HTA & Patient Group Involvement

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What is a health technology?

'A health technology is the application of organized knowledge and skills in the form of devices, medicines, vaccines, procedures and systems developed to solve a health problem and improve quality of lives.'

World Health Organisation

What is Health Technology Assessment (HTA)?

'HTA is the systematic evaluation of properties, effects and/or impacts of health technologies and interventions. It covers both the direct, intended consequences of technologies and interventions and their indirect, unintended consequences'

World Health Organisation

Challenge

Limited national budgets, high cost of novel therapies and limited clinical trial data collection of quality of life and activities information is proving a challenge to access new effective treatments for CLL.

Why involve patients in HTA ?

Patient groups can keep **evidence of a solution** to a health problem and i**mprovement to quality of life** at the forefront of the HTA process.

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Why involve patients in HTA ?

Provide ‘Real Life’ qualitative information to strengthen the case for access.

‘Real Life’ refers to patients actual experience living with and dealing with the disease.

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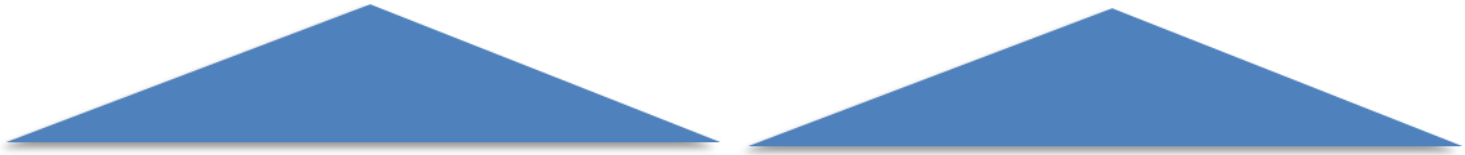
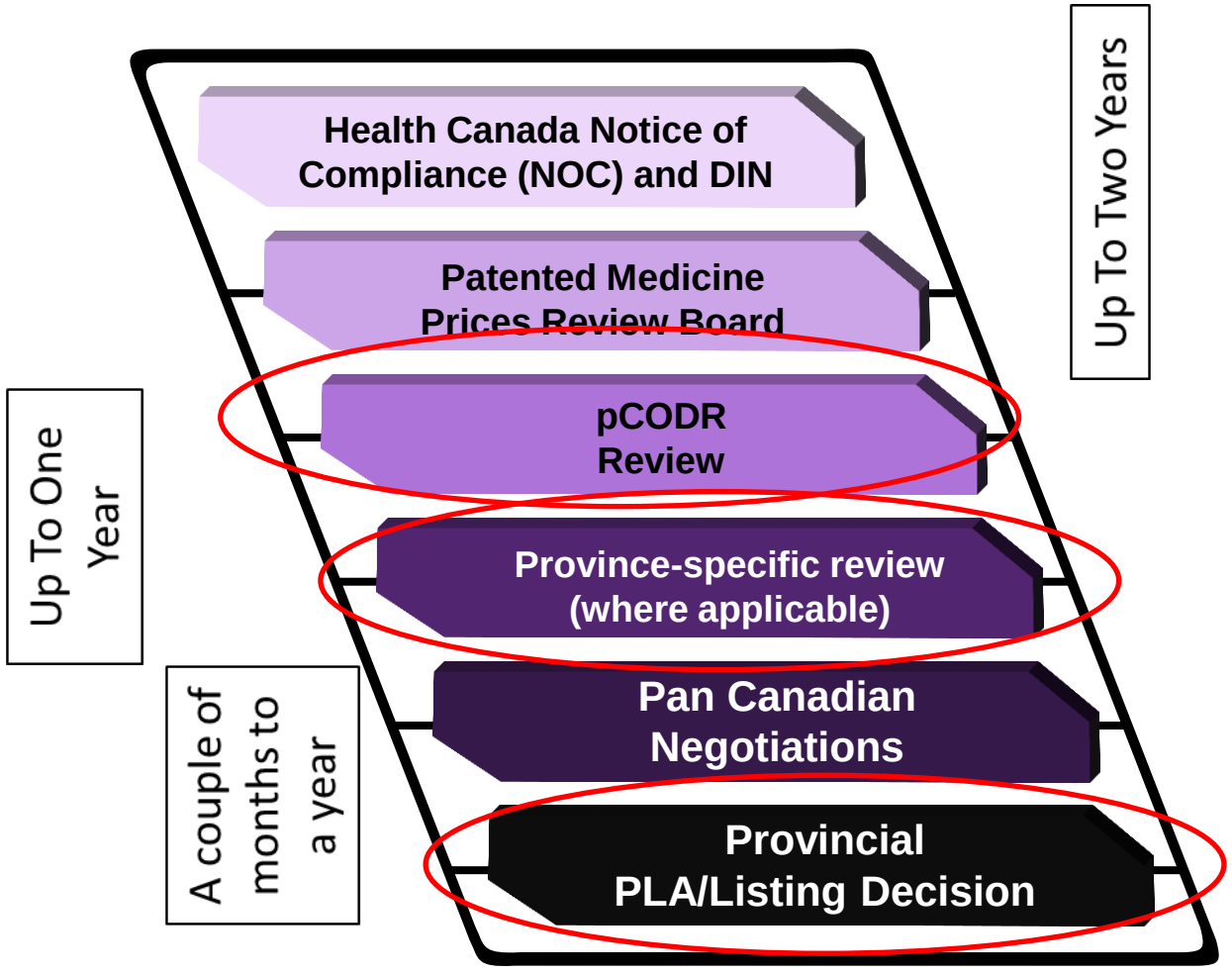
Pros and Cons of the Two Main HTA Models



Country	**Australia, Canada, UK**	**France, Germany, Spain, Italy**
Concept	**Cost/QALY vs ICER Threshold**	**2 steps: 1) Clinical benefit 2) Price negotiation on "added value" and Budget Impact**
Applicability & Learnings	• Difficult for non-health economists to understand • Technical limitations; e.g. end of life or data availability • What is the right threshold?	• Clinical benefit focus more understandable for patients and public • What is the right local comparator? • Less resource intensive

Canadian Example

Why do we need to have a role?



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Key Points for Canada

- Not all drugs approved by Health Canada are publically funded.
- Each province and territory has their own publicly funded prescription drug benefit program and they vary a lot.

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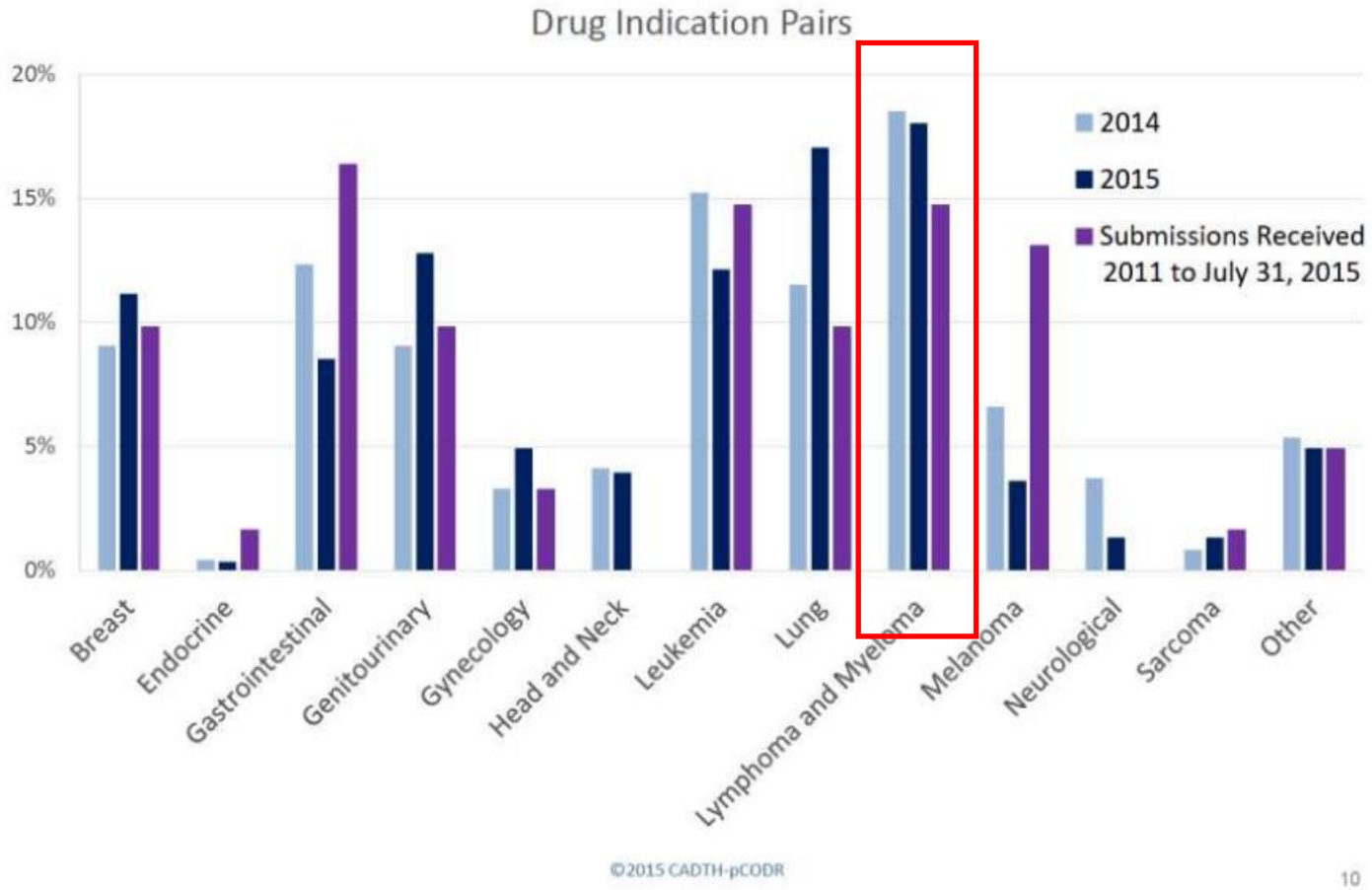
CADTH | pCODR PAN-CANADIAN ONCOLOGY DRUG REVIEW

Distribution by Tumour Site



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pan-Canadian Oncology Drug Review (pCODR)

- pCODR assesses cancer drugs and makes **recommendations** to the provinces and territories to help guide their drug funding decisions.
- pCODR is designed to bring consistency and clarity to the assessment of cancer drugs by looking at **clinical evidence**, **cost-effectiveness**, and **patient perspectives**

Patient Perspective

To be eligible to register with pCODR, patient advocacy groups must:

1. Have a mandate that pre-dates the group's decision to register with pCODR.
2. Have a means of contacting and communicating with its membership.
3. Represent patients or caregivers impacted by cancer.
4. Receive funding from more than one funder and no single funder can provide more than 50% of the group's operating funds.

Patient Perspective

- Form provided with specific questions to gather real-world information from patients and caregivers (8 page maximum)
  - Section 1: General information
  - Section 2: Disease and current management
  - Section 3: Drug under review
  - Section 4: Additional information
- 10 business days notice

How do we get input?

- Survey online via Survey Monkey
- Telephone interviews
- Recruit patients & caregivers through:
  - ✓Email lists
  - ✓Social media
  - ✓Website
  - ✓International patient organizations
  - ✓Clinical trial locations

Content of Submission - Data

- # of participants
- Country of residence
- Diagnosis date
- Troublesome symptoms
- Effect on day-to-day life
- Psycho-social impact
- Treatment(s) to date
- Treatment effectiveness
- Access to treatment
- Financial impact
- Side effects of treatment
- Effect on caregivers
- Unmet need – do we need more treatment options?
- Experience with new drug
- Would you recommend new treatment?
- Willingness to tolerate side effects

Content of Submission - Quotes

“My illness has robbed me of so many goals I had for my life and my family. I know I could have grown my business to a much greater level. I couldn't make it to work every day from the fatigue or was at another clinic appointment. The illness plays on your mind and you are angry that it was me it picked. I must depend on my wife and children to help me out to do manual chores or submit paper work to get some reimbursement from insurance companies. I have cancelled holidays with family and friends because my platelet counts are too low and I might have a life threatening bleed. My wife and I plan our lives around my clinic appointments.”

(Male, 45-54; Canada)

Content of Submission - Quotes

"All treatments wiped out my good blood components and made me tired. As treatment went on with each of these therapies I developed more complications that made it unsafe for me to continue to receive treatment. Hence I endured the chemo treatments but had complications like low platelets; low neutrophils and was unable to finish the full treatment of each of these lines of therapy…My remissions were short before the leukemia came back..."

(Male; 45-54; Canada)

Content of Submission - Quotes

"Yes *(new drug)* has extended my life. I thought it was quite possible that I wouldn't make it to the end of 2014 as my health was deteriorating fast. My QoL is now normal for a 56 year old. People who don't know my history are amazed when I tell them I have advanced CLL. I have no idea what the future holds, but I have had over 2 years of very good quality of life that I would otherwise not have had."

(CLL; 17p deletion; Male; 56; UK)

Process

- Step 1.0 Pre-submission Planning Activities by drug manufacturer or a tumour group.
- Step 2.0 Prepare and Submit Request for Drug Review
- **Step 3.1 Screen Submission and Initiate Review Process**
- Step 4.1 Conducting a Clinical Review
- Step 4.2 Conducting an Economic Review
- Step 5.0 Summarize and Review with pERC

Process

- Step 6.0 Prepare and Publicly Post Initial Recommendation and Reviews
- **Step 7.1, 7.2, 7.3 Get Input from Stakeholders**
- Step 7.4 Assess Eligibility for Early Conversion
- Step 8.0 Summarize and Review with pERC
- Step 9.0 Prepare and Publicly Post Final Recommendation and Post All Feedback Received

Impact

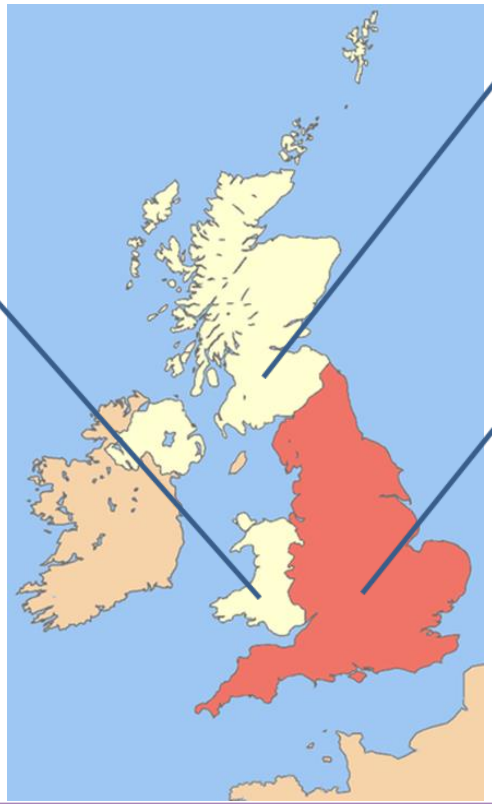
"pERC noted the **breadth and depth of the patient input**, which was **well structured and organized**, provided pERC with a **much deeper understanding of patients' experiences** with relapse or refractory CLL/SLL and its treatment."

UK Example

UK Health Technology Assessment (HTA) agencies

Scottish medicines Consortium (SMC)

All Wales Medicines Strategy Group (AWMSG)



National Institute for health and Care Excellence (NICE)

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UK - test case

Ibrutinib for relapsed/refractory and 17p TP53 mutated CLL – ONGOING

- Conflict with how the appraisal agencies measure evidence especially in novel innovative and transformational therapies.
- Protracted process, high demand on patient group resources.
- Ultimately cost of therapy versus QALY thresholds and available budget.
- How this has affected access:
  - Delayed access
  - Restricted access ? TBC ??

What do I need to know?

- Much variation of HTA processes across countries and agencies
- Understand your own country's HTA processes and the roles available to patients and patient groups.
- High demands on limited resources
- Value and benefits in collaborating with other groups
  - Extend resources and share skills
  - Increase patients centric evidence
  - Show of unity and strengthen patient voice

Important to remember

- Privacy is key. Group data is shared; individual data is not. Quotes are anonymous.
- Communicate back to participants what was decided by the review board.
- Find out who would be willing to do more (share personal story, speak with officials)
- Time consuming

Conclusion

Much needed, available and effective CLL treatments must become consistently available and affordable for all sub groups and populations the treatment has indications for.

Patient group participation and training in HTA, along with collaboration with all stakeholders during development processes and "real world" data collection, is becoming increasingly important.

Conclusion

'Ultimately, HTA bodies need to understand how all patients with a condition will be affected by a new technology. Research that involves a large representative sample of local patients and using good research approaches will be most useful for HTA bodies. Beyond relaying personal accounts, patient groups may also consider how they can best develop or provide population-based research to these HTA processes.'

From European Patients' Academy (EUPATI)

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