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: 1 Date: 13 Sep 2019

Reviewer: Reviewer 2

Reviewer's report:

PEER REVIEWER ASSESSMENTS:

OBJECTIVE - Full research articles: is there a clear objective that addresses one or several testable research questions? (Brief or other article types: is there a clear objective?)

Yes - there is a clear objective

DESIGN - Is the current approach (including controls and analysis protocols) appropriate for the objective?

Yes - the approach is appropriate

EXECUTION - Are the experiments and analyses performed with sufficient technical rigor to allow confidence in the results?

Yes - experiments and analyses were performed appropriately

STATISTICS - Is the use of statistics in the manuscript appropriate?

N/A - there are no statistics in this study

INTERPRETATION - Is the current interpretation/discussion of the results reasonable and not overstated?

No - there are minor issues

OVERALL MANUSCRIPT POTENTIAL - Has the author addressed your concerns sufficiently for you to now recommend the work as a technically sound contribution? If not, can further revisions be made to make the work technically sound?
Probably - with minor revisions

PEER REVIEWER COMMENTS:

GENERAL COMMENTS: The authors have generally handled most of the reviewers' comments reasonably well. However, there is one issue raised by previous reviewers 2 and 3 that I do not think has been appropriately dealt with and that further revisions are needed. The conclusion (the last 3 sentences of it) still appears to be overstated and not consistently supported by the results. This is even more of concern when considering the sample of patients recruited and the authors' acknowledgement that participants were more assertive than is perhaps 'the norm'.

REQUESTED REVISIONS:

Abstract and Title: suggest it's added that this study was conducted in the Netherlands as these issues and experiences are likely to be very different in different countries/health systems.

Title: suggest that the first part of the title (What do patients with unmet medical needs want") is removed as this is a very vague phrase and could be referring to numerous aspect of unmet medical needs. The second half of the title is much more informative.

Limitation: With no mention that any reflexivity was undertaken in this qualitative analysis (which may account for some of the overstating of conclusions), this should be acknowledged in the limitations section.

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.
Quality of written English

Please indicate the quality of language in the manuscript:

Acceptable

Declaration of competing interests

Please complete a declaration of competing interests, considering the following questions:

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