FROM THE REGION

ISPOR's Health Technology Assessment and Patient Representative Roundtables: Strengthening Patient-Centered Decision Making in Asia Pacific and Globally

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Against the backdrop of rapid institutional reforms and development for national health technology assessment (HTA) in China, ISPOR hosted HTA and Patient Representatives Roundtable discussions in Beijing, China on 25 October 2019. ISPOR's HTA and Patient Representative Roundtables are platforms to advance scientific methods, facilitate information sharing about the development of HTA, and strengthen the role HTA plays in optimizing healthcare decisions. These roundtables are ideal opportunities for ISPOR to bridge the gap between technology assessors, private and public payers, regulators, and patients, and the discussions focus on innovative ways to improve health globally and make healthcare decision making more patient-centric. ISPOR HTA and Patient Representatives Roundtables are convened regularly in Asia Pacific, Europe, Latin America, Middle East and Africa, and North America.[1,2]

The ISPOR Asia-Pacific HTA and Patient Representatives Roundtables enjoyed broad representation from key experts and decision-making bodies from the region, including the Pharmaceutical Benefits Advisory Committee (Australia); Health Technology Assessment in India (India); HTA Committee (Indonesia); Health Insurance Review & Assessment Service and National Evidence-based Healthcare Collaborating Agency (South Korea); Center for Drug Evaluation (Taiwan); National Institute of Public Health and National Institute of Social Security and Population Research (Japan); Health Technology Assessment Section, Ministry of Health, Malaysia; Philippine Health Insurance Corporation (Philippines); Agency for Care Effectiveness (Singapore); Health Intervention and Technology Assessment Program (Thailand); and HTA department (Mongolia). Patient organizations that were represented included the Heart to Heart Foundation (Thailand), Lymphoma Association of China, China Organization for Rare Diseases, Philippine Alliance of Patient Organizations, Vietnam Hemophilia Group, China Rare Disease Organizations Development Network (Mainland China), and the Psoriasis Association Taiwan.

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The key topics of the roundtables centered on managing high-cost therapies and patient participation in HTA and healthcare decision making. Participants presented specific cases of managed entry schemes, negotiation mechanisms and approaches for pricing and reimbursement, HTA harmonization across the globe, HTA in universal health coverage implementation, and patient involvement in healthcare decisions.

Improving Patient Access to Innovative Technologies

A major thread of discussion centered around ways payers are bringing access of high-cost innovative therapies to patients while still maintaining acceptable budgets. Jurisdictions are taking highly varied approaches to this issue, ranging from direct centralized negotiation in China to outcomes-based arrangements in Australia and South Korea. Chinese payers have leveraged their purchasing power and large market to extract steep price cuts for many orphan drugs and have also sped up review and approval processes significantly. The Chinese government is also conducting more frequent comprehensive reviews and updates of the national reimbursement drug list, with the latest update occurring in 2019. Currently, all new therapies under consideration are required to undergo review in the areas of clinical efficacy, pricing benchmarks, budget impact analysis, and health economics (budget impact analysis and health economic analysis were optional previously). Additionally, the government is also engaging in a program of high-volume centralized purchasing of generics through their "4+7 Plan," which has led to lower prices for a wide variety of medicines.[3] While such approaches have yielded rapid and dynamic results, how these changes will affect the healthcare system in terms of systematic and transparent processes toward value and efficacy assessment, prioritization and access, and health technology innovation remains to be seen.

Risk-sharing agreements or other managed-access programs have been in practice in South Korea and Australia for several years and have provided incentives and pathways for the adoption of promising new technologies for vulnerable patients where limited data may exist. While there have been some examples of success with these programs, significant challenges remain, particularly regarding capacity and bandwidth of payers in collecting data and assessing the relevant evidence. And since many of these arrangements are only active for 4 to 5 years per contract, the questions surrounding long-term efficacy and value are harder to answer. Many studies that utilize narrow time horizons or surrogate endpoints for the candidate interventions are said not to adequately capture the full costs and value that are expected to be realized throughout the technology's life cycle. Additionally, the arrangements themselves can bring substantial risk and uncertainty.

Thus, some payers still feel hesitant to pursue these types of arrangements (except in very special cases). It was expressed by many participants that to make such arrangements more feasible in the region, additional work needs to be done by innovators to generate acceptable evidence for payers to mitigate uncertainty and risk wherever possible. For example, there should be enough of an initial correlation within the clinical trials and a sufficiently robust accompanying body of outcomes data to support effective decision making.[4] Payers should also have a better understanding of the potential market impact of reimbursement and renegotiation decisions, which could affect the availability of certain products in their countries.

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Patient Involvement in HTA in Asia Pacific: Where Are We?

As patients and patient advocates are becoming empowered to take ownership of their healthcare, they are increasingly laying pressure on HTA bodies and policy makers and emphasizing the importance of their involvement in informing policy and HTA decisions. At the same time, there is rising consensus in the region among policymakers that healthcare decision making and delivery should be patient-centric and equitable. Many jurisdictions in the region have already formally incorporated patient involvement in their HTA processes. In Taiwan, 2 patient representatives are invited to participate in the Pharmaceutical Benefit and Reimbursement Standard joint meeting as nonvoting members. In Australia, the Pharmaceutical Benefits Advisory Committee has 2 expert consumer (patient) representatives, and schedules consumer hearings to facilitate dialogue. Australia also established the HTA Consumer Consultative Committee in 2017 that provides strategic advice and support to the principal Health Technology Assessment Committees and the Department of Health with the inclusion of consumer representatives. While these examples are encouraging, there are still questions among patients as to whether this is enough, as these representatives do not have voting power in some jurisdictions and may have a limited capacity in providing input. Patient groups also question whether such a small patient delegation on these committees could be truly representative of the broader community, even if they may be expertly qualified. And for groups that still lack formal participation mechanisms in their respective jurisdictions or feel that such processes are lacking, advocacy remains their primary recourse, which has its own limitations. While progress is occurring, much more needs to be done to ensure that these processes are achieving the ultimate objective of making decisions patient-centric. To that end, key questions have emerged, namely: (1) What is the proper role of patients in HTA and healthcare decision making? (2) Where should patients get involved in the process? and (3) What can patients meaningfully contribute to the process?

Managing Uncertainty: Local Data Constraints and Future Investment

Many jurisdictions in Asia Pacific struggle with a paucity of local population data, which means that many important reimbursement decisions must be taken based on potentially limited relevant evidence. Challenges remain in making data and evidence available and adaptable for local considerations. China is taking large strides toward incorporating and utilizing big data in healthcare decision making at all levels, with the establishment of a China Real World Data and Studies Alliance (ChinaREAL) and investment in data infrastructures.[5] The ChinaREAL collaboration has resulted in the production of technical guidance documents including databases and registries for research purposes, epidemiological and statistical considerations in the assessment of treatment effects, and key methodological issues in pragmatic randomized controlled trials. Regionally, however, there are questions surrounding managing uncertainty, including what structure and resources are needed to clarify the impact and relevance of data. Specifically, how do we collectively define an intervention's level of impact or magnitude of benefit? Is it just high unmet need being met? What is a significant clinical benefit—is it defined in terms of breadth or depth? How do patients value judgments differ from society as a whole? And the question of changing priorities and realities in the light of evolving evidence and perspectives necessitates clarity of approaches surrounding disinvestment and de-listing of technologies.

What Can Patients Contribute?

Based on the notable efforts many patient organizations are making in the region, it was clear that patient data are one of the most powerful witnesses they can provide. According to one prominent patient advocate in the region, data are an important tool for patient organizations to present their case to decision makers, and that without data, a patient is just another person with an opinion. Patient representative organizations have taken incredible efforts to generate patient-centric data for decision makers, as well as publishing reports and presenting to policymakers to emphasize the special considerations that HTA needs to make for rare diseases. Patient-generated data can provide insights into patient preferences and priorities for policymakers, and patient inputs can help researchers to better capture the burden of disease and cost of illness. Jurisdictions in the Asia Pacific region have incorporated various mechanisms for capturing patient data and perspectives. For example, Taiwan has fielded a patient questionnaire with an online submission form and guidelines to generate patient feedback; Australia also utilized a similar feedback process. Nonetheless, quality of feedback and patient data remains a challenge, as there is no formal system for assessing validity or considering conflicts of interest (lobbying influences) in Australia.

A key challenge for the future will be making patient inputs and data more meaningful for payers and impactful in

health policy. The first part of this relates to the ability of patient organizations to effectively leverage their voice and position as a credible and vital stakeholder in the process. To lend more weight to their voices, "expert patients" are needed—both globally and regionally—to strengthen the foundation for organizational/institutional participation and incorporation of perspectives, and education will remain a critical part of this. Patients should also be better advocates (not just for their specific diseases but for their stakeholder group as a whole), as they will be more effective in a unified way. A "turfing" mentality still exists among some patient societies as they vie for influence and limited resources.

The other part of this relates to the quality of patient data. For policymakers the question becomes: What kind of data are really helpful for decision making? With respect to qualitative data, decision makers count specific and rich patient testimonials (ones that share patients' personal disease experience and effects on the quality of their lives) as most useful to them. From a research standpoint, patient perspectives have the potential to ensure that clinical trial and observational study designs have assumptions, objectives and endpoints that are better aligned with the real world to optimize outcomes.[6]

Conclusions

For HTA to be successful, it should be timely, relevant and practically usable for decision makers, and follow an inclusive and transparent process that proactively emphasizes local horizon scanning and priority setting. Patients are a key stakeholder group for healthcare and should be actively involved in HTA, but where and how they are involved in the process needs to be clarified further. Moreover, there is an important role for patients to play in clinical trial design and in the design and interpretation of observational studies.

Development and utilization of local data will be an essential priority for Asia Pacific countries in the immediate term to mitigate global data reliance. Patient-reported outcomes data are also set to play a more prominent role in future evidence considerations, including in China. Further works needs to be done to strengthen health infrastructures and to bridge evidence gaps globally through health economics and outcomes research. Finally, it will be essential for HTA stakeholders to more actively facilitate translation of their recommendations into policy. A model for this could be Malaysia, which involves government payers in assessment priority setting through criteria and discusses evidence with decision makers on the local context.

This report is adapted from presentations and discussions that occurred during ISPOR HTA and Patient Representative roundtables - Asia Pacific on 25 October 2019.

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Additional information

The next ISPOR Asia Pacific HTA and Patient Representative Roundtables will take place during the ISPOR Asia Pacific 2020 Conference, to be held on 12-15 September 2020 in Seoul, South Korea. For more information on these and other initiatives, please visit: www.ispor.org/member-groups/councils-roundtables.