Reviewer's report

Title: A new model of financing hemophilia

Version: 2
Date: 6 May 2015

Reviewer: Joanne Wu

Reviewer’s report:

• Discretionary Revisions (which are recommendations for improvement but which the author can choose to ignore)

1) On page 4, line 5, please provide reference for haemophilia prevalence statistics.

2) Should the sections of Background, Haemophilia Costs, Integrated Disease Management be organized to a section as “Introduction” that should be the same level as Materials and Methods, Results, Discussion, and Conclusions.

3) On page 8, the section of integrated disease management discussed the health systems and integrated disease management program for couple diseases in Portugal. However, it is unclear how these programs related to haemophilia care. Authors should provide information on how such programs have impacted on haemophilia care.

• Minor Essential Revisions (such as missing labels on figures, or the wrong use of a term, which the author can be trusted to correct)

1) The numeric format presented in the text is confused. I guess that a decimal should represent the standard format of thousand separator. For example, 5.000-10.000 male births (page 4, line 5) should be 5,000-10,000 (five thousand to ten thousand).

2) On page 4, line 9, it is unclear what are the percentages represent? Are they factor lever? Please specify.

3) Overall, the presentation format should be revised to make it easy to follow. Many sentences were in one paragraph. They should be grouped to a specific topic in one paragraph. Specifically,

# On page 4, lines 9 to 18 should be in the first paragraph to discuss what haemophilia is and its treatment.

# On page 4 line 15, “As haemophilia is an inherited deficiency of one clotting factor” is redundancy, author may consider delete it, and start with the statement “The treatment of haemophiliac is.....”

4) On page 5, line 15, the paragraph should be moved to follow the above paragraph.

5) On page 6, line 8, should the word “that” be “than”. The sentence should read as: Although prophylaxis seems to be more costly than on demand therapy.....

6) On page 6, line 11, it is unclear how depression related to the topic. Should
you specify haemophilia patients with depression?

7) On page 6, line 20, the Globe’s example should be specified for the U.S.

8) On page 8, line 4, it is unclear what is excellent outcomes, what is the definition of outcomes?

9) On page 7, last paragraph, it is unclear to me where “increase of 165% …” come from the table 1, (35,300-13,200)/13,200=167%, not 165%. The table 1 did not show national costs of hospital drugs rather than total drug cost. Should you include the hospital drug cost?

10) On page 8, line 5, should 10,2% be 10.2%?

11) On page 9, line 24, it is unclear what risk do the authors meant. Is it risk of bleeding?

12) The descriptions in Table 2 should be consistent with the text. The age group should be adults and children, not adult and pediatric. The table 2 should separate the title and footnote. The additional description of the table should be included in the footnote, not in the title. The table only included age as demographic information; other variables belonged to clinical characteristics. Therefore, should the title be revised to reflect the table contents? Does the data set include employment, education, income, and other demographic variables? Moreover, Table 2 should be part of the results, and should be presented in the results section.

13) On page 9, line 23, EPE (CHSJ) should be spelled out at the first time appeared in the text.

14) On page 10, line 14, study purpose should be moved up to the section of introduction.

15) On page 10, line 22, it is unclear how the authors converted the outpatient visits, inpatient admission, surgeries and emergencies to monetary values using the prices of hospital funding. Please specify or provide examples. What cost components were included in the total costs?

16) On page 11, line 4, again, the descriptions of health care utilization should be in the results section rather than method section.

17) On page 11, lines 6 to 11, the paragraph should be presented in the results section.

18) On page 11, lines 12 to 18, the paragraph discussed the health care utilization which should be in the discussion section.

19) On page 12, line 9, cost comparisons between severe and moderate/mild should be included p values. The table 3, 4, 5 also should include the p values for comparisons.

20) On page 12, line 22, please specify what are the two groups, adults compared with children?

21) It looks like the results section also included some discussion (e.g. page 13, lines 14 to page 14 line 2. I would suggest separating these discussion to the discussion section.
22) On page 15, line 1, do you mean the small sample size rather than the reduced study sample? Why the study sample was reduced?

• Major Compulsory Revisions (which the author must respond to before a decision on publication can be reached)

1) It is unclear the study design whether the patients were selected as study sample from Hemophilia Comprehensive Care Centre or all patients received care from the centre were included. What are selection and exclusion criteria? The major concern is how a small sample like the current study can represent benchmark of haemophilia care for the Portugal.

2) Since the study period covered 3 years from 2011 to 2013, it is unclear how the costs were constructed, e.g. what time period to construct costs for each patients, 6 months or one year, or two years, or three years?

3) The methods section lacked of statistical analysis method.

4) Table 3, it looks like the annual total cost is the sum of clotting factor costs and hospital visits and hospitalizations costs. Then, does the hospital visits include outpatients and emergency room visits cost?

5) It is unclear to me what do the comprehensive price of hemophilia mean at Table 6. How and why the authors proposed the numbers in the table 6.

Level of interest: An article of limited interest

Quality of written English: Not suitable for publication unless extensively edited

Statistical review: Yes, and I have assessed the statistics in my report.