



Effective Health Care

Bone Marrow Transplants in Children Nomination Summary Document

Results of Topic Selection Process & Next Steps

- Bone marrow transplants (BMT) in children will go forward for refinement as a comparative effectiveness or effectiveness review. The scope of this topic, including populations, interventions, comparators, and outcomes, will be further developed in the refinement phase.
- When key questions have been drafted, they will be posted on the AHRQ Web site and open for public comment. To sign up for notification when this and other Effective Health Care (EHC) Program topics are posted for public comment, please to go <http://effectivehealthcare.ahrq.gov/index.cfm/join-the-email-list1/>.

Topic Description

Nominator: Public payer

Nomination Summary: The nominator asks for which conditions BMT has been shown to be effective and for which conditions is BMT investigational only.

Key Questions from Nominator: None

Considerations

- The topic meets all EHC Program selection criteria. (For more information, see <http://effectivehealthcare.ahrq.gov/index.cfm/submit-a-suggestion-for-research/how-are-research-topics-chosen/>.)
- A more specific term for 'bone marrow transplantation' is 'hematopoietic stem cell transplantation' (HSCT). HSCTs are used to treat both malignant and non-malignant diseases in children. HSCT is associated with high morbidity and mortality in all ages and all diseases. Factors that impact the appropriateness and success of any HSCT include the stage and/or severity of the disease, the patient's age and history of previous transplants, the type of transplant, and the source of the HSC.
- Due to the large number of diseases for which HSCT is a treatment option (or experimental therapy), there is a large body of literature regarding HSCT across many diseases. Given the cost of treatment, the severity of diseases that HSCT is used to treat, and the prevalence of adverse events associated with HSCT, a comprehensive review that focuses on both benefits and harms across a set of diseases

will likely be beneficial. The diseases covered in the AHRQ review will be defined during the refinement phase.