TGA seeks to incorporate patient and community input at all levels of decision making, including:

- Individual decision-making processes with advisory committee support
- Development of broad regulatory policy
- Community engagement initiatives
- Specific sectoral initiatives.

Consumer representation in advisory committees

TGA's seven statutory advisory committees feature consumer representation, helping to balance and complement clinical input. These committees advise on authorisation matters, restrictions, indications, and labelling, often addressing specific consumer-related issues. They also help to shape Consumer Medicines Information, providing key information about medicines that support informed healthcare decisions.

Shaping regulatory policy

TGA's Consultative Committee and Advertising Code Committee have strong consumer engagement and provide awareness of and input into broad regulatory policy. The agency is working to increase the role of the advisory committees beyond individual decisions to also inform regulatory policy. This would help to provide further opportunity for consumer and patient engagement in the decisions and direction of TGA.

Use of RWE and PROs

Following a review in 2021, several changes were introduced to clarify how real-world evidence (RWE) and patient-reported outcomes (PROs) are incorporated into regulatory decisions, providing greater transparency to sponsors. RWE can include secondary or exploratory objectives, natural history studies, registry data, health records, and human factor studies. The utilisation of RWE and PROs in TGA's decision making are documented in product assessment reports.

Community consultation

The TGA maintains public trust through extensive community consultations, exemplified by recent prominent cases involving vitamin B6 and medicinal cannabis regulation. Annual stakeholder surveys are conducted, giving opportunity for consumers, health practitioners, and industry representatives to give feedback to TGA.

Specialised working groups

Working groups on specific sectoral topics serve as valuable sources of patient insights. For example, the Women's Health Product Working Group involving clinical groups, professional bodies, and patient representatives, helps

TGA to address regulatory and access challenges, including reproductive health products, health literacy, and misinformation around weight loss and cosmetic injectables.

Conclusion

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. 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.

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Challenges and value add

The challenges of engaging with patients is also what provides the value add

Challenges		Value add
Communicating regulatory concepts in ways that resonate with consumers and translating their lived experience into meaningful regulatory insights	→	We must consider the real-life implications of a regulatory approach
Consumers experience is of the health system as a whole – it is not bound by regulatory scope	→	Provide us valuable insight into the impact of our regulations on consumer needs, expectations and trust in the system more broadly

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Session 3 discussion summary

At the end of Session 3, the speakers were asked to share their perspectives on the biggest challenge and opportunity for involving patients in regulatory processes.

Key challenges

- **Information accessibility**: A key challenge is ensuring that pharmaceutical and medical information are understandable and accessible to consumers, requiring support from health professionals to help patients interpret information correctly.
- **Language barriers**: Language is a key consideration when looking to enhance patient engagement capabilities.
- **Regulator mindset**: Regulators' preference for control and established patterns of published, peer-reviewed evidence can create resistance to alternative forms of evidence and patient perspectives.
- **Avoiding tokenism**: Regulatory agencies must be genuinely prepared to accept patients as active participants at decision-making tables rather than token representatives.
- **Meaningful data**: Ensuring patient involvement results in meaningful, comprehensive, and actionable data that can be effectively utilised in drug development and regulatory decision-making processes is complex.
- **Trust-building**: Building trust between regulators and patient groups is particularly challenging in rare disease contexts where patient populations are limited.

Key opportunities

- **Perspective as expertise**: Recognising lived experience and patient perspectives as valuable expertise enhances decision-making processes and ensures discussions remain grounded in patient needs.
- **Partnership model**: Viewing patients as partners rather than adversaries opens doors to richer collaboration and more robust data collection.
- **Improved health outcomes**: Evidence demonstrates that engaging patients in healthcare decisions at both individual and system levels leads to better outcomes and more appropriate decisions for the communities being served.
- **Early multi-stakeholder dialogue**: Bringing regulatory bodies, healthcare providers, industry, and patient representatives together from the earliest stages of drug development enables better alignment of perspectives and potentially faster, higher-quality treatment solutions.

Session 4: What best practices and considerations should guide the practical and sustainable implementation of PE in an agency?

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

Hayley Chapman, Executive Director - Operations, Patient Focused Medicines Development, The Synergist

Background

Patient Focused Medicines Development (PFMD) is a global, nonprofit, multi-stakeholder consortium dedicated to elevating patient voices in product development and healthcare systems. PFMD brings together diverse stakeholders including industry representatives, patient communities, academia, regulators, and HTA bodies through a collaborative approach. PFMD has co-created numerous open-access resources and tools, including the Patient Engagement Management (PEM) Suite and Synapse, a digital network and platform mapping patient engagement (PE) activities globally.

Global PED Navigator

PFMD and its collaborators co-created the Global Patient Experience Data (PED) Navigator as a foundational tool for clarifying and structuring the understanding of PED. It addresses five critical questions: identifying patient needs most important to those living with diseases or undergoing treatment, and what methods can be used to prioritise those needs, determining appropriate measurement methods and tools, understanding who uses this data, and establishing when in the product development lifecycle, and in the healthcare journey, this information is most valuable.

The tool encompasses various methodologies including clinical outcome assessments (COAs), qualitative research methods and patient preference studies. It emphasises that PED must be fundamentally grounded in PE, requiring patient involvement in design, generation, contextualisation, and dissemination of data.

Systematic integration of PE and PED

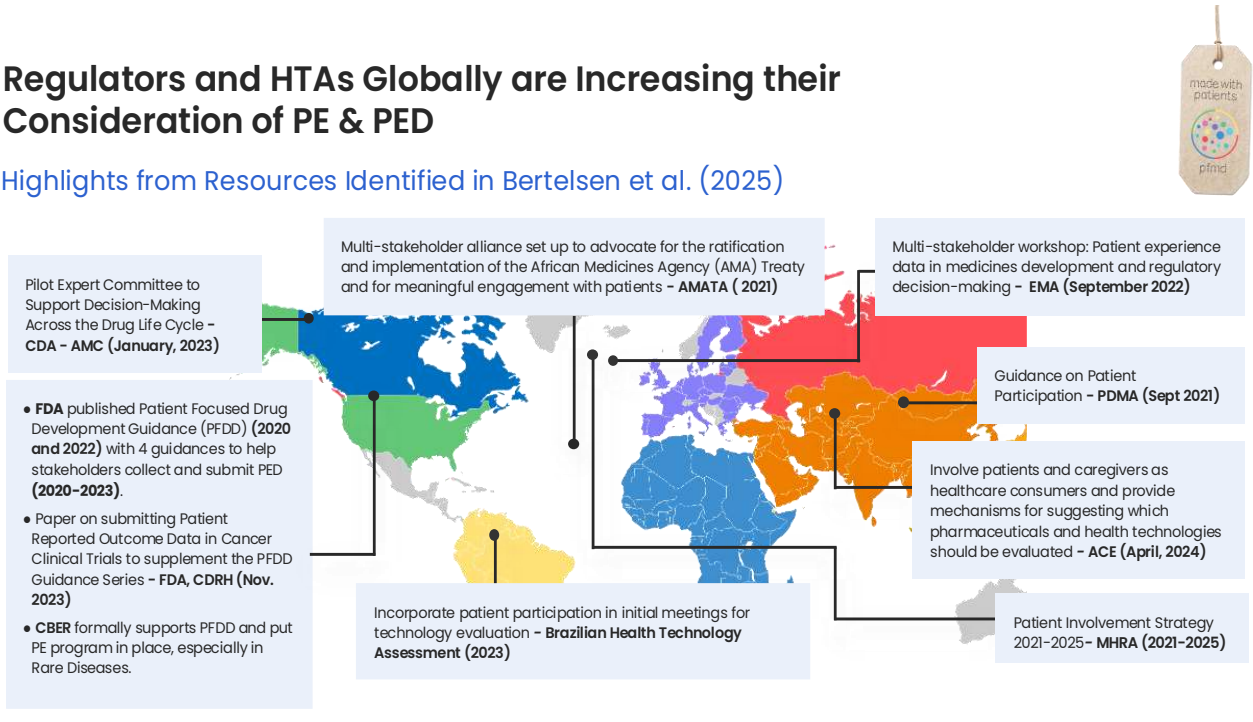
The global healthcare sector is experiencing a shift towards systematic integration of PE and PED. This transformation is evident across multiple stakeholders: regulators are showing increased consideration for patient input, HTA bodies are evolving their approaches, and patient communities are developing stronger expectations for meaningful participation in healthcare decisions.

Notably, the investment community, including private equity and venture capital firms, is demonstrating growing interest in understanding patient impact of new therapies. This interest stems from both risk mitigation perspectives and environmental, social, and governance (ESG) considerations.

Studying the PE/PED landscape

Recent landscape research examining publicly available PE and PED resources from HTA bodies and regulators worldwide reveals significant growth in this area. Analysis shows 75% of identified resources focus on PE, 46% address PED, and crucially, 30% combine both approaches.

The most significant finding indicates that combined PE and PED resources were entirely absent in the previous landscape study in 2023, demonstrating rapid evolution in this field. This growth is exemplified by recent initiatives such as the US Food and Drug Administration (FDA)'s second workshop on PED methodological challenges and the European Medicines Agency's PED reflection paper.



Challenges and opportunities

Three primary challenges stand in the way of patient input influencing regulatory and HTA decisions. These are: lack of definitional clarity regarding PE and PED terminology, incomplete feedback loops and limited transparency, and infrastructure asymmetry reflecting varying maturity levels across different agencies and regions.

Opportunities for advancement include earlier integration of patient input, potentially extending to preclinical stages and target product profiles, enhanced methodological rigour with structured, transparent approaches linking insights to decision making, shared accountability through multi-stakeholder collaboration, and a cultural shift from consultation to co-creation.

Conclusion

Patient communities should not merely have seats at decision-making tables but should actively participate in constructing those tables. This shift to co-creation requires collaborative efforts across industry, patient communities, regulators, HTA bodies, healthcare professionals, and academia. The goal is establishing systematic integration of PE-informed PED throughout healthcare development and delivery, supported by robust methodological approaches and transparent decision-making processes that demonstrate clear connections between patient input and healthcare outcomes.

Global vs Asia-Pacific findings from HTAi 360 studies

Fiona Pearce, Co-Chair of the HTAi Patient and Citizen Involvement in HTA Interest Group (PCIG), and Senior Advisor, Agency for Care Effectiveness (ACE), Singapore

Background

Established in 2005 as a small group of dedicated individuals, the HTA International (HTAi) Patient and Citizen Involvement in HTA Interest Group (PCIG) has evolved into a robust network of over 300 members spanning more than 40 countries. The group operates under a multi-stakeholder steering committee and maintains several key objectives: promoting robust methodologies that incorporate patient and citizen perspectives into HTA, advocating for systematic and transparent inclusion of patient input across all HTA lifecycle stages, sharing best practices whilst building global capacity, and supporting countries with limited HTA experience to incorporate patient perspectives effectively.

PCIG resources and projects

Over its 20-year history, PCIG has developed an extensive collection of tools and resources that have standardised and strengthened approaches to patient involvement across different HTA systems whilst maintaining sufficient flexibility for local adaptation. The PCIG currently supports four active projects run by different project subcommittees, all focused on research related to patient involvement in HTA processes.

A particularly significant contribution is the second edition of *Patient Involvement in Health Technology Assessment*, featuring 33 chapters from over 120 global experts, many of whom are PCIG members. This edition builds on the first one published in 2017, and notably expands discussions to include emerging HTA systems, particularly those in the Asia Pacific region, providing both conceptual frameworks and practical implementation examples.

Current state of patient participation in HTA

Recent research reveals that patient participation occurs most commonly during assessment and appraisal stages of the HTA process, primarily through evidence submissions. However, participation remains uneven across the entire HTA lifecycle, indicating there's room for improvement.

Comparing established HTA systems (primarily European) with emerging Asia-Pacific systems reveals several critical differences. In established systems, patient organisations demonstrate greater awareness of HTA processes and available engagement opportunities, supported by agency-led efforts in education and awareness-raising. Participation mechanisms in these systems are more formalised, including submission processes, advisory committee membership, and stakeholder consultations.

Conversely, many emerging HTA systems in the Asia-Pacific show limited awareness, with participation primarily occurring through pilot projects, research collaborations, or informal consultations rather than established pathways. Support structures also differ, with established systems providing training, template resources, toolkits, and dedicated engagement staff, whilst many of these supports remain under development in emerging systems.

Asia Pacific 360 study

To address these gaps in understanding, the HTAi PCIG has initiated the Asia Pacific 360 study, conducting a comprehensive landscape analysis of patient involvement practices across the region. The study encompasses jurisdictions with both formal HTA systems and those with more informal processes, recognising that a one-size-fits-all approach would be inappropriate given the region's system diversity.

The study employs a two-track methodological approach: gaining detailed qualitative insights through literature reviews and three multi-stakeholder workshops (completed), and broader regional perspectives through stakeholder surveys and interviews (ongoing). The outputs from both tracks will inform the development of recommendations to strengthen patient involvement and a living repository of patient involvement practices from each jurisdiction that will be made publicly available and updated as processes evolve.

Key findings

Findings from the Asia Pacific 360 study so far demonstrate strong consensus among regional stakeholders that patient involvement in HTA is both valuable and essential for achieving fair, context-appropriate, and patient-centred healthcare decisions. Meaningful engagement requires early involvement, capacity building, transparent co-design processes, structural support, and mandates to move beyond tokenism.

Key enablers identified include recognising patients as experts in their lived experience, raising awareness of patient input value, capacity building through training programmes, transparent involvement frameworks, best practice sharing, and adequate funding (see below). Major barriers encompass limited HTA awareness among patient groups, complex technical language, lack of formal participation processes, resource limitations, insufficient feedback mechanisms, and hierarchical institutional cultures.

Conclusion

The HTAi PCIG Asia Pacific 360 study represents a crucial step towards strengthening patient involvement in HTA across the Asia-Pacific. Future recommendations will likely focus on establishing clear participation pathways, strengthening capacity building, improving transparency and feedback mechanisms, and demonstrating institutional commitment to patient involvement. The ultimate goal is creating HTA systems that recognise patient experience as essential evidence whilst fostering regional collaboration and shared learning to ensure engagement practices continue evolving to meet patients' needs effectively.

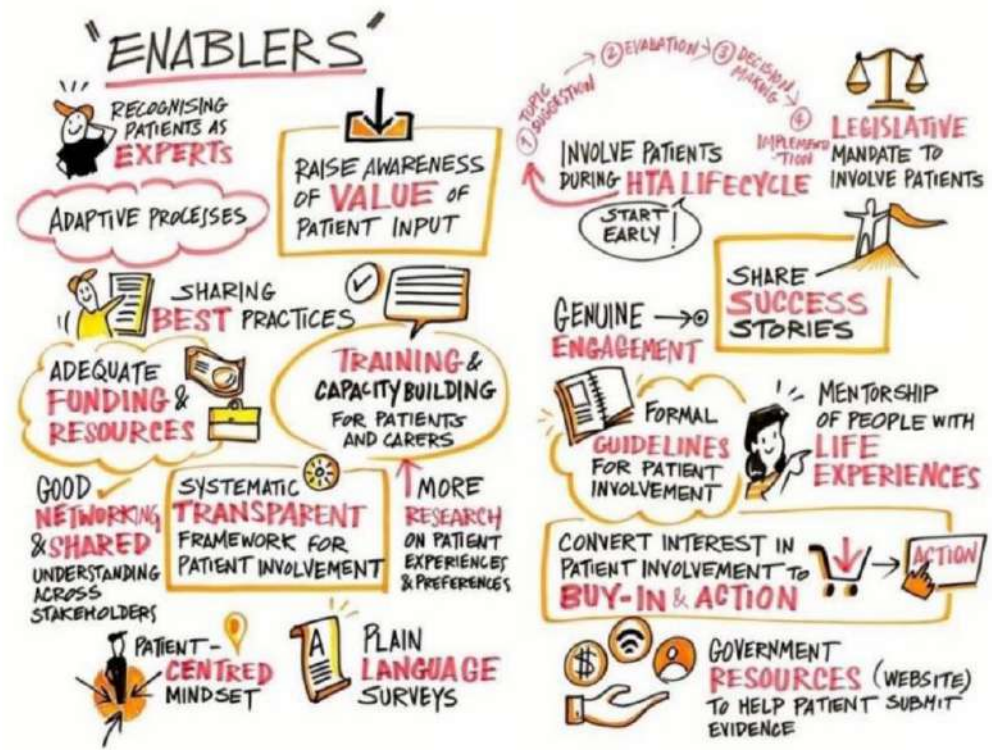


Image created by patient advocate Ekawat Suwantaroj from Thailand, August 2025.

Patient voice in regulatory evaluations and lifecycle engagement

Integrating patient engagement and experience data into EMA evaluations

Prof Steffen Thirstrup, Chief Medical Officer, European Medicines Agency (EMA)

Stakeholder engagement framework

The European Medicines Agency (EMA) has developed a comprehensive framework for engaging with patients, using various mechanisms such as keeping patients informed, consulting with them, involving them in guideline development, and cooperating with them wherever possible. EMA's engagement approach is built upon several key principles: transparency, independence and integrity in organisational relationships, accountability, appropriate interaction methods, broad representation ensuring EU-wide patient representation, effective communication, and continuous improvement.

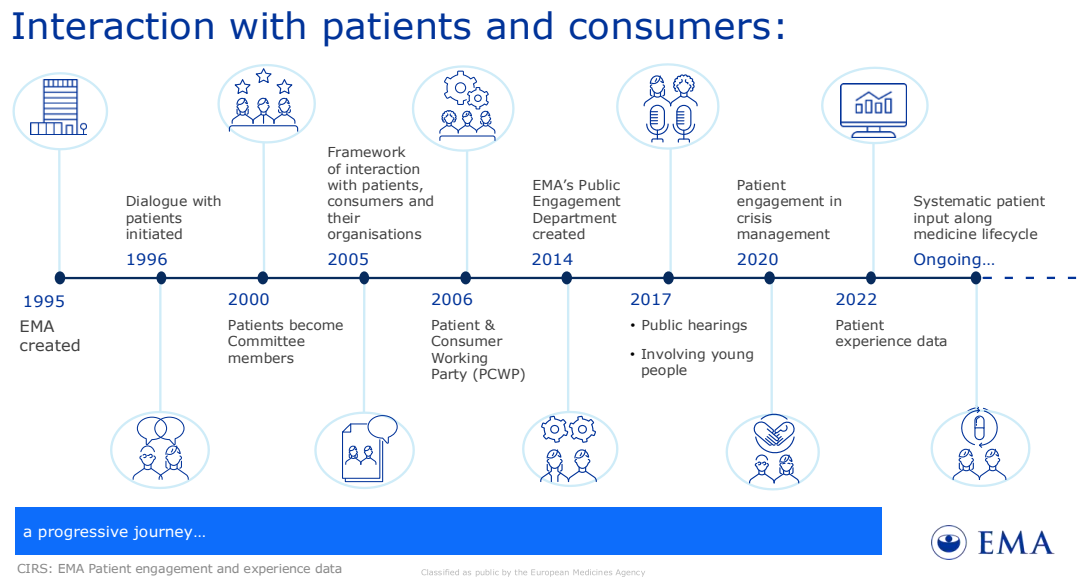
Transparency measures

Patient stakeholders must meet specific criteria to participate in EMA activities, for example, they cannot receive direct funding from single pharmaceutical companies. Individuals are required to sign declarations of conflict of interest, which are published, along with their CVs, on the EMA website. Patient organisations must similarly meet eligibility criteria, including publishing their financial support information on their websites, distinguishing between funding from groups of pharmaceutical companies versus single companies.

In return, EMA commits to full transparency by publishing all agendas, meeting minutes, committee highlights, and proactively publishing clinical trial data. Patient representatives are included in public hearings and public meetings when they occur.

Historical development

Patient engagement at EMA has evolved over three decades since the agency was founded (see below). The journey began in 1996 with early engagement initiatives, progressing to patients becoming non-voting committee members in 2000. In 2006, the Patient and Consumer Working Party was established, followed by the creation of a dedicated engagement department in 2014.



Levels of patient involvement

Patient engagement within the EMA operates at multiple levels depending on the specific role and representation required. Patients representing the broad patient community serve as members of the Management Board alongside the heads of the 27 national medicines agencies. Disease-specific patient representatives participate in working parties, serve as consultants for policies and guidelines, and contribute to public workshops.

Individual patients serve as expert witnesses for scientific advice, ad hoc scientific consultations, document reviews, and committee consultations. EMA provides training and resources to help patient representatives become familiar with regulatory processes and terminology.

Legislative changes

Significant changes are anticipated following the review of the EU Pharmaceutical Legislation. The EMA committee structure will be reduced from six to potentially two or three committees, with patient representatives gaining voting rights for the first time. Initially proposed as four patient representatives with voting rights, the final compromise resulted in two patient representatives sharing one vote between them.

Scientific advice

Patient involvement in scientific advice has become increasingly important, particularly when companies propose using patient experience data (PED). Statistics show high levels of patient engagement in EMA scientific advice procedures, with patient input leading to further reflections in approximately half of cases.

PED Reflection Paper

The PED Reflection Paper sets out 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 draft concept paper underwent public consultation with extensive feedback received, and finalisation is expected by 2026.

Challenges and solutions

Several challenges have been identified in patient engagement, including finding suitable English-speaking patients across the diverse European population, managing availability constraints, addressing conflicts of interest, managing expectations, and ensuring appropriate representativeness. A significant breakthrough was achieved in addressing remuneration issues. Patient representatives now receive the same compensation rates as other experts, including hourly rates for work and coverage of travel and accommodation expenses.

Conclusion

Patient engagement is essential for bridging the gap between clinical trial data and real-world medicine use, bringing everyday aspects of living with disease into scientific discussions. EMA’s approach emphasises building trust in regulatory science through transparency, awareness, and community engagement. Success requires a stepwise approach with mutual learning, clear expectation management, defined roles, and appropriate compensation for patient contributions.

Operationalising patient engagement in HTA submissions

Dr Nick Crabb, Chief Scientific Officer, National Institute for Health and Care Excellence (NICE)

The imperative for patient involvement

Patient contributions provide several critical elements to the evaluation process. They offer new evidence and information that may not be captured through traditional clinical research methods. Patients can challenge existing evidence or conventional wisdom, particularly when clinician-identified outcomes may not align with patient priorities and experiences. Additionally, patient input provides essential qualitative context to complement quantitative data, offering insights that professional assumptions might otherwise overlook.

Operational framework for patient engagement

NICE has established multiple pathways for incorporating patient voices throughout its work. The company submission process, whilst primarily focused on clinical and cost-effectiveness data, may reflect patient perspectives through company engagement activities. However, the most direct patient input occurs through stakeholder submissions from consultees and experts, where patient organisations, individual patients, professional health organisations, commissioners, and clinical experts can submit evidence for consideration. The guidance consultation period provides additional opportunities for patient input, which may help to address evidence gaps that emerge during the evaluation process.

Patient representation in committees

Independent advisory committees serve as the cornerstone of NICE's guidance development and are responsible for developing recommendations. The committee composition includes two lay members who serve as full voting members, expected to reflect patient and public views. These lay members possess equal decision-making authority alongside clinical and academic members.

Additionally, patient experts provide advisory input to committees, speaking at meetings and offering guidance, though they do not participate in formal voting processes. This dual approach ensures both direct patient representation in decision making and access to specialised patient expertise during deliberations.

Patient evidence submissions

Submissions from nominated patient experts and patient organisation stakeholders form part of the evidence considered in medicines evaluation. A comprehensive template and guide supports these submissions, focusing on key areas including the lived experience of conditions, current care pathways and unmet needs, potential advantages and disadvantages of new medicines, and considerations around patient subgroups and equity issues. Patient organisations may also include other information, for example, survey data from their members, in their submissions.

Summary of Information for Patients

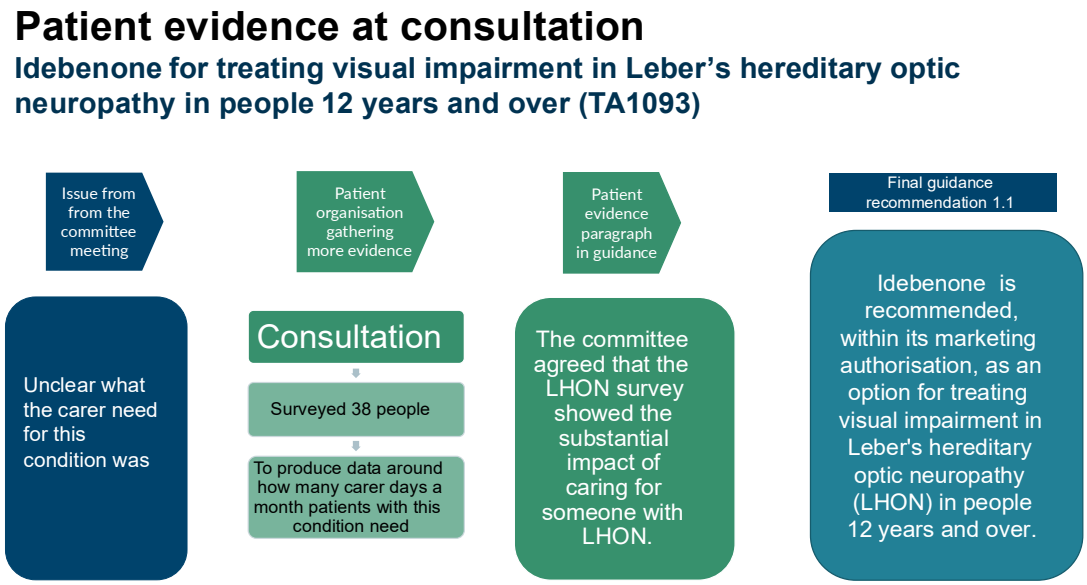
The Summary of Information for Patients (SIP) is a plain English summary of the company's submission to help patients and patient groups input into an HTA. The SIP is written by the company as part of their submission to NICE. The SIP was developed through international multi-stakeholder collaboration to support inclusivity, transparency and health literacy. The SIP framework includes templates, guides for both patient organisations and HTA bodies, and introductory materials, with international adoption demonstrating its broader value.

Impact of patient contributions

Committee member feedback demonstrates the tangible value of patient input in the NICE evaluation process. Patient evidence helps committee members understand quality of life impacts and lived experiences, whilst enabling verification that company submissions accurately reflect patient perspectives.

The impact of patient input appears greater in situations where clinical evidence is limited. With increasingly adaptive licensing approaches used by regulators, medicines often launch with clear safety profiles and some efficacy evidence, but with uncertainty regarding the magnitude of efficacy and quality of life impacts. In this evolving evidence landscape, patient input becomes increasingly critical for filling evidence gaps and explaining missing or inadequate information in formal submissions.

The NICE evaluation of idebenone for rare disease Leber's hereditary optic neuropathy demonstrates the meaningful impact patient evidence can have in committee decision making (see below).



Future directions

NICE has developed a comprehensive "Working Alongside People and Community Strategy" outlining planned improvements to engagement and involvement processes. This strategy encompasses three key networks designed to enhance patient evidence identification and utilisation across NICE's work programmes.

NICE sends feedback surveys to patient organisations when guidance is published, patient experts after they have attended committee meetings, and lay members when they leave committees. Various initiatives are underway to improve communication about how patient input influences guidance decisions, with ongoing efforts to enhance transparency and demonstrate the genuine impact of patient contributions on final recommendations.

Conclusion

Patient engagement in HTA represents both a practical necessity and an ethical imperative for developing meaningful healthcare guidance. The systematic integration of patient perspectives throughout NICE's processes demonstrates that meaningful patient involvement is operationally feasible and demonstrably valuable. As the evidence landscape continues to evolve, the role of patient input becomes increasingly critical for addressing evidence gaps and ensuring that guidance reflects real-world patient experiences and priorities.

Panel discussion: Measuring and reporting the impact of patient input in regulatory and HTA decision making

Moderated by Ratna Devi, Board Member, International Alliance of Patients' Organizations (IAPO)

Panellists:

- **Patient perspective - Chris Munoz,** President, Philippine Alliance of Patient Organizations (PAPO)
- **HTA agency perspective – Dr Miyoung Choi,** Director of Clinical Evidence Research, and Director of NECA GRADE Center, National Evidence-based Healthcare Collaborating Agency (NECA), South Korea
- **Regulatory agency perspective – Prof Anthony Lawler,** Head, Therapeutic Goods Administration (TGA), Australia
- **Company perspective – Rob Berlin,** Head, Regulatory Policy, Vertex, USA

Several themes arose during the panel discussion:

Defining 'good' patient data

Different stakeholders (regulators, HTA agencies, patient groups, policymakers) view patient experience data (PED) through different lenses, and this is appropriate because each manages different risks and endpoints. A 'good decision' incorporates all perspectives and evidence types - whether meta-analysis, randomised controlled trials, or patient-reported experiences. The challenge is not creating a hierarchy of evidence but recognising different forms of evidence have different values in different contexts.

Valuing patient input as core evidence

Supporting patient engagement should be an integral part of regulatory work, similar to how agencies would support toxicology or clinical pharmacology expertise. The key question is whether regulatory agencies view patient input transactionally on a decision-by-decision basis or as something that fundamentally makes them better regulators.

Transparency and trust

Transparency requires clear and upfront communication about how patient input will contribute to and has contributed to final decisions. Transparency helps to build trust, a key component for effective collaboration between agencies and patient communities.

Increasing capacity and health literacy

Patients need to understand that negative outcomes don't always mean a drug is clinically ineffective – for example, pricing, indication specificity, or health system factors may influence HTA recommendations. Improving health literacy among both patients and prescribers is essential for meaningful communication about regulatory and HTA processes.

Patient groups across the Asia-Pacific are at varying maturity levels, requiring educational and financial support for patient involvement, ideally from governments.

Incorporating PED in regulatory processes

PED often contextualises disease understanding but doesn't always translate clearly into regulatory decisions. For example, the FDA's PED table shows what type of PED was submitted and considered in the review, but not how the PED influenced decisions. Companies should be clearer in their applications about how patient input influenced study design and endpoint selection.

PED is rarely included in drug labelling because regulators often don't consider it to be essential scientific information, yet including it would validate patient input and help providers understand product performance better.

Global access and equity

Pharmaceutical companies have a responsibility to ensure medicine access anywhere in the world, not just in high-income countries. When patients contribute to global clinical trials but don't receive access to approved medicines in their regions, it raises serious equity concerns. Companies must accept the risk of registration and maintain access even if market conditions are unfavourable or only one patient in a country needs the medicine.

Session 5: 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: Policy enablers to aid integration of PE and PED in decision making

Chair: Prof Steffen Thirstrup, Chief Medical Officer, EMA

Rapporteur: Stephanie Chen, Associate Director, Asia Pacific Regulatory Policy, MSD, Singapore

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.

Policy changes and guidance needed

The group agreed that, to encourage alignment on the value of PE/PED, agencies must align with global guidelines whilst adapting them to their local contexts. Global guidelines serve as overarching advice, but local markets must determine what makes sense within their specific healthcare environments.

In addition, regulatory and HTA agencies should align early in the development process on agreed endpoints, with regulators perhaps taking the lead on harmonising important common endpoints. The group agreed that harmonisation related to PE/PED is more feasible among regulators than HTA bodies, as HTA involves local context considerations. This approach may make PE/PED more useful and avoid duplicative work for companies.

Addressing company challenges

Structured channels for companies to engage regulatory and HTA agencies on PED are essential for predictability. Currently, such conversations occur on an ad hoc basis, meaning PED may be gathered too late in the development process. Pre-competitive dialogue involving multiple companies working in the same therapeutic area, together with regulatory and HTA agencies, could help to reduce duplication.

When asked about how to encourage routine integration of PE/PED in development, the group concluded that "routine" may not be appropriate terminology, as PE/PED use depends on its relevance to support the development process or complement the evidentiary picture. The application is highly case-by-case and depends on the therapeutic area.

Adapting global standards and frameworks

To strengthen the credibility and acceptance of PE/PED in company development, regulatory submissions, and HTA submissions in the Asia-Pacific, adapting global guidance is key. On the regulatory front, FDA has a series of Patient-Focused Drug Development guidance, the ICH E22 Guideline on patient preference studies is under consultation, and the EMA PED Reflection Paper is being finalised. These guidelines establish regulatory standards among regulators and provide useful alignment opportunities for those utilising reliance. For HTA agencies, global guidance should detail common aspects among markets whilst acknowledging differences in healthcare contexts that require consideration.

Flexibility mechanisms – such as use of RWE/RWD, surrogate endpoints, adaptive/iterative pathways – are needed to support rare disease and precision medicine development due to evidence gaps.

Considerations for reliance mechanisms

Where reliance mechanisms are used for regulatory decision making, preserving and contextualising foreign PED locally – without losing relevance or creating duplication – presents challenges. The approach depends on whether PED was used by the reference agency for pivotal decisions and how assessment reports are interpreted. Local PED should be generated to fill gaps where necessary.

When considering foreign PED in decision making, regulatory and HTA agencies should be aware of differences to the local context, such as in diagnostic criteria, disease manifestation, healthcare systems, epidemiology, and standard of care or comparator variations. Despite these differences, agencies should consider extrapolating foreign PED, without diluting the original patient insights.

Recommendations for further work and research

- 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 patient-centric 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.

Breakout B: Assessing and communicating the impact of PE and PED

Chair: Prof Manuel Antonio Espinoza, Associate Professor, Division of Health Economics, Policy and Management, The University of Hong Kong

Rapporteur: Joanne Tan, Senior Manager, Regulatory Affairs, APAC, BioMarin, Hong Kong

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.

This breakout group 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.

Current visibility in public-facing documents

Examples shared by the group revealed variations in where and how PE/PED is referenced in public-facing regulatory and HTA documents. PE/PED guidance are established and actively used in regulatory pathways in the US, EU, Australia, Japan, and China, but this is not commonly observed in other jurisdictions. For HTA pathways, PE/PED references and processes are established in Australia, Ireland, and Singapore for initial evaluations, though PE/PED use appears more common during drug re-evaluations.

A key challenge identified was how to integrate PE/PED into clinical trial applications without impacting speed to global approval for a multi-centre clinical trial. In addition, it is difficult to quantify the contribution of PE/PED to regulatory and HTA outcomes, though its use in providing contextualised disease and unmet need information is clear.

Communication of use and impact

From a regulatory perspective, communication of PE/PED occurs upstream during Investigational New Drug (IND) and clinical development planning phases. However, at the approval stage, PE/PED is not usually specifically highlighted in approvals or labels. For HTA, whilst assessment reports are openly accessible in some markets, there is limited communication about how PE/PED has impacted decisions.

The gaps include lack of open communication on PE/PED impact to patient groups or the public, despite accessible assessment reports. Communication about PE/PED impact on HTA decisions requires careful articulation to minimise misperception amongst stakeholders.

Feedback mechanisms to patients

Delivering feedback to patients should focus on statutory feedback loops or forums for multi-stakeholder communication. To distinguish meaningful integration from tokenistic practices, regulators and HTA agencies should provide written feedback (in simple language) about why certain inputs were selected whilst others were not. This enables patients to provide further relevant input into decision-making processes. Continuity and stability in feedback processes are key.

Tools, templates, and standards

The group discussed tools, templates and standards that could help to improve consistency and transparency in PE/PED reporting. While some reporting templates and tools do exist in certain jurisdictions, their adaptability to other jurisdictions remains subjective and requires further research.

For regulatory agencies, consistency in maintaining and communicating safety updates is crucial. In most jurisdictions, there is an established route for adverse event reporting and communicating labelling changes. However, there is not usually a feedback route to individual patients, who may value knowing when their reported adverse events become valid safety signals.

Challenges in impact assessment

The group found it difficult to identify quantitative or qualitative indicators that could be used to assess the impact of PE/PED in regulatory and HTA decisions. Evaluation using a mix of methods, including not only metrics but also patient narratives and quotes, may be the best approach.

Recommendations for further work and research

- 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 of activities, reducing duplication.
- 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.

Breakout 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

Background

As patient engagement (PE) and patient experience data (PED) become increasingly important inputs into regulatory and HTA decision making, attention is shifting from why patient involvement matters to how it can be implemented in a systematic, credible, and sustainable way. Across the Asia-Pacific region, patient organisations, regulators and HTA agencies are at different stages of readiness, with uneven access to skills, infrastructure, and resources needed to support meaningful PE and the generation and use of PED.

These challenges are amplified in the context of rare diseases and precision medicine, where patient populations are small, evidence is often limited or evolving, and traditional assessment frameworks may not fully capture outcomes that matter most to patients. In such settings, alignment between patient organisations and agencies—around expectations, methods, standards, and roles—is essential to ensure that patient input is not only heard, but trusted and used in decision making.

This breakout focused on the practical dimensions of capacity-building and alignment. Participants explored concrete actions agencies, companies, and patient organizations can take over the next 3–5 years to embed PE and PED across the medicine lifecycle as well as discussing shared priorities, and opportunities for collaboration that are realistic within diverse Asia-Pacific health system contexts.

This breakout group comprised of representatives from the pharmaceutical industry, regulatory and HTA agencies, and patient organisations explored the capabilities needed by both patient organisations and agencies to help embed PE and PED across the medicine lifecycle.

Infrastructure needs

The group discussed what is needed at the system level to generate, use, and meaningfully translate PE and PED. Key system requirements include appropriate tools, legal and data protection mechanisms, and addressing communication gaps that prevent meaningful stakeholder engagement.

Whilst stakeholders understand their roles in principle, uncertainty exists regarding the right format, timing, personnel, and methodology. For example, patients possessing relevant data may lack knowledge about appropriate presentation formats to make it viable evidence. Industry partners seeking this data may not know which patient organisations to approach. Regulatory bodies receiving data may lack clarity on interpretation methods, and HTA bodies may understand the importance of data but lack guidelines for converting qualitative information into quantitative metrics.

Stakeholder capacity gaps

The group highlighted capacity and capability gaps across all stakeholders, with engagement often occurring in an ad hoc rather than formalised manner. Key challenges include limited infrastructure and access to high-quality patient data, low awareness of the value of patient input, and unclear roles and mechanisms for collaboration between HTA bodies and patient organisations.

For patient organisations, there are skills gaps are in health literacy, data literacy, and evidence generation. HTA bodies require data literacy, data governance frameworks, and capacity to interpret patient evidence. Regulators need training in data governance systems as primary recipients of stakeholder data. Companies require soft skills for engagement, including facilitation, communication, and translating technical information into patient-friendly language.

Multi-stakeholder body proposal

The group's proposed solution to addressing infrastructure needs and capacity gaps was to establish a formal multi-stakeholder body to support patient engagement across the Asia-Pacific. This body would coordinate capacity building, onboard patient advocacy group representatives as recognised experts, create accessible patient data resources, strengthen data privacy protections, enable coordination and unified representation, establish formal communication channels and governance mechanisms, provide dedicated funding mechanisms, and support formal patient-academic-clinician partnerships for evidence generation.

Alignment challenges and solutions

The group agreed that current alignment gaps stem from lack of formal top-down mandates, unclear rules and expectations, limited understanding among patient organisations of regulatory and HTA systems, and the need for internal processes and capacity building within regulatory and HTA agencies. Solutions include training for regulators and HTA bodies, incentivising industry and agencies to involve patient organisations, capacity building workshops, and development of patient registries to strengthen the patient evidence base. Regional collaboration and a central coordination mechanism for patient engagement activities would help to avoid duplication and ensure consistency and impact.

Role of regional collaboration

The group discussed how existing collaborative bodies and initiatives could be leveraged to accelerate capacity building and reduce duplication in PE/PED. There could be a role for the Asia Pacific Alliance of Rare Disease Organisations (APARDO) to assist country-level patient organisations with registry development and capacity building. While there are existing collaborative structures/organisations within the regulatory and HTA communities that issue recommendations for PE practices, these could go even further by assessing the implementation of PE practices in regulatory and HTA agencies.

Other suggested collaborative opportunities included creating compensatory consultation roles for patient advisory groups in national bodies to facilitate co-creation, developing region or country-specific tools and training programme based on the European Patients' Academy (EUPATI) experience, and establishing a regional information-sharing platform under the Coalition to Accelerate Patient Engagement in Asia-Pacific (CAPE).

Practical steps to build sustainable PE/PED capacity

Proposed short-term actions included establishing consistent funding and open communication channels, clarifying patient engagement terminology across the healthcare sector, and incorporating patient input and feedback mechanisms from preclinical stages.

Long-term actions were to develop clear policies in patient engagement, digitalise patient data and registries, promote cross-country learning to improve data collection and management infrastructure, and mandate capacity building for regulators and industry to facilitate patient advocacy group engagement.

Recommendations for further work and research

- 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.

Session 6: PE and capacity building – How should this evolve and what are some of the challenges?

Panel discussion: What is needed to address the challenges patients face and ensure support for patient organisations in their advocacy roles?

Moderated by Prof John Skerritt, Enterprise Professor in Health Research, University of Melbourne, Australia

Panellists:

- **Patient perspective - Wee Seng Phua**, Executive Director and Chief Rare Advocate, Rare Disorders Society Singapore
- **Company perspective – Susumu Kitamura**, Head of Global Regulatory Affairs (Japan), Sanofi, Japan
- **Regulatory agency perspective – Dr Iris Conela Tagaro**, Medical Specialist III, Head of Clinical Research Section, Philippines Food and Drug Administration
- **HTA agency perspective – Dr Phatcharee Chukaew**, Researcher, Health Intervention and Technology Assessment Program (HITAP), Thailand

Several themes arose during the panel discussion:

Funding and sustainability challenges

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. Transparency about funding sources and maintaining organisational independence are key.

Increasing capacity and capability

Effective patient engagement requires investment in capacity building on both sides. Patient organisations need training to understand regulatory and HTA processes, while agencies and companies need to understand patient-lived experience and the value of patient data. Sustaining patient engagement requires more than invitations to meetings and roundtable discussions—it demands investment in training programmes and dedicated staff to support patient involvement.

Patient organisations as collaborators

Patient organisations cannot be experts in all technical aspects of regulatory science, clinical development, or health economics, but they can be excellent collaborators who bridge knowledge gaps. Building credibility requires demonstrating capabilities through programmes and initiatives—such as annual awareness campaigns and multi-stakeholder forums—earning "a seat at the table" through consistent, valuable contributions.

Institutionalising patient engagement in regulatory and HTA processes

A critical gap exists across many Asia-Pacific regulatory and HTA systems: the lack of formal mechanisms to systematically integrate patient input into decision-making processes. Potential solutions include establishing structured communication channels for patient groups, formally including patient involvement in policies and

guidelines, and recognising patient experts with the same credibility as clinical experts. Agencies should activate existing processes and safeguards—such as confidentiality agreements, conflict of interest policies, and compensation frameworks—to support patient engagement just as they do for external clinical experts.

Geographic disparities in patient engagement

In multinational companies, patient engagement is often concentrated in the US and EU, limiting engagement in Asia and other regions. As patient engagement 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.

Leveraging technology and data

Technology offers opportunities to gather and organise patient experience data (PED) more systematically. Artificial intelligence (AI)-based social media analysis can help collect patient insights at scale, identifying critical patient priorities that might be undervalued in traditional clinical endpoints. However, appropriate validation and local contextualisation are essential, especially for rare diseases where uncertainty is high.

Consistent patient engagement across the medicine lifecycle

Patient engagement must occur throughout entire processes, not just at decision points. In HTA, patient voices should be integrated from topic nomination through prioritisation, assessment, and appraisal. Patient input helps improve relevant outcome measures that truly matter to patients, identifies real-world feasibility barriers before implementation, and improves equity considerations in decision making—such as balancing fairness with cost-effectiveness when making reimbursement decisions.

For industry, engaging patient groups during protocol preparation and clinical study development ensures patient perspectives shape research design from the beginning. Patient input on unmet needs and treatment access challenges can also guide prioritisation of product reviews when regulatory agencies face backlogs. Companies should aim to establish strong global-level alignment on patient engagement and patient experience data, advancing with a unified and consistent strategic direction across their organisation.

Engaging umbrella versus disease-specific patient groups

National regulatory authorities are sometimes challenged with whether to engage with umbrella patient organisations or disease-specific groups. Engaging umbrella organisations first can be more efficient because they are knowledgeable about individual member organisations and their capabilities, and can help identify representatives that best match specific disease areas for regulatory engagement. This approach allows regulatory authorities to leverage the umbrella organisation's broader knowledge and network while still accessing disease-specific expertise when needed.

Panel discussion: The next five years – Shaping the future of PE and PED in regulatory and HTA decision making in the Asia-Pacific

Moderated by Prof John Skerritt, Enterprise Professor in Health Research, University of Melbourne, Australia

Panellists:

- **Patient perspective – Nadiah Hanim,** President, Malaysia Rare Disorder Society
- **Company perspective – Dr Nikki Kitikiti**, Vaccines External Engagement & Policy Lead, Takeda, Singapore
- **Regulatory agency perspective – Prof Steffen Thirstrup,** Chief Medical Officer, EMA
- **HTA agency perspective – Dr Li-Ying (Grace) Huang,** Senior Director, Division of HTA, Taiwan Center for Drug Evaluation
- **HTA agency perspective – Dr Nick Crabb,** Chief Scientific Officer, NICE, UK

The final multi-stakeholder panel of the workshop focused on the evolution of PE and PED in regulatory and HTA decision making in the Asia Pacific over the next five years.

Key themes of discussion:

Shifting patient expectations

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.

Opportunities through technology

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.

Joint scientific advice

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.

Early and strategic engagement

PE should inform R&D and clinical trial design from the earliest stages, including influencing target product profiles, trial endpoints, and appropriate patient-reported outcomes. This "product by design" approach prevents duplication of patient consultations later.

Collaboration and contextualisation

Panellists highlighted that HTA agencies alone cannot independently develop all aspects of patient engagement infrastructure and methodologies, and ecosystem-wide collaboration is essential.

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.

Barriers identified:

- **Policy and regulatory frameworks** - Lack of clear legislation and guidelines for cross-border data sharing, data privacy and protection, and data governance in the Asia-Pacific region.
- **Limited clinical trial participation** - Most trials conducted in the US and Europe; Asian patients engaged later in development, missing early input opportunities.
- **Resource constraints** - Regulatory and HTA bodies requesting duplicate consultations from patient groups; limited time and financial resources of patient organisations.
- **Mindset and trust issues** - Limited recognition that patient expertise is equal to, or sometimes more valuable than, clinical/economic data alone.
- **Lack of traceability** - Insufficient mechanisms to show patients how their input influenced (or didn't influence) final decisions, eroding trust.
- **Conflicts of interest management** – Declaration of funding sources and conflicts of interest management not well-developed in Southeast Asia.

Potential solutions:

Multi-stakeholder dialogue

- Promote joint scientific advice where industry can consult regulators, HTA bodies and patients simultaneously.
- Move toward disease-level pre-competitive dialogue rather than product-specific dialogue.

Structured data frameworks and standards

- Adopt standardised, validated patient-reported outcome measures.
- Implement secure, independently governed digital platforms where patients control their own data.
- Develop regional data sharing legislation and governance frameworks.

Transparency and traceability mechanisms

- Create documented feedback loops showing how patient insights influenced regulatory and HTA assessments.
- Establish public registries for conflict-of-interest and funding disclosures.

Early engagement in clinical development

- Increase clinical trials in the Asia-Pacific to enable patient engagement from early development stages.

Sustainable models for patient participation

- Respect time and resource constraints of patient groups.
- Provide adequate compensation and support.
- Build capacity within patient organisations.

Mindset shift

- Recognise patient expertise as distinct, valuable knowledge that complements (not replaces) clinical and economic data.
- Move beyond "motherhood statements" to intentional, active inclusion of patient voice.

Regional cooperation and learning

- Build sustainable frameworks across Asia through sharing of experiences and joint learning.
- Leverage regional cooperation among regulatory bodies, HTA agencies, patient groups, academia, and industry.

Conclusion

The workshop highlighted both the progress made and the complexities that remain in embedding meaningful patient involvement across regulatory and HTA decision making in the Asia-Pacific region. While there is increasing recognition of the value of patient perspectives—particularly in areas of high uncertainty such as rare diseases and precision medicine—approaches to engagement remain heterogeneous, reflecting differences in infrastructure, experience, and available resources across jurisdictions. These variations underline the need for context-specific strategies that build on existing practices while enabling alignment where possible.

A key theme throughout the discussions was the importance of moving from ad hoc engagement toward more structured, purpose-driven approaches that integrate patient input early in the development and assessment lifecycle. Ensuring clarity around the role, influence, and limitations of patient involvement is essential, both to enhance the quality and relevance of contributions and to build trust among all stakeholders. In this context, strengthening capabilities within agencies, industry, and patient organisations was identified as a critical enabler of more effective and sustainable engagement practices.

The workshop also reinforced the value of regional collaboration in accelerating learning and reducing duplication. Sharing experiences, tools, and emerging best practices across the Asia-Pacific region can help to address common challenges, such as limited guidance, resource constraints, and variability in processes for incorporating patient input. At the same time, fostering collaboration with international initiatives and leveraging global evidence can support the development of more consistent approaches while respecting local healthcare priorities and decision-making frameworks.

Looking ahead, sustained progress will depend on continued dialogue and coordinated action across stakeholders to translate principles into practice. This includes developing clearer guidance on the generation and use of patient-informed evidence, improving transparency on how patient input influences decisions, and investing in the long-term support and capacity of patient organisations. Ultimately, advancing patient involvement in the Asia-Pacific region will require a collective shift from recognising its importance to systematically embedding it within regulatory and HTA systems, with the shared goal of supporting more informed, inclusive, and patient-relevant decision making.

List of attendees

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

Patient organisations		
Nadiah Hanim	President	**Malaysia Rare Disorder Society**
Araceli Lanorio	President	**Neurofibromatosis Friends Philippines**
Hwan Ruey (Eric) Liu	Secretary General	**Taiwan Young Patients Association, Chinese Taipei**
Christopher Munoz	President	**Philippine Alliance of Patient Organizations**
Wee Seng Phua	Executive Director and Chief Rare Advocate	**Rare Disorders Society Singapore**
Nidhi Swarup	Chair	**Alliance of Patients' Organizations, Singapore**
Dr Durhane Wong-Rieger	President/President & CEO	**Asia Pacific Alliance of Rare Disease Organisations and Canadian Organization for Rare Diseases, Canada**
Regulatory agencies		
Dr Ying Chen	Center for Drug Evaluation	**National Medical Products Administration, China**
Ooi Jin Cheong	Senior Principal Assistant Director	**National Pharmaceutical Regulatory Agency, Malaysia**
Mindy Cher	Senior Principal Assistant Director	**National Pharmaceutical Regulatory Agency, Malaysia**
Shin Ee Haw	Pharmacist	**National Pharmaceutical Regulatory Agency, Malaysia**
Mdm Wan Noraimi Wan Ibrahim	Director	**National Pharmaceutical Regulatory Agency, Malaysia**
Prof Taruna Ikrar	Chairperson	**Indonesian Food and Drug Authority**
Dr Tri Asti Isnariani	Director of Drug Registration	**Indonesian Food and Drug Authority**
Siti Noor Haryani Ismail	Senior Principal Assistant Director – Pharmacist	**National Pharmaceutical Regulatory Agency, Malaysia**
Hu Suk Kwan	Pharmacist	**National Pharmaceutical Regulatory Agency, Malaysia**
Mdm Rosliza Lajis	Deputy Director	**National Pharmaceutical Regulatory Agency, Malaysia**
Prof Anthony Lawler	Deputy Secretary	**Department of Health, Disability and Ageing, Australia**
Jeevanandan A/L Narayanan	Regulatory Pharmacist	**National Pharmaceutical Regulatory Agency, Malaysia**
Prasad A/L Narayanan	Senior Principal Assistant Director	**National Pharmaceutical Regulatory Agency, Malaysia**
Dr Hsiao Ling Phuar	Senior Principal Assistant Director	**National Pharmaceutical Regulatory Agency, Malaysia**
Preeyaphon Puttijugluksa	Pharmacist, Practitioner Level, Medicines Regulation Division	**Food and Drug Administration, Thailand**

Dr Noraisyah Mohd Sani	Head of New Drug Product Section	**National Pharmaceutical Regulatory Agency, Malaysia**
Dr Iris Conela Tagaro	Medical Specialist III, Head of Clinical Research Section	**Food and Drug Administration, Philippines**
Charlene Tay	Senior Principal Assistant Director	**National Pharmaceutical Regulatory Agency, Malaysia**
Prof Steffen Thirstrup	Chief Medical Officer	**European Medicines Agency**
Sher Rine Yap	Senior Principal Assistant Director	**National Pharmaceutical Regulatory Agency, Malaysia**
Naoyuki Yasuda	Special Advisor to the Chief Executive & Associate Executive Director for International Programs	**Pharmaceuticals and Medical Devices Agency, Japan**
HTA agencies and coverage bodies		
Dr Syaqirah Akmal	Deputy Director, Medical Development Division	**Malaysian Health Technology Assessment Section, Ministry of Health, Malaysia**
Agnes Chan (virtual attendance)	Director, Therapeutic Products Branch	**Health Sciences Authority, Singapore**
Dr Miyoung Choi	Director of Clinical Evidence Research, Director of NECA	**National Evidence-based Healthcare Collaborating Agency, South Korea**
Dr Phatcharee Chukaew	Researcher	**Health Intervention and Technology Assessment Program Foundation, Thailand**
Dr Nick Crabb (virtual attendance)	Chief Scientific Officer	**National Institute for Health and Care Excellence, UK**
Dr Li-Ying (Grace) Huang	Senior Director, Division of Health Technology Assessment	**Center for Drug Evaluation, Chinese Taipei**
Dr Xue Li	Department of Health Technology Assessment	**China National Health Development Research Center**
Ully Adhie Mulyani	Staff, Center for Health Resource System Policy	**Center for Health Resource System Policy, Ministry of Health, Indonesia**
Fiona Pearce (Pre-recorded presentation)	Senior Advisor, and Co-Chair of the HTAi Patient and Citizen Involvement in HTA Interest Group (PCIG)	**Agency for Care Effectiveness, Singapore**
Shawn Quek	Senior Specialist (Consumer Engagement and Education)	**Agency for Care Effectiveness, Ministry of Health, Singapore**
Dr Roza Sarimin	Head of HTA Unit	**Malaysian Health Technology Assessment Section, Ministry of Health, Malaysia**
Chotika Suwanpanich	Researcher	**Health Intervention and Technology Assessment Program Foundation, Thailand**
Due Ong The	Head of Health Financing and HTA Department	**Health Strategy and Policy Institute, Ministry of Health, Vietnam**
Lupi Trilaksono	Director	**Center for Health Resource System Policy, Ministry of Health, Indonesia**
Tisha Isabelle de Vergara	Project Technical Specialist III, Health Technology Assessment	**Department of Science and Technology, Philippines**

Pharmaceutical companies		
Rob Berlin	Head, Regulatory Policy	**Vertex, USA**
Logan Caragata	APAC Policy Head	**Roche, Malaysia**
Bindoo Chahal	JAPAC Area RA TA Head	**AbbVie, Singapore**
Sandy Chan	Director, Asia Pacific Leader, Global Regulatory Policy and Intelligence	**Johnson & Johnson, Singapore**
Stephanie Chen	Associate Director, AP Regulatory Policy	**Merck Sharp & Dohme, Singapore**
Rebecca Chng	Head, Medical Affairs, SINMAL	**Astellas, Singapore**
Lan-Sim Chong	Head of Regulatory Affairs, HP ROPU ASKAN	**Boehringer-Ingelheim, Singapore**
Ruben Duran	Director, Global Public Policy	**Merck Sharp & Dohme, Singapore**
Christina Keok	Associate Director – Regulatory Lead SEA	**Bristol-Myers Squibb, Singapore**
Susumu Kitamura	Head of Global Regulatory Affairs Japan	**Sanofi, Japan**
Dr Nikki Kitikiti	Vaccines External Engagement & Policy Lead	**Takeda, Singapore**
Helen Ling	RAPV/QA SINMAL Team Lead	**Astellas, Singapore**
Aeron Oh	Policy and Access Partner	**Roche, Malaysia**
Stephanie Ong	Head of Regulatory Affairs, Malaysia, Singapore and Brunei	**Sanofi-Aventis, Malaysia**
Helene Sou	International Regulatory Policy, Director	**Takeda, Singapore**
Joanne Tan	Senior Manager, Regulatory Affairs APAC	**BioMarin, Hong Kong**
Thipsuda Thippayawan	Market Access Director	**GlaxoSmithKline, Thailand**
Steven Xu	Director, Global Regulatory Affairs	**Chiesi, China**
Dr Svetlana Yanchuk	Country President	**AstraZeneca, Malaysia**
Celest Yong	Head, Focus Brands Singapore, Malaysia & Indonesia and Regional Market Access, APAC	**Bayer, Singapore**
Dr Sia Lee Yoong	Regulatory Policy Executive	**GlaxoSmithKline, Singapore**
Dr Arshiya Zaheer	Regional Patient Engagement Lead	**Merck Sharp & Dohme, Singapore**
Academic and research organisations		
Mugdha Barik	Senior Program Manager	**Dakshayani and Amaravati Health and Education, India**
Hayley Chapman	Executive Director - Operations	**Patient Focused Medicines Development, Canada**
Dr Ratna Devi	CEO	**Dakshayani and Amaravati Health and Education, India**
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Supplementary Information

Recommendations from Breakout C: Building capacity and alignment on PE/PED

1- Establish a Formal Multistakeholder Coordination Mechanism for Patient Engagement *Create a formal, regionally coordinated multistakeholder platform to institutionalise patient engagement and align regulators, HTA bodies, industry, and patient organisations*
Stakeholders to Implement • Primary: Regulators, HTA bodies, and patient advocacy groups (PAGs) • Supporting: industry associations, research
Timeline • Short term: Establish structured engagement platforms and communication channels • Long term: Introduce formal mandates embedding patient engagement into regulatory and HTA processes
Mode of Implementation • Establish a formal coordination body or engagement forum • Define clear roles, expectations, and governance structures across stakeholders • Create structured consultation mechanisms between patient organisations and agencies • Introduce early patient engagement across the product lifecycle (including trial design and evidence generation) • Promote regional collaboration to reduce duplication and improve knowledge sharing
Pipeline: Identify active stakeholders within respective countries -> elect country rep (1 each for regulatory, Industry, PAG) -> create regional working groups (WG) for each stakeholder with the elected reps (so PAG WG has all country leaders who represent PAG, replicable for industry and regulatory) -> WG will be responsible for policy reforms and legal and political stability; country leads will be responsible for rolling out the actions within their countries; local level stakeholders to operationalise -> fixed funding to be generated for each WG and distributed equitably within the member countries

2- Build Cross-Stakeholder Capacity for Meaningful Patient Engagement and Patient Evidence Generation

Develop coordinated training and capacity-building programmes across patient organisations, regulators, HTA bodies, and industry to enable effective participation in patient engagement and patient evidence generation

Stakeholders to Implement

- Primary: Multistakeholder coordination platform (or academia/patient organisations-led initiative)
- Supporting: Regulatory, industry,

Timeline

- Short term: Launch targeted training programmes and workshops
- Long term: Institutionalise capacity-building frameworks within agencies and stakeholder organisations

Mode of Implementation

- Provide health literacy, data literacy, and evidence generation training for patient organisations
- Train regulators and HTA bodies on interpreting and integrating patient evidence into decision-making
- Develop data governance training and frameworks within agencies
- Provide soft skills training for industry to facilitate meaningful patient engagement
- Establish mechanisms to onboard patient representatives as recognised experts, including appropriate compensation

Pipeline: identify existing bodies with most adaptable and comprehensive frameworks -> negotiate running a pilot for a dedicated course on patient engagement -> explore adopting the functional framework to country levels -> identify umbrella organisations such as APOS- Singapore, PAPO-Philippines, IAPG- India for country level implementation (to receive manpower and funding support for localising the framework, revising the course content to be more accurate for their country, vetted by subject matter experts)

3- Develop Patient Data Infrastructure to Support Evidence Generation and Decision Making

Strengthen the patient evidence ecosystem by establishing shared patient registries, data platforms, and governance frameworks that enable credible patient evidence generation.

Stakeholders to Implement

- Primary: Government health authorities, patient organisations, and research institutions
- Supporting: HTA bodies, regulators, and industry

Timeline

- Short term: Pilot patient registries and develop standardised data collection frameworks
- Long term: Build integrated regional infrastructure for patient data collection, sharing, and analysis

Mode of Implementation

- Develop disease-specific patient registries to strengthen the evidence base
- Establish shared patient data platforms accessible to key stakeholders
- Implement robust data governance and privacy frameworks
- Support patient–researcher–clinician partnerships for evidence generation
- Use registry and patient-generated data to inform regulatory and HTA decision making

NOTE: to mandate patient engagement training, quantify value of patient experience data in decision making, reward dossiers with real world data integration

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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