Cancer Patients' Role in HTA

An International Perspective

Deborah Maskens, MA, MSM

Kidney Cancer CANADA

CDDF Cancer Drug Development Forum

IKCC International Kidney Cancer Coalition

Key Messages

- To HTA Agencies: “If you’re not engaging patients, you’re not doing HTA!”
- To Industry: “If you are not improving patient relevant outcomes, your chances for successful reimbursement are diminished!”

Source: Dr. Brian O’Rouke, CADTH President and CEO; CTO Conference, October 2016

CDDF
Cancer Drug Development Forum

COI Disclosure

LIFE	Kidney Cancer
1960	
	1974 Surgery
1987	
1989	
1992	
	1996 Surgery x 2
	1999 Surgery
	2004 Surgery
	2006 Nexavar
	2007 Surgery
	2009 cMET trial U.S.
	2015 SBRT
	2015 Exome Sequenced; RNA …
	2017 ?

What Patient Input is NOT:

- Mediated via clinicians, other health system "experts"
- Public/citizen/taxpayer representation
  - Bias of the well vs. reality of those directly affected
- Tokenism… single patient representation on committees.

What Patient Input IS:

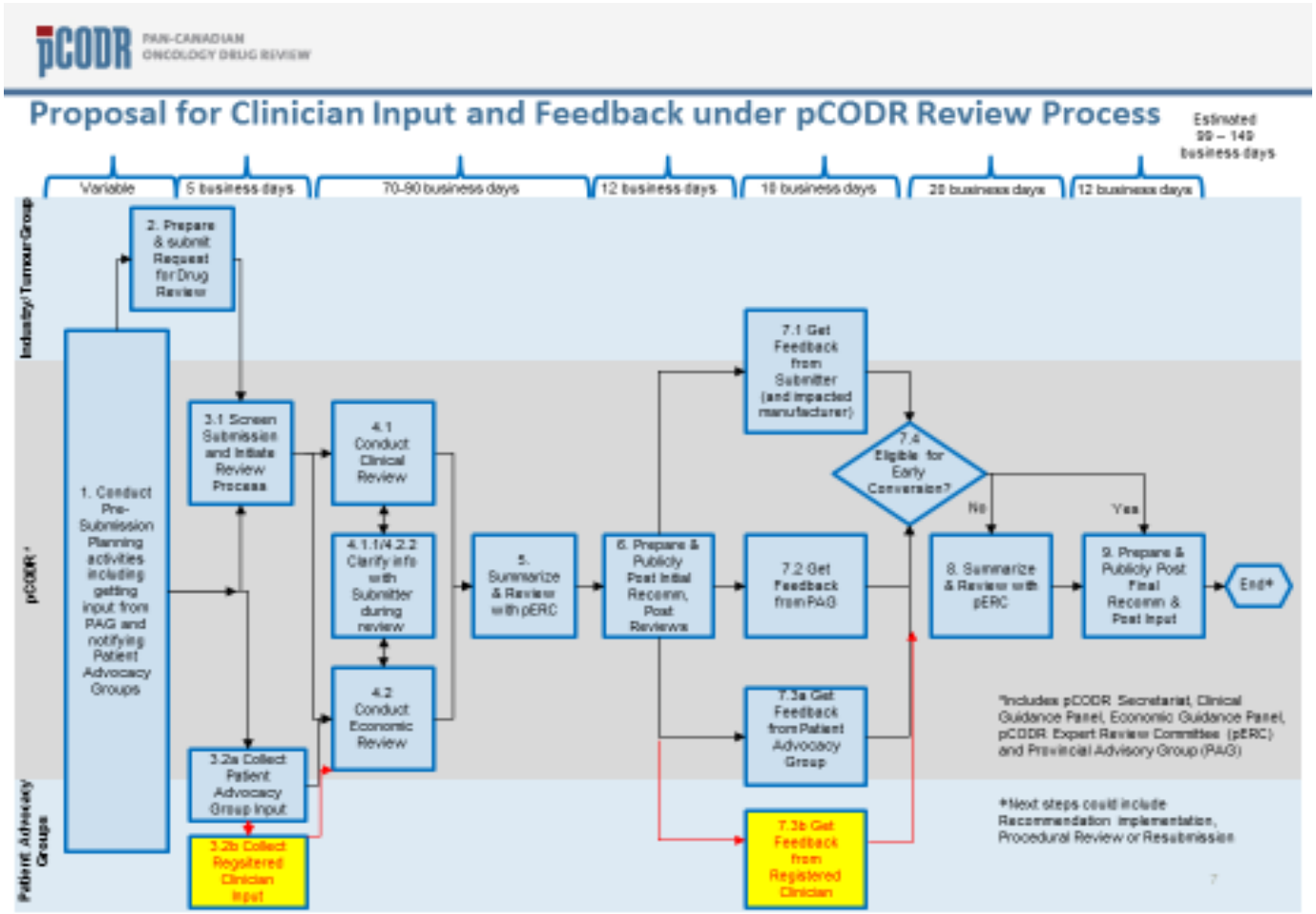
Based upon HTAi Value Standards :

- **Relevance**
  - Patients have knowledge, perspectives and experiences that are unique and contribute to essential evidence for HTA.
- **Fairness**
  - Patients have the same rights to contribute to the HTA process as other stakeholders and have access to processes that enable effective engagement.
- **Equity**
  - Patient involvement in HTA contributes to equity by seeking to understand the diverse needs of patients with a particular health issue, balanced against the requirements of a health system that seeks to distribute resources fairly among all users.
- **Legitimacy**
  - Patient involvement facilitates those affected by the HTA recommendations/decision to participate in the HTA; contributing to the transparency, accountability and credibility of the decision-making process.
- **Capacity building**
  - Patient involvement processes address barriers to involving patients in HTA and build capacity for patients and HTA organizations to work together.

Source: HTAi.org Patient & Citizen Involvement Working Group

CDDF
Cancer Drug Development Forum

Integrated (pCODR -Canada)



CDDF Cancer Drug Development Forum

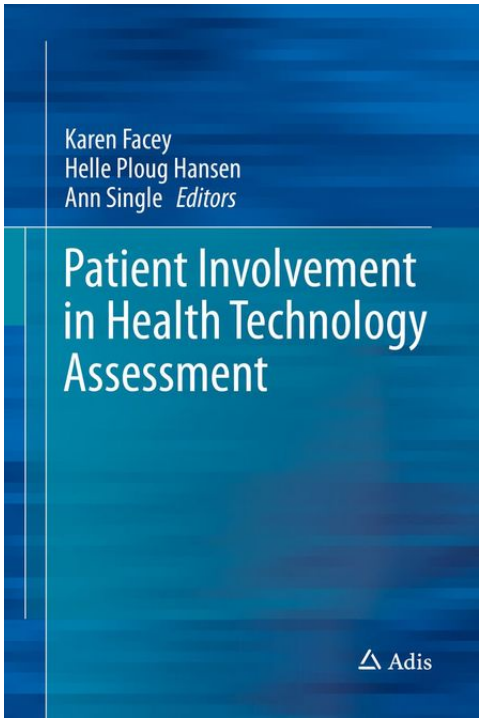
Recent: Request for Advice (RFA)

- pCODR issued RFA to registered stakeholders
- New data on “drug A” vs “drug B” in 2nd line
- Patient group response:
  - Accessed RWE database of 9,000+ patients
  - Data extract demonstrated equivalency of outcomes
  - Led to change in listing/criteria.

CDDF
Cancer Drug Development Forum

Best Practices Internationally

- SMC – patient representatives at the deliberation table
- NICE – patient reps involved in early scientific advice
- CADTH – feedback to patient groups
- PBAC – consumer representation; managed entry; RWE

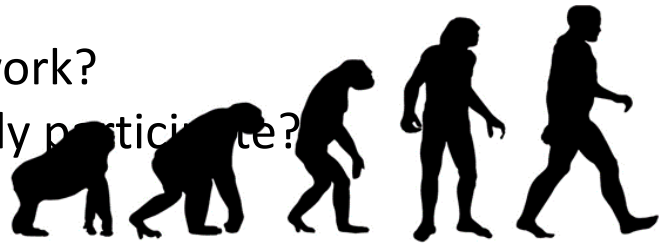


Where are we going?

Experienced patient groups:

- From “template submission” to interactive dialogue
- From end of process to beginning of discussions
  - Early scientific dialogue; clinical trial design
  - Design of relevant PRO measures

- Patient groups: “Be careful what you ask for…”
  - Significant work involved
  - Resource/ funding for this work?
  - Can all patient groups equally participate?



Experience with 'Patient Experience'

- pCODR Patient Evidence Process
  - Significant amount of work for patient orgs
    - Online survey, then 100+ hours per submission
  - pCODR expects input to be:
    - Current, Canadian*, Specific to new molecule
    - Also: rural/urban, pan-Canadian, online/offline, representative of socio-economic spectrum, indigenous/northern, balanced +ve/-ve, …
    - Techniques suggested:
      - Online surveys, but also: focus groups, IDIs (one on one interviews), telephone interviews, outreach to HCPs.
  - Reality…

CDDF
Cancer Drug Development Forum

Issues: what keeps us up at night?

- Are we wasting our time on “low level evidence”
  - Ethical dilemma of surveying patients
- Funding from industry / COI
  - Can we say “not this drug, not yet…”?
- What if we just stopped participating?
  - Would recommendations/decisions be any different?

Can we be smarter about this?

- Global collaboration:
  - Collaborate with other organizations nationally?
    - Same mutation/same drug/issues
  - Collaborate with HTA agencies at national/regional level
    - What is our Opinion?
    - How can we work together?
  - Collaborate internationally
    - Towards a “Global Patient Evidence Submission”

CDDF
Cancer Drug Development Forum

Radical thoughts

1. Given limited patient advocate resources:

- Where would you spend YOUR time?
  - Working with trial data after the fact?
    - Poorly designed trials; wastage; duplication
    - Lack of PROs, QoL data, value
    - Historical control arm; no longer relevant

- OR
  – Early involvement in drug development process?

CDDF
Cancer Drug Development Forum

Radical thoughts

2. What if we agreed:
"No data collection = no drug". Period.

Outcomes matter

- Clinical trial and/or
- Registry
- Managed entry program

- With:
  - Patient-reported outcomes beyond "EQ-5D"…

Radical thoughts

3. How do we measure success together?

Do we value?

- Cost containment
- Transparency
- Patient & public engagement
- Acceptance if not agreement?

“What kind of society do we want to live in?”

CDDF
Cancer Drug Development Forum

Thank you

Deborah Maskens
dmaskens@rogers.com
Twitter: @DebMaskensKCC
@IKCCorg

CDDF
Cancer Drug Development Forum