Reviewer’s report

Title: Patient organization involvement and the challenge of securing access to treatments for rare diseases: report of a policy engagement workshop

Version: 0 Date: 19 Mar 2017

Reviewer: Rosie Gowran

Reviewer’s report:

Dear Authors, the concepts within this paper are important in the treatment of rare diseases. The sample size in small and the data collected could be considered limited given the overall presentation of this paper. I am unsure if the data presented is strong enough evidence.

Title/ key words: these could be reviewed as they do not clearly reflect the content of this paper.

Abstract: this could be more clearly laid out indicating: Introduction, Aim, Method, Findings, Recommendations

Introduction / Literature review:

Background and need for this research needs to be developed further with evidence of broader reading specifically related to the key concepts: Defining rare diseases (choosing some examples and specific considerations for these groups); defining treatment development, intervention and evaluation strategies; current benefits and reported challenges; the need for further investigation leading to the aim of this study.

Method: Given the limited data this could perhaps be presented as a single case study, with clearly described methodology and theoretical basis.

This needs to be more clearly laid out and include ethics (permissions to name participants?)

Three distinct groups appear to be identified - these need to be clearly laid out and represented within the table provided.

How were participants selected and why? How many participants were invited and how many attended from each distinct group.

Workshop two day format and agenda should be included. How were these days facilitated? How was data collected, collated and analysed to represent the stakeholder view points? This is important to include so study could be replicated by others.
Results / Findings (as qualitative)

These read more like a discussion rather than an analysis. It is unclear how these key headings / themes emerged and how they represent the view points of participants.

Discussion:

This requires further development based on comments above. Include strengths and limitations. Include recommendations relating to key messages and need for larger study working with specific sub-groups as they will all have distinct needs. Overall conclusions.

My overall suggestion is to develop this work further (methodology) by considering more specific sub-groups and a series of workshops with clearly defined aims and objectives.

Wishing you all the best with this work.

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

Quality of written English
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