Reviewer’s report

Title: Patients' perspectives can be integrated in health technology assessments: an exploratory analysis of CADTH Common Drug Review

Version: 0 Date: 18 Dec 2015

Reviewer: Karen Facey

Reviewer's report:

OVERARCHING COMMENT

There are very few evaluations of how patient input has been used in HTA, so this research by a highly regarded HTA agency will be an important contribution to the field of HTA. The paper is well written and introduces the complex world of this specific HTA process well. However, there is a fundamental issue of interpretation in this research - the issues extracted from the patient input are not all "outcomes" and it would be more helpful if the broad potential for use of this input was more clearly delineated.

SPECIFIC COMMENTS

ABSTRACT

Will need to be rewritten given comments below.

PLAIN LANGUAGE SUMMARY

Second sentence - this seems to imply that patients only provide input on benefits the treatment should achieve. However, the submission template covers their experience of living with the disease and of using current treatments. So this is much broader and actually is important when you see the results later, many of the items relate to issues of living with the disease.

Bullet 2 may be better as the first bullet
Bullet 3 could be made shorter to make it clearer.

The final sentences do not flow clearly. Would a more valuable final sentence be about the value of patient input to decision makers.

BACKGROUND

Paragraph 2 - it would be helpful to provide a link to the patient group submission form.

Page 5- line 9 - "peek" is an odd word to use in this context, provide a more professional term

Page 5 - line 27 - The sentence "within the stakeholder principle" does not make sense. If you are referring to principles for HTA you need to be clearer about this and reference them, and I assume that you are referring to stakeholder INVOLVEMENT.

Page5 - line 37 - unique is an odd word to use in this context - do you mean different - do you mean covering different medical conditions?

Page 6 - top sentence - what about the role of clinical opinion?

Page 6 - lines 11-18 - This doesn't seem to be a full and accurate reflection of the impact of patients perspectives in HTA. Patients perspectives can provide more information than just a specific treatment need and clinical and economic evaluations do not merely seek to show whether a new treatment meets that need. This section really needs to be "unpacked" and should influence all the later work.
METHODS

This section needs to be formalised.

The section about the documents is long and not really about methods. It's setting context, so perhaps move it to background or make a table. Methods should start by saying what the sampling frame was - as described in the paragraph on page 8, lines 14-29.

Section 2 - it states that the protocol should reflect outcomes of importance identified in the summary of patient input, but there is a need to explain how these outcomes are elicited as the patient group submission form does not explicitly ask for outcomes - does the reviewer determine the outcomes from the submission forms - how are forms from different groups handled?

Explain whether the form of patient input and the process of HTA remained the same over the period and if not, in discussion describe what impact this may have had.

Page 7, from line38, needs to be clearer about actual process. "through iterative readings" is too vague - who did the "reading", how was information extracted?

Page 8, last paragraph - please describe how patients were involved in this research (advising on design, helping with interpretation of results, etc)

Page 9 - needs some text to explain that there are a wide range of conditions that were included, so you may expect that different issues would be important.

Give further justification as to why you chose to categorise using Porter's hierarchy given the patient group submission template has different sections. In particular discuss whether it is appropriate for terminal illnesses given the second category is about recovery.
More explanation is needed about how you put issues in the hierarchy. In table 2, it's not entirely clear why some of the issues are in tier 2 and not tier 1 - eg why is avoiding hospitalization about progress of recovery and not about health status achieved or retained.

In addition, some items seem to be squeezed into a tier - eg cost.

RESULTS

1st sentence specify tiers from the Porter's... hierarchy.

The table is a little difficult to read, so it could help to describe one row. For example - In the 30 assessments, 25 of the patient summaries mentioned symptom relief as outlined in the description column. These issues were mentioned in 21 of the protocols, 22 of the drug trials and 17 of the CDEC discussions.

WHAT IS IMPORTANT TO PATIENTS?

Presumably this is part of the results section, but that's not obvious - it reads more as a discussion, with a wider interpretation of the results. This would be better moved to a discussion section.

CAPTURED WITHIN CDR ASSESSMENT PROTOCOLS? - GENERAL COMMENTS FOR ALL THESE SECTIONS

This sequence of sub-titles is not very clear.

As explained above the results need to be written in a different way. Patient group submission forms do not specifically talk about outcomes as you imply and neither to appraisal committees - they consider a wide range of issues to determine the value of a treatment - the most valuable input from patients may be about context not about specific outcomes. This is exemplified in lines 24-31 on page 15.
There is an implication that we need to add a range of outcomes that patients consider to be important into trials and assessments, but no discussion of the fact that a confirmatory trial has one primary outcome, several secondary outcomes and perhaps some other outcomes. They must be tested carefully to avoid biases due to multiplicity and trials are designed to have power for the primary outcome only. Furthermore, for many of the issues raised, there are no outcomes and so they would need to be developed.

SPECIFIC COMMENTS ON THESE SECTIONS

Page 14 - top full sentence. This is a rather odd statement about safety, which should be evaluated by regulators and data should not be lacking about safety of comparators, otherwise treatments would not be recommended for marketing.

Page 15 - lines 29-38 - what evidence do you have that this is the case? There may be a range factors that influence CDEC members, such as clinical experience, personally knowing a patient, general categories of consideration (e.g. end of life, unmet need etc).

IMPLICATIONS FOR HTA AGENCIES

1st paragraph - Many HTA agencies don't use assessment protocols, so can the implications for HTA agencies be made more general?

Somewhat surprising that this section doesn't discuss the potential value of patient input and the need to measure its impact and consider how its used by appraisal committees.

DISCUSSION (suggested section)

Section 1 in methods explains that CADTH reviewers summarize patient input. CADTH is the only agency to do this. Other agencies make the full input available. Discuss the impact of this on your research and implications for others.
Did you consider looking at any sub-groups?

CONCLUSIONS

3rd paragraph - Patient input isn't necessarily qualitative, it may present a well organised survey or other quantitative research.

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1. I have received fees from pharma, HTA organisations and patient organisations. 
2. No
3. No
4. I have received 2 one off fees and travel expenses for giving lectures at CADTH. 
5. No
6. I do voluntary work for HTAi relating to patient involvement and for Scottish public sector relating to HTA

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