PERCEIVED IMPACT OF PATIENT INPUT ON HTA DECISIONS: COMMON VERSUS SPECIALTY DRUGS AND PATIENTS CF. NON-PATIENTS

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Presented at ISPOR 21st Annual International Meeting, May 21-25, 2016, Washington, DC, USA

ABSTRACT

Objectives: The Canadian Agency for Drugs and Technologies in Health (CADTH) allows patient input on drugs under review. Data on the perceived role and value of patient input were collected in (patient) training sessions. Secondary objectives included comparison of perceived value for common vs. specialty (rare condition, high unmet needs) drugs and the perceptions of patients and "non-patients."

Methods: Across six training sessions over two years, in small groups simulating HTA committees, participants received information about four conditions (two common and two rare), including disease severity and prevalence, and four drugs, including alternative treatments, comparative clinical and cost effectiveness, and budget impact. In each group, participants made individual recommendations followed by a group recommendation and subsequently received a "patient submission" summarizing patients' perceptions of the condition, current therapies, unmet need, proposed therapy, and benefits-risk trade-off. They were given the option of changing their recommendation, with rationale. Each participant provided feedback on the impact of the patient submission on their individual judgement and the group decision.

Results: About one-third of decisions changed from "not recommend" to "recommend" or "recommend with conditions" for specialty drugs, whereas fewer than one in ten recommendations were modified for common drugs. Reasons for change were increased understanding of the condition, lack of alternatives and willingness to tolerate risk. There was no difference between patients and "non-patients."

Conclusions: Patient input may be important when committees have less knowledge about the condition or patients' perceptions of value and risk tolerance. There may be little impact on drugs (with comparators) for common conditions. The findings from the simulations reinforce observations of real-world HTA processes.

INTRODUCTION

Patient involvement or engagement in health-related policy, decision making, service delivery, and evaluation has become the norm in health discourse and its lexicon, if not in widespread acceptance and practice. Patients are increasingly consulted and/or engaged, from individual participation in informed consent/choice in what healthcare service or technology they receive to providing patient perspectives on research (e.g. clinical trial design), regulatory approvals (e.g. patient-relevant outcomes and benefit-risk preferences), health technology assessment (e.g. unmet needs and impact of new technology), and, to a limited extent, resource allocation (e.g. drug reimbursement committees).

In the realm of health technology assessment (HTA), over the past few years, there has been considerable discussion of the importance of patient involvement based, in part, on principles of fairness and "robustness" in decision-making[1]. However, there is little consensus on how and when to engage patients[2] and even less evidence of effectiveness of various approaches[3]. Recently, the predominant form of patient involvement has been the patient submission to the appraisal process, with groups such as NICE (National Institute for Health and Care Excellence) touting the impact of patient input4 and the preparation of patient submission templates by the Patient and Citizen Involvement Interest Group of HTAi[5].

Since the introduction of the patient submission process at the Canadian Agency for Drugs and Technologies in Health (CADTH) in 2010, we have sought to discern the impact of patient input on appraisals and recommendations, primarily by examining the Final Recommendation Report, analyzing interpretations of the patient submission (and more recently comparing these to the published Patient Submissions), and seeking clarification from the agency (to the extent possible)[6]. In Europe, HTA agencies were queried as to their perceptions of the impact of patient engagement on their assessment process[7]. However, there has been no empirical research on how the patient submission may influence the appraisal and decision making process.

OBJECTIVES

The Canadian Agency for Drugs and Technologies in Health (CADTH) allows patient input on drugs under review. The primary purpose of this study was to collect data on the perceived role and value of patient input in training sessions by conducting simulations of the HTA committee decision-making process. The sessions included both patients and other stakeholders, including industry representatives, policy makers, and reimbursement specialists. Secondary objectives of the study were to compare the perceived value of the patient submission when appraising common vs. specialty (rare, unmet needs) drugs and to compare the perceptions of patients and "non-patients" as to the value and impact of the patient perspective.

METHODS

Over the course of two years of workshop sessions with patient organizations, 36 small groups of 6-8 patients and non-patients, totalling 168 participants, were provided descriptions of four diseases, (two rare and two more prevalent), and four anonymised drug profiles. The disease descriptions included disease severity and prevalence, and the drug profiles included alternative treatments, estimates of clinical effectiveness, cost-effectiveness and budget impact.

Table A. Descriptions of conditions and treatments.

Condition	Prevalence	Impact of disease	Treatment Description	Treatment Costs
1. Chronic metabolic	High	Progressive worsening of symptoms despite continuous monitoring and starting many different (onerous) treatments; weight gain; anxiety, joint pain and depression; secondary infections, risk of death, or other complications from invasive interventions such as splenectomy or transplant.	"Fast-acting" version of the existing medicine, administered continuously via a small device, programmable to patient activity and food consumption level. Results in a more stable level of the drug but also a greater risk of side effects such as site infections.	$5,250 for the device plus $1,500 per year for supplies, totalling $11,250 for 4 years.
2. Blood disorder	Low	Night sweats, fatigue, shortness of breath, pain, enlarged spleen resulting in abdominal swelling; loss of appetite; weight loss; rash/itching and fever; therapies may not improve quality of life; sides effects of nausea, fatigue, diarrhea, abnormal liver function and blood counts.	First approved therapy targeted for a subpopulation with a specific gene mutation. Primary outcomes are reduced spleen size and improved quality of life, but there are no long-term survival data.	Daily pill to be taken for the rest of the patient's life at an average cost of $60,000 per year. The ICER is between $199,118 and $259,698 per QALY.
3. Subgroup of common cancer	Medium	Non-curable disease; chemotherapies improve survival and quality of life; improvements modest with median time to progression four months.	Treatment, based on a specific genetic mutation, has a 57% response rate after just 8 weeks. Adverse effects are mild.	Cost is $8,900 per month with an average regimen of 9 months. The estimated ICER is between $283,303 and $301,141 per QALY. Access to the therapy relies on a positive test for the genetic mutation, costing about $1,400 per person.
4. Inherited metabolic disorder	Low	Nightly dosing affects family quality of life; because of odor, children often stigmatized; difficult to stay on treatment; marriages often fail due to strain.	Current treatment (A) is an oral therapy that must be taken every six hours; resulting in an unpleasant odor similar to rotten eggs, which is a deterrent to compliance. Costs about $15,000 a year and is covered by most private insurers and some provincial plans, upon individual application. Drug B is a "slow-release" reformulation of the same molecule, equivalent in safety and efficacy, and needs to be taken every 12 hours. There is also NO odor associated with metabolism. There is no evidence of reduction in kidney failure or improved long-term survival.	Cost per patient per year of Drug B is approximately $115,000. The ICER has been estimated to be $120,000/QALY.

The deliberative process followed the format described below.

STEP 1: For each of the four health conditions participants received information on prevalence and outcomes. For each of the new drugs under consideration, information was provided regarding clinical and comparative effectiveness, cost effectiveness (including ICERs), quality of life impact, and budget impact. These details are shown in Table A above. Each participant was asked to make an individual recommendation as to whether each drug should be recommended for funding.

STEP 2: Working in small groups simulating a review committee, participants were instructed to share their individual recommendations and to arrive at a group consensus. Each group was allowed to choose only two of the four drugs to recommend for funding.

STEP 3: For each drug, the group was presented with a "patient submission" summarizing patients' perceptions of the condition, current therapies, unmet need, proposed therapy, and how they would manage the benefits-risk trade-off, as described in Table B below. The group was asked to consider whether they wished to change their recommendation, and to provide the rationale for the decision.

Table B. Patient submission contents.

Condition	Relevant Outcomes	New drug experience
1. Chronic metabolic	Serious long-term complications incl. blindness, heart disease, kidney problems, nerve damage; disease management (keeping blood levels stable) with manageable treatment routines while leading a normal life	Mostly positive, with side effects that improved over time; first time successful in managing their condition; 20% said did not work; 10% stopped due to side effects
2. Blood disorder	Survival; quality of life, reduction in symptoms	Significant improvement in quality of life; continue to work and spend time with family; drug more effective and very well tolerated
3. Subgroup of common cancer	Patients choose between highly toxic chemotherapy with debilitating side effects to months without therapy followed by palliative care	Adverse events of visual disorders, nausea, vomiting, diarrhea, and constipation similar to current therapy and tolerable
4. Inherited metabolic disorder	Effective but less burdensome therapy which maintains vital treatment adherence	Parents overwhelmingly positive about dosing schedule and lack of odor; patients able to stay on therapy and participate in school and social activities; risks small and benefits significant

STEP 4: Each participant provided feedback on the impact of the patient submission on their individual judgement and the group decision.

RESULTS

Results were combined from 168 patients and other stakeholders across six training sessions conducted over two years. In total, there were results from 36 groups making 144 recommendations about drug funding. In addition, at the end of the exercise, each group was required to choose only two of the four drugs to recommend for funding.

Table C. Approvals before and after patient submission; provision within constrained budget.

	Chronic Metabolic	Rare Blood Cancer	Subgroup of common cancer	Inherited Metabolic Disorder
Pre-patient submission	67% (24/36)	61% (22/36)	56% (20/36)	47% (19/36)
Stated rationale	Improved quality of life; no ICER but reasonable increase; high potential budget impact because of numbers	No other treatments; no proof of long-term benefit; small numbers; approve despite very high $/QALY	Response rate moderate; no long term survival data, very high ICER plus unknown cost of screening test	Large impact on patient and family quality of life; no difference in short-term outcomes; no long-term data; very high ICER
Post-patient submission	61% (22/36)	86% (31/36)	64% (23/36)	75% (27/36)
Stated rationale	Not working for 1/3; still required monitoring and diet	Impact on quality of life; return to work and daily activity; side effects tolerated	No alternatives; costly to screen many to find few, possible false positives	Major impact on quality of life for children and family
Difference	-6%	+25%	+8%	+28%
Final 2/4 inclusion	14% (5/36)	86% (31/36)	25% (9/36)	75% (27/36)

Patients and non-patients did not differ significantly in terms of their recommendations. Moreover, findings were similar across sessions in different locations and at different times. Prior to the patient submissions, there was relatively little difference between common and rare disease drugs in terms of recommendations for approval, with 67% and 56% support for common conditions as compared to 61% and 47% support for rare conditions.

Following review of the patient submissions, about one-third of groups changed their decisions from "do not recommend" to "recommend" or "recommend with conditions" for specialty (rare disease) drugs, whereas fewer than one in ten recommendations were modified for the drugs for more common conditions. Recommendations for the treatment for rare blood cancer increased from 61% to 86%; similarly, the recommendation for funding for the rare genetic condition went up from 47% to 75%. The reasons given by the participants for the changes were increased understanding of the condition, lack of alternatives, impact on quality of life, and willingness to tolerate risk.

When asked to make a "forced choice" as to which of the two (out of four drugs) to recommend for funding, the majority chose the rare conditions, (86% voted for the rare blood cancer treatment and 75% agreed to recommend the new drug for rare genetic condition). Only 14% chose the improved therapy for the chronic metabolic condition, whereas 25% chose the treatment for the genetically identified subgroup of the common disease.

CONCLUSIONS

Patient input may be important when committees have less knowledge about the condition or patients' perceptions of value and risk tolerance. There may be little impact on drugs (with comparators) for common conditions. The findings from the simulations reinforce observations of real-world HTA processes.

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