Reviewer's report

Title: Cost Utility Analysis of Reduced Intensity Hematopoietic Stem Cell Transplantation in Adolescence and Young Adult with Severe Thalassemia Compared to Hypertransfusion and Iron Chelation Program

Version: 2 Date: 19 November 2012

Reviewer: Nandita Khera

Reviewer's report:

This study by Sruamsiri is a cost-utility analysis using Markov model to compare reduced intensity HCT using a related donor with iron-chelating therapy for adolescent and young adults with thalassemia in Thailand. It is a well-designed study that attempts to answer a very relevant and timely question given the ongoing general discussions of costs of healthcare even in the developed nations.

Major Revisions:

a) In the Methods section: would describe the base case scenario and the assumptions that you make for its analysis.

b) In the Discussions section: Would consider adding review of prior studies (both clinical and economic) in this area to put the results of your study in a broader perspective.

c) Would also consider pointing out the fact that the cost-effectiveness of transplant has the potential to improve further as transplant therapy is a moving target while the improvement in non-transplant therapy has been a relatively slower process.

d) In the last paragraph in Discussion section where decision-making for policy makers is described, would consider also adding some information about decision-making at individual level. Studies have shown lower acceptability of HCT as treatment for non-malignant heme disorders considering the higher short-term mortality, especially in pediatric population where parents are the decision-makers which may impact the extent to which HCT is used. (Kodish et al. NEJM 1991; 325:1349)

Minor revisions:

a) In the Background section: would consider replacing reference 7 by (Gaziev J et al. Ann NY Acd Sci 2005, 1054: 196) since it is more specific to thalassemia and specifically for RIC rather than non-myeloablative regimen (which is only TBI 200cGy+/- Flu)

b) Would consider providing some information about the accuracy in estimation of long-term cost data for HCT. In the US, one of the major problems in estimating long-term costs of HCT is the fragmentation of care of these patients since they are usually transitioned to their oncologist/primary physician from the
transplant center after a certain period of time. If the practice in Thailand is different and the transplant center continues to follow the patient for prolonged periods of time, it should be mentioned; if not, it should be indicated that the long-term costs may be under-estimated.

c) In Table 1: would consider adding a notation to remind the readers that the costs beyond Q4 are on an annual basis and not for 3 month period.

Level of interest: An article whose findings are important to those with closely related research interests

Quality of written English: Needs some language corrections before being published

Statistical review: Yes, but I do not feel adequately qualified to assess the statistics.

Declaration of competing interests:
'I declare that I have no competing interests'