Reviewer’s report

Title: What do patients with unmet medical needs want? A qualitative study of patients’ views and experiences with expanded access to unapproved, investigational treatments in the Netherlands

Version: 0 Date: 13 Sep 2018

Reviewer: Ghislaine Van Thiel

Reviewer's report:

This manuscript addresses the relevant topic of patients' experiences with and views on expanded access and other non-standard treatment options. The authors set out to address a relevant knowledge gap. The qualitative research methods are suitable and well executed.

I have three areas of comment: (1) definitions (2) presentation of results and (3) justification of the conclusion.

(1) Throughout the manuscript a variety of situations is discussed: the use of unapproved drugs, access to and participation in trials, the use of approved but not (yet) reimbursed drugs, complementary therapies and non-standard treatment options. The results show that patients' knowledge does not allow them to make clear distinctions between these situations. However, the authors can, but they chose not to structure the results in such a way that would elucidate the patients views on each of these situations. This is in my view important because a patients' willingness to use an approved, yet not reimbursed drug is essentially different from participating in a phase 1 trial, which in turn is very different from an phase 3 trial. The confusion about the exact topic of the paper is therefore very present and makes the paper difficult to read.

- In the background section, the authors state that the use of investigational drugs is an 'entirely different option' than complementary and alternative medicine. However throughout the manuscript, these treatments resurface and it seems that patients do not think or know that these are so different. What does this mean for the main research question?

(2) The presentation of the results starts with the theme of patients experiences with standard of care. The paper is already quite long and the relation between this theme and the main topic is not well addressed. I suggest the authors provide a link between the theme and the paper's topic or omit this section.

(3) The conclusion starts with reclaiming that patient understanding is low. I was really surprised to read the conclusion that 'The findings strengthen the notion that patient may be capable of making well-considered choices with regard to the use of unapproved, investigational drugs'. Of course, in theory every competent person may be capable to decide, but that is not what this conclusion refers to. In my view this conclusion cannot be supported by the results (minimum conditions for informed choice are not met). If the conclusion leans on the 'suggestion' that significant increase of high expectations or false hope will not occur: I do not see how this is
supported by the results, or any other studies. The patients views seem to me to be all about hope and expectation. I would be interested in the authors’ motivation to draw this conclusion.

Are the methods appropriate and well described?
If not, please specify what is required in your comments to the authors.

Yes

Does the work include the necessary controls?
If not, please specify which controls are required in your comments to the authors.

Yes

Are the conclusions drawn adequately supported by the data shown?
If not, please explain in your comments to the authors.

No

Are you able to assess any statistics in the manuscript or would you recommend an additional statistical review?
If an additional statistical review is recommended, please specify what aspects require further assessment in your comments to the editors.

Not relevant to this manuscript

Quality of written English
Please indicate the quality of language in the manuscript:

Acceptable

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