Reviewer's report

Title: The Patient Reported Outcomes, Burdens and Experiences (PROBE) Project: Development and Evaluation of a questionnaire assessing patient reported outcomes in people with hemophilia

Version: 0 Date: 12 Nov 2017

Reviewer: Gareth McCray

Reviewer's report:

General comments

This paper reports on a multi-country pilot study whose overarching objective is to develop a questionnaire to gather data on outcomes relevant to those with haemophilia.

Congratulations on the completion of such an ambitions and wide-reaching study. I intend this to be constructive criticism and hope you will accept it as such. I enjoyed reading the first half of your paper and the project itself sounds fascinating, however, I do not think that the work presented in this paper is currently strong enough for publication. That said, you clearly have enough data that has gone unreported to remedy this. My main problem with this paper is that I am not sure how interesting or instructive it is to a more general audience. I think firstly, the qualitative work that went into the construction, i.e., the development, refinement and finalisation of the measure and secondly, some initial psychometric properties of the measure need to be reported here.

The details regarding the construction of the tool, something I would have been very interested to read, are simply presented as methodology and not elaborated upon in the results. I would have liked to have read, e.g., i) more information from the focus groups and interviews on how exactly the tool was refined, ii) more details of how the questionnaire was finalised, and specifically how completeness, relevance, and clarity were defined and assessed, iii) the domain structure of the tool (i.e. the theoretical domains, how many items measuring each theoretical domain, etc.), iv) how the tool is intended to be scored, v) any translation issues that emerged, and, vi) most importantly, specifically, what kinds of things the scores will be used for and how this will be of benefit to stakeholders. N.B. you show that the tool is likely to be low-cost, but you do not spell out the benefits well enough, to my mind.

Given the relatively large amount of data you have, I would have liked to have read about the initial psychometric properties of the items, and seen the items themselves. If this is not possible for commercial reasons, then perhaps some summary of the item could be used as proxy rather than the final wording. Specifically, if you are intending to construct a score, I would have liked to have seen, e.g., i) Item statistics (facility values, point biserial), ii) reliability statistics
(Cronbach's alpha, alpha if removed), and, iii) initial scores across countries. I know that this is a pilot study, but 656+ participants is more than enough for initial, and valuable, psychometric assessment.

The abovementioned additional information is all related to the piloting and construction of the first iteration of your tool. My intuition is that you are intending to publish this in additional papers. Clearly, you have too much for one single paper, but it is my opinion that this paper, as it stands, is "too thin" and needs strengthening to be suitable for publication.

Additional issues are:

i) It seems that you seem to mix up the objectives of the project and the objectives of the part of the project reported in the paper, at times. This is very confusing for the reader. I only worked about what the paper was reporting on out half way through reading it. Please tell me what the specific objectives of this paper is in the abstract.

ii) I feel you often present more detail than is needed for the general reader; e.g., giving exact dates of meetings, too much description research group (we all do this but it is a bit tedious for a general reader).

iii) Sometimes you stray a bit far from the path of plain English, e.g., "through an iterative, bi-directional, and non-structured consultation approach" (we all do this, but maybe just reign it in).

Major issues

P8L107-10: I do not really know what this sentence means and by extension what your objective are. Please could you nudge the language towards plain English.

P14L242-243: Is this a good way of calculating cost per questionnaire? Isn't it the case that the more questionnaires are collected the lower the cost per questionnaire, i.e. economies of scale?

P13L233-236: Please can you state this objective, in this way, earlier? It is only after reading this line that I have worked out what the point of the study is. Previous formulations of the objectives did not really get this across.
Minor issues

P4L49: Briefly say what kind of experiential data.

P4L59: Briefly say what the "EQ-5D-5L" and "EQ-VAS" are here.

P4L61: "...recruiting 656 participants. Most participants (474, 71%) completed the questionnaire...".

Just say 71% of participants, no need to say exactly how many, or use the qualifier "mostly".

P6L82: In British English this would be "capable of maintaining". Maybe check "Capable to maintain" is acceptable in American English.

P6L87: You say "Assessment of patient important outcomes beyond bleeding frequency and functional status, including for example burden of treatment, impact on lifestyle and life choices are major aspects that need to be quantified". Could you just add a few words to the end of this sentence to say why this is true? E.g., "in order to ensure patient centred treatment" It would tee up the following paragraph much better.

P6L92: Move "(HTA)" to directly follow the words for which it stands, i.e., move it to before the word "agencies"

P6L93 I would say "... patients have unique perspectives and may consider …".

P10L149 - 150: Why are the date and place important to the reader?

P8L110: "overarching goal". Are there not multiple goals, this is unclear.

P8L114 - P9L131: I am not sure why this much detail about the research group's formulation is relevant or interesting in this paper.

P9L139: Be more specific than "geography".
P11L182: What do you mean by "ancillary material"?

P11L185: What do you mean by "completeness relevance"? Do you mean content validity?

P12L200: I am not sure what you mean by "grade level scoring".

P14245-P15L266: I think that this paragraph belongs in the methodology.

Nitpicking

P8L118 Why use capital C on "Co-investigators"?

P8L116: too many commas after MS

P9L136-137: "through an iterative, bi-directional, and non-structured consultation approach". Unless this is key terminology that I am entirely ignorant of, I am not sure it adds anything to the substantive meaning of the text. If it does add something be specific about what it adds, in plain English.

P9L138: Why capital I on Investigators?

P10L165: Perhaps use a weightier verb than scan.

P9L140: The "and" is in the wrong place.

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An article whose findings are important to those with closely related research interests

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Please indicate the quality of language in the manuscript:

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