IP12: HOW CAN WE EMPOWER THE PATIENT VOICE IN HEALTH CARE DECISION MAKING AT THE POLICY LEVEL?

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GUIDING QUESTION: How can we inform healthcare decision making, at HTA/ payer level, by empowering the patient perspective on the value of medicines?
Our Panelists

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PhD, Professor, Pharmaceutical Policy & Outcomes Research, School of Pharmacy, Sungkyunkwan University, Suwon, South Korea

Richard Vines
Chairman, Rare Cancers Australia, Bowral, Australia

IP12: HOW CAN WE EMPOWER THE PATIENT VOICE IN HEALTH CARE DECISION MAKING AT THE POLICY LEVEL?

Empowering the Patient Voice in Health Care Decision Making at Policy Level in Asia Pacific
- Challenges & Opportunities in Korea -

Eui-Kyung Lee, PhD, Prof.
Pharmaceutical Policy & Outcomes Research
School of Pharmacy
Sungkyunkwan University (SKKU), Korea
I. HTA & Patient Engagement

- HTA can play a key role in supporting rational decision-making about health technologies based on appropriate evidence

- HTA for new drugs in Korea
  - Positive list system (PLS) was implemented in Dec 2006 as the core plan for drug expenditure rationalization plan.
  - Cost-effectiveness became the important decision criteria.
    - Cost-effectiveness decision is based on the implicit ICER threshold, $20,000/QALY in Korea
    - Cost-effectiveness became a 4th hurdle besides safety, efficacy and quality for market access


Limited Access to Drugs

Decision at NICE (UK)

Source: Bae E-Y et al., Health Policy (2016)
Importance of Patient Engagement in HTA

- To input on patient-relevant outcomes and real-world benefits and adverse effects
  - Experiential knowledge about living with an illness
  - Unmet need: its treatment of importance to patients
- Patient psychological and social Impact
  - Lifestyle, ability to work, tolerance, family, last chance, hope
  - SF36, EQ5D: cannot capture all the symptoms patients are experiencing
- Burden of an illness to patients including wider societal cost
- Appropriate use
  - To make appropriate choices, adhere to optimal use

II. Patient engagement status in Korea

- Increasing attention to involving patients in funding decisions
  - Legislation on patient safety in 2015
- Korea Patients Advocacy Organizations
  - Korea Organization for Patient Group
  - Korea Association of Leukemia Patients
  - Korea Association for Children with Leukemia and Cancer
  - Korea Organization for Rare Disease
  - Korea Congenital Heart Disease Patient Group
II. Patient engagement status in Korea

• Approaches for Patient engagement in Korea
  ▫ **Communication**
    • Web & social media (HTA → Pt)
  ▫ **Consultation**
    • Solicitation of input, feedback on draft documents, comment on policy (Pt → HTA)
    • Individual patients, Patient Advocacy Group, Medical associations input unmet need of access to innovation and reimbursement through oral/written statement, meeting to Government (HIRA, MoHW, NHIS).
    • Advisory role for the general policy development

• **Direct Participation** in Government committees
  • Information Exchange : HTA ←→ Pt
    • Reduce the risk of miscommunication, discuss options
  • Representative of **KAPO** (Korea alliance of patients Organization) was appointed as a member of **NHIPRC** (National Health Insurance Policy Review Committee) from Jan 2016
    • But not participate at the reimbursement decision for a specific technology and drug
    • Final stage for decision making
  • Representatives of **KORD** (Korean Organization for Rare Disease) are participating in **co-payment review committee for orphan disease** from Aug 2016
Korea Cancer Care Alliance (KCCA) Initiatives

- Stakeholders representing oncology issues: HCP, Media, Patients, National Assembly, Pharma association
- To propose new policy and deliver patient voice in HTA decision making
- Raising public awareness of patient access issues

Challenges for Patient Input in HTA

- Knowledge & Experience limited
  - Patients: technical language on HTA and economics are difficult to engage with
  - HTA Agency, Society: limited understanding
- Patient advocacy group (PAG) limited
  - not represent diverse diseases
  - most patients unaware of PAG
- Distrust: Fair-minded? Value-neutral?
  - patients relations with pharma companies raises question of conflict of interest, diminution of input
- Patient Role and the Process poorly defined
  - no systematic process to integrate patient submissions
- Patient Evidence
  - No consensus on the method to obtain patient evidence
  - Qualitative information: difficult to integrate
Ⅲ. Better Patient Engagement in HTA

• HTA is a complex field
  ▫ that should reflect social, economic, political and cultural circumstances
  ▫ based on local evidence, values and priorities.

• Several competing values to consider
  ▫ Efficiency: Value for Money
  ▫ Clinical Usefulness
  ▫ Financial Sustainability: Controlling costs
  ▫ Ensuring access to treatments
  ▫ Providing innovation incentives
  ▫ Equity for all patients (children, disadvantaged)
  ▫ Fairness: for rare diseases, high costs of intervention

• To balance the economic need for fair allocation of resources, with the patients’ equal opportunities to access health

• Decision-making must be fair and inclusive
  ▫ To enhance the legitimacy and acceptability of resource allocation decisions, more inclusive opinions and preference needs to be reflected in the process
  ▫ by reflecting patients problems, lived experiences, outcomes and preferences in HTA

• To improve patients’ understanding of HTA
Best practice for patient engagement

• CADTH Patient and Public Involvement in Canada
  • Incorporates patient input systematically throughout drug review and recommendation-making process
  • Uses template, guide, online submission form

Template for Submitting Patient Group Input to the Common Drug Review at CADTH (1)

• **Conflict of Interest Declarations**
  - financial support from the pharmaceutical industry [e.g., educational or research grants, honorariums, gifts, and salary],
  - affiliations or personal or commercial relationships with drug manufacturers or other interest groups

• **Information Gathering**
  - Objective, experiential information that is representative of the majority of the patient group is preferred
  - **Method**: through personal experience, focus groups, one-to-one conversations with a number of patients
Template at CADTH (2)

- **Condition and Current Therapy Information**
  - **Impact of Condition on Patients**
    - impact the patients’ day-to-day life and quality of life
  - **Patients’ Experiences With Current Therapy**
    - Unmet needs, access, safety, effectiveness
  - **Impact on Caregivers**
    - caregivers’ daily routine or lifestyle, adverse effects

- **Information about the Drug Being Reviewed**
  - **Expectations for the New Drug**
    - life expectancy, unmet need, adverse effects
    - How much improvement in the condition would be considered adequate?
  - **experience (by Clinical trial) for the New Drug**
    - positive and negative effects, symptom management, adverse effects (acceptable), easy to use, long-term health and well-being

Patient Engagement as a System (1)

- **A Framework for Involving Patients in drug-Review Process**
  - More explicit role of patients in the HTA process
    - Clarification on what information, how to collect, how to present
  - Selection of Patient Representative based on knowledge, experience, conflicts of interest
  - Strengthen the patients’ competence and capacity to contribute HTA
    - Education: Training program: on-line training module, on-going educational supports regarding HTA process & decision making
Patient Engagement as a System (2)

- Encourage Active Participation
  - Patients as Partners in HTA
  - Full voting right
  - The right to be heard reinforces patients’ interests in the process

- Embed into HTA Decision making
  - Integration of evidence on patients’ preferences, patient values/perspectives into HTA
  - Involve Pt from the early stage of HTA process
  - through in-depth consultation, qualitative research & patient representation on advisory committees
  - Transparency of decision making process

MCDA (Multi-Criteria Decision Analysis)

- Need efforts to produce conceptually robust, evidence-informed frameworks to guide patient engagement in HTA

- One of the principal options is MCDA
  - Structured Supporting Tool for Decision-Making to aggregate various values: more holistic perspective
  - Consider wider set of explicit criteria, leading to a more complete assessment of value
  - Reflect differences in their relative importance
  - Stakeholder engagement \(\rightarrow\) Social consensus
MCDA (Multi-Criteria Decision Analysis)

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Approaches to MCDA

(1) Quantitative approach
- A wide range of techniques for eliciting preferences
- Complexity of method: DCE, AHP, Swing etc
  - require high level of expertise and experience
  - The capacity varies among countries: HTA infrastructure
- In Korea, even though the basic capacity has been built, more expertise needed.

(2) Qualitative or Semi-Quantitative approach
- Decision tool based on a checklist for rapid assessment
  - Ex: mini-HTA in Denmark, Risk-benefit framework in FDA
- In Korea evaluation check-list is used for the medical device reimbursement: clinical usefulness (effectiveness, adverse event, QOL), cost-effectiveness, innovation
### Preferences for criteria on cancer drug reimbursement

<table>
<thead>
<tr>
<th>General Pop. Criteria</th>
<th>RI</th>
<th>Professionals Criteria</th>
<th>RI</th>
</tr>
</thead>
<tbody>
<tr>
<td>1 Disease severity</td>
<td>0.229</td>
<td>1 Unmet need</td>
<td>0.302</td>
</tr>
<tr>
<td>2 Unmet need</td>
<td>0.211</td>
<td>2 Clinical benefit</td>
<td>0.237</td>
</tr>
<tr>
<td>3 Population size</td>
<td>0.164</td>
<td>3 Cost effectiveness</td>
<td>0.198</td>
</tr>
<tr>
<td>4 Budget impact</td>
<td>0.146</td>
<td>4 Budget impact</td>
<td>0.117</td>
</tr>
<tr>
<td>5 Clinical benefit</td>
<td>0.108</td>
<td>5 Disease severity</td>
<td>0.079</td>
</tr>
<tr>
<td>6 Cost effectiveness</td>
<td>0.091</td>
<td>6 Innovation</td>
<td>0.055</td>
</tr>
<tr>
<td>7 Innovation</td>
<td>0.051</td>
<td>7 Population size</td>
<td>0.012</td>
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</tbody>
</table>

**RI**: relative importance

<table>
<thead>
<tr>
<th>Weight Rank by DCE (Discrete Choice Experiment)</th>
<th>Weight Rank by AHP (Analytic hierarchy process)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>General Pop. Criteria</strong></td>
<td><strong>Professionals Criteria</strong></td>
</tr>
<tr>
<td>Mean</td>
<td>Mean</td>
</tr>
<tr>
<td>Clinical Benefit</td>
<td>0.218</td>
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<tr>
<td>Cost effectiveness</td>
<td>0.165</td>
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<tr>
<td>Disease Severity</td>
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<td>Population size</td>
<td>0.123</td>
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<tr>
<td>Budget Impact</td>
<td>0.095</td>
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<tr>
<td>Therapeutic need</td>
<td>0.091</td>
</tr>
<tr>
<td>Innovation</td>
<td>0.076</td>
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</tbody>
</table>

**RI: relative importance**

*EK Lee, MCDA on Cancer drug reimbursement, 2014*

### Receptivity as a Decision Making Tool

- **Comprehensive, integrated decision-making approach to balance the multiple criteria**
  - Make decision consistent, transparent, predictable

- **Challenges**
  - Uncertainty of the study results based on methodology
  - Decision criteria not developed yet, No clear threshold
  - Additional burden on the society for evidence development
  - No foreign experience as a formal decision-making

- **Suggestion as a decision aid tool**
  - **Supplement tool to Adjust HTA decision**, rather than **Substitute for HTA**
  - **Voluntary option** for better assessment of values
Thank you for your attention!

Richard Vines
Rare Cancers Australia – Chief Executive
Cancer Drugs Alliance – Co-Chair
Patients – not just sick people!
• Experts in their own disease
• We may all be patients one day
• Lawyers, Politicians, Scientists, Bankers, Bakers etc
• Tax Payers and in most jurisdictions
• Voters – (Voter Adjusted Life Years – VALY’s)
• Passionate Advocates
• The Community is the cancer community!
• Community sentiment drives change.

What should the patient voice be saying
• To be a voice for those who don’t have one
• To advocate for fairness and equity
• To stay outside the frame
• To bring different life experience
• To communicate to the community
• To focus on the real value of the treatment to the patient
How is the Australian patient voice heard at present?

**Formal**
- *Pharmaceutical Benefits Advisory Committee* has one Patient Representative out of 17
- *Patient Representative on Drug Utilisation Sub Committee*
- *None on Economics Sub-committee*
- *Patients submissions to PBAC – 6 week window*

**Informal**
- *Patient Groups*
- *Media – Poster Patients; Fundraising*
- *Lobbying Politicians*
- **Kicking, Screaming, Shouting!**

What happens if the patient is ignored?
How do we empower the voice?

- Provide guidance and education – over AP 300 groups
- Listen and take heed.
- Inform and help them access and build data
- Understand that patient knowledge is different from yours not less important
- Build on strength
- Respect, respect, respect

An Example

*Rare Cancers Baseline Report*

*“Just a little more time”*

Sources

- Australian Institute of Health and Welfare
- Cancer Australia
- Pharmaceutical Industry

Objective

“To present a summary of the current state of RLC Cancers in Australia that would kick-start the search for improved research, diagnosis and treatment”
"Just a little more time"

**What are RARE AND LESS COMMON CANCERS?**

A ‘rare cancer’ is defined as a cancer type found in less than 6 per 100,000 Australians per year.

And ‘less common’ cancers found in between 6 & 12 per 100,000 Australians per year.

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“Just a little more time”

**What is THE DIAGNOSIS?**

EVERY YEAR 42,000 AUSTRALIANS ARE DIAGNOSED WITH A RARE OR LESS COMMON (RLC) CANCER.

Overall an estimated 124,910 cases of cancer will be diagnosed in Australia each year.

This means rare cancers make up about 30% of all diagnoses but account for 50% of cancer deaths.
"Just a little more time"

The rate of RLC diagnosis and mortality is currently **TWICE** that of the population growth.

**MORTALITY Rates**

- 22,000 Australians will die due to an RLC every year, accounting for **50%** of cancer deaths.
“Just a little more time”

RESEARCH and TREATMENT

RLC Cancers receive compared to their contribution of
13.5% 12.6% 50%
of research of PBS Funding of all cancer of 1 in 2 DEATHS
funding deaths

What NEEDS TO HAPPEN?

Innovative research that leads to earlier detection and better treatment

Increased availability of PBS-funded treatments and equal access to care and treatment regardless of cancer type

Without concerted action in research, diagnostics and treatment, Australia could be confronting over 30,000 deaths from RLC Cancers by 2020 and over 40,000 by 2030.
Open Discussion

• What opportunities/ challenges do you see for cross sector collaboration (academia, payer/ government, patients/ civil society, industry) to advance patient engagement at HTA level?

• If you had the power to change one thing in the HTA process to create better patient engagement – what would that be?

• Can you describe a best practice (from your country or another) and what makes it a best practice?