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

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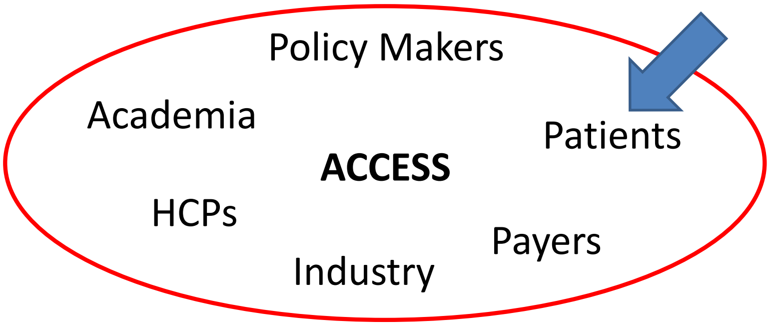

Policy Makers
Academia
Patients
ACCESS
HCPs
Payers
Industry

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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7th AP ISPOR Singapore
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Empowering the Patient Voice in Health Care Decision Making at Policy Level in Asia Pacific

- Challenges & Opportunities in Korea -

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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

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Drug Reimbursement Decisions (2007-2014)

Reimbursement decisions of the PBCAC (January 2007–December 2014) Unit: number of molecules, %.

Year	Accept		Reject	Total (C)	Acceptance rate A/C % (B/C %)
	Accepted by the PBCAC(A)[a]	Reached price agreement (B)[b]			
Total	175	166	78	253	69.2 (65.6)
Oncology drugs[c]	16	16	15	31	51.6 (51.6)
Others[d]	159	150	63	222	71.6 (67.6)

Abbreviations: PBCAC, Pharmaceutical Benefit Coverage Assessment Committee.

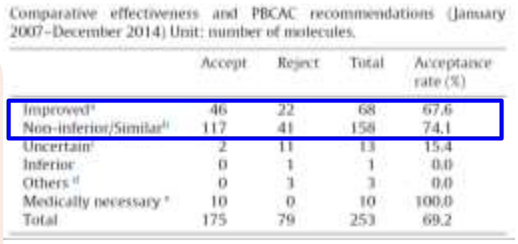
[a] Number of drugs that are positively recommended by the PBCAC.

[b] Number of drugs listed successfully as reimbursable drugs on the Korean NHI as a result of successful price negotiations.

[c] Drugs classified as L01 following three digits of ATC code.

[d] All other drugs excluding oncology drugs.

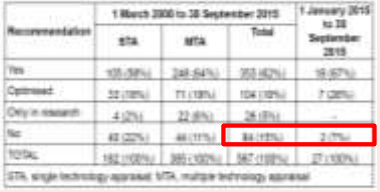
Limited Access to Drugs



Comparative effectiveness and PBCAC recommendations (January 2007–December 2014) Unit: number of molecules.

	Accept	Reject	Total	Acceptance rate (%)
Improved[a]	46	22	68	67.6
Non-inferior/Similar[b]	117	41	158	74.1
Uncertain[c]	2	11	13	15.4
Inferior	0	1	1	0.0
Others[d]	0	3	3	0.0
Medically necessary[e]	10	0	10	100.0
Total	175	79	253	69.2

Decision at NICE (UK)



Recommendation	1 March 2000 to 30 September 2015			1 January 2015 to 30 September 2015
	STA	MTA	Total	
Yes	105 (58%)	248 (64%)	353 (62%)	18 (67%)
Optimised	33 (18%)	71 (18%)	104 (18%)	7 (26%)
Only in research	4 (2%)	22 (6%)	26 (5%)	–
No	40 (22%)	44 (11%)	84 (15%)	2 (7%)
TOTAL	182 (100%)	385 (100%)	567 (100%)	27 (100%)

STA, single technology appraisal; MTA, multiple technology appraisal

Source: Bae E-Y et al., Health Policy (2016)

http://dx.doi.org/10.1016/j.healthpol.2016.03.013

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

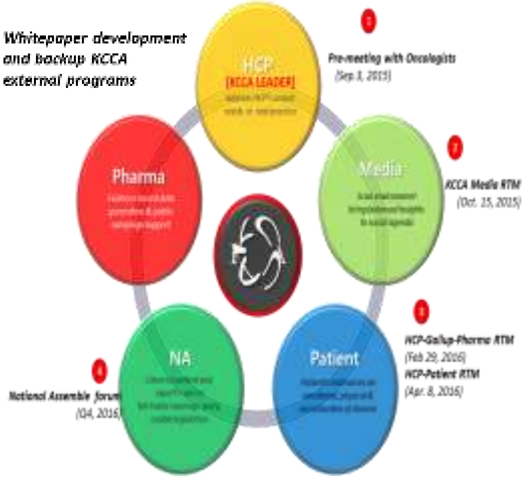
- Increasing attention to involving patients in funding decisions
  - *Legislation on patient safety in 2015*
- Korea Patients Advocacy Organizations
  - Korea Organization for Patient Group
  - Korea Association of Leukemia Patients
  - Korea Association for Children with Leukemia and Cancer
  - Korea 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

2013 CADTH SYMPOSIUM

How patient input is used

Canadian Patient Groups

Patient Input Submission

CADTH Review Team

Patient input collated, summarized, used to inform protocol & included review report

Original input & summary sent to CDEC

Expert Committee

Patient input presented, used in deliberations & reflected in recommendations

Reasons & recommendations published

Drug Plans

Patient input shared

Patient input shared

Patient input shared

Patient input shared

- **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

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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 → Social consensus

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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 expertize 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

MCDA study in Korea

Preferences for criteria on cancer drug reimbursement

Weight Rank by
DCE (Discrete Choice Experiment)

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

RI: relative importance

Weight Rank by
AHP (Analytic hierarchy process)

General Pop.			Professionals		
	Criteria	Mean		Criteria	Mean
1	Clinical Benefit	0.218	1	Clinical benefit	0.229
2	Cost effectiveness	0.165	2	Cost effectiveness	0.185
	Disease Severity	0.151	3	Disease severity	0.131
4	Population size	0.123	4	Therapeutic need	0.126
5	Budget Impact	0.095	5	Budget impact	0.099
	Therapeutic need	0.091	6	Population size	0.084
7	Innovation	0.076	7	Innovation	0.068

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!

rare cancers AUSTRALIA

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"

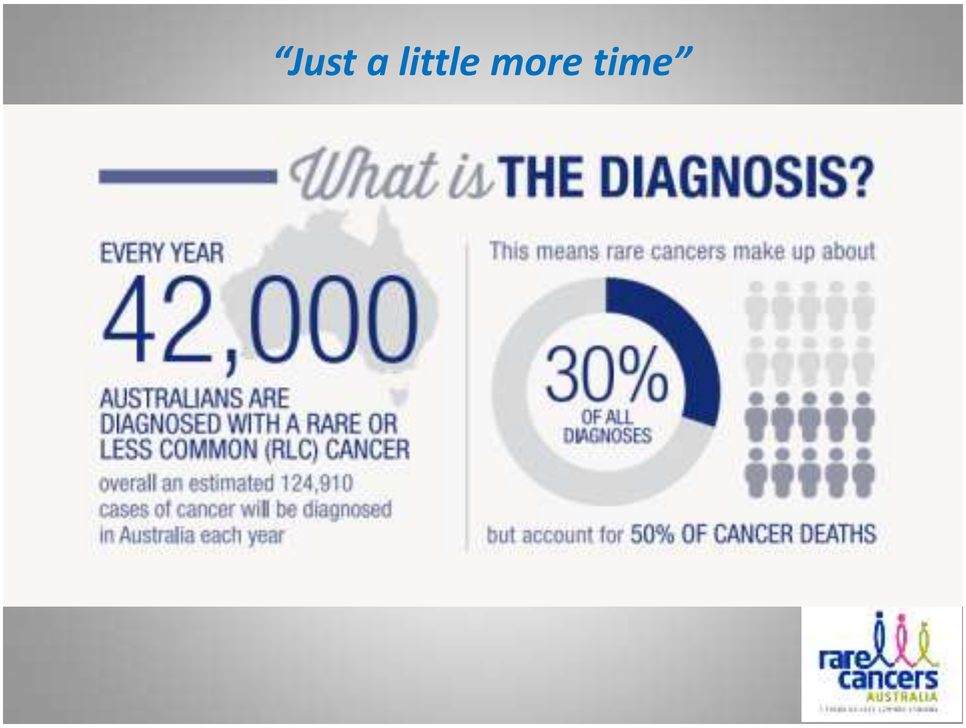
"Just a little more time"

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



"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"

Common cancers vs **rare and less common** cancers

The rate of RLC diagnosis and mortality is currently

TWICE THAT OF THE POPULATION GROWTH

"Just a little more time"

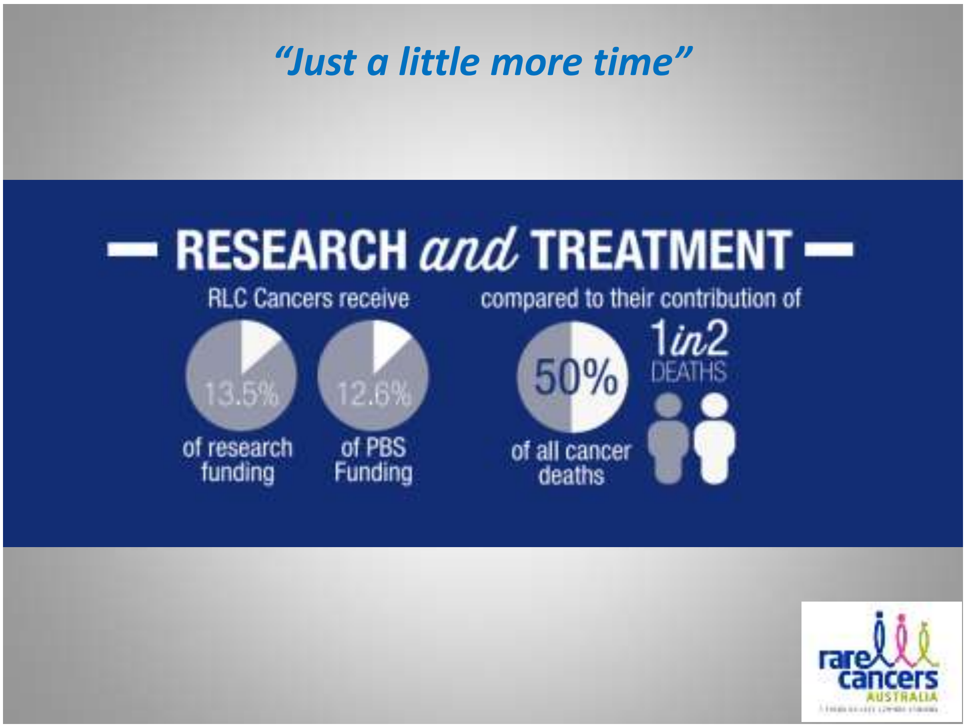
MORTALITY *Rates*

22,000

Australians will die due to an RLC every year

accounting for 50% OF **CANCER DEATHS**

Common vs RLC Mortality Increase since 1990 compared to Australian population increase



"Just a little more time"

RESEARCH *and* TREATMENT

RLC Cancers receive

13.5% of research funding

12.6% of PBS Funding

compared to their contribution of

50% of all cancer deaths

1 *in* 2 DEATHS

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"Just a little more time"

***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

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Rare Cancers Australia

Thank you

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?