Reviewer's report

Title: Stem cells and beta cell replacement therapy: a prospective health technology assessment study

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Reviewer: Hans-Ewald Hohmeier

Reviewer's report:

The authors investigate the cost-effectiveness of stem cell-derived implantable insulin replacement therapy in comparison to intensive insulin therapy.

Many parameters of this analysis are based on a cost-effectiveness model of islet transplantation the authors published previously. However in this analysis the authors assume that the novel encapsulation technology removes the need for immunosuppressive therapy.

Critique:

1. Stem-cell derived beta-cell replacement therapy is a novel technology that is currently in phase 1 trials. The authors have included various scenarios for some of the unknown costs. At this point the viability and longevity of the implanted cells in humans is not known. In the previous paper the authors considered up to 4 transplantations with increasing proportion of functioning grafts from first to fourth transplant. Could the authors comment on the assumed average viability per treatment used in this analysis?

2. One of the assumption for this analysis is that no immunosuppressive therapy will be necessary. In the results section (line 226 and 227) the authors refer to an analysis including immunosuppression. Did the authors run an analysis including immunosuppression?

3. It is not clear how the challenges of donor-harvested transplants in Canada as discussed in lines 249-260 affect costs of the stem-cell replacement therapy.

This is a very thorough analysis of the cost-effectiveness of stem-cell derived beta-cell replacement therapy.
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.

Unable to assess

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

Yes

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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