



Title	Clinical Effectiveness and Cost Effectiveness of Stem Cell Transplantation in the Management of Acute Leukemia: A Systematic Review
Agency	NETSCC, HTA, NIHR Evaluation and Trials Coordinating Centre Alpha House, University of Southampton Science Park, Southampton, SO16 7NS, United Kingdom; Tel: +44 2380 595 586, Fax: +44 2380 595 639; hta@soton.ac.uk, www.hta.ac.uk
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Aim

To provide a systematic overview of the best available evidence on the clinical and cost effectiveness of stem cell transplantation (SCT) in treating acute leukemia.

Conclusions and results

Specific objectives: 1) systematically identify and review published systematic reviews, meta-analyses, and economic literature; 2) systematically identify new evidence from randomized controlled trials (RCTs) and donor versus no donor (DvND) studies not included in previous reviews and meta-analyses; and 3) map information from the above sources and inventory the best available evidence to help inform future research.

Fifteen systematic reviews/meta-analyses met the inclusion criteria for the review of clinical effectiveness, 13 of which were published from 2004 onward. Most reviews appeared to have omitted an appreciable proportion of potentially available evidence. The best available evidence for effectiveness of allogeneic SCT using stem cells from matched sibling donors came from DvND studies: sufficient evidence supported the use of allogeneic SCT in adult acute myeloid leukemia (AML) in first complete remission (CR1) except in good-risk patients, in childhood AML in CR1 (role of risk stratification unclear), and in adult acute lymphoblastic leukemia (ALL) in CR1 (role of risk stratification unclear). There was conflicting evidence in childhood ALL in CR1 and a paucity of evidence from DvND studies for all decision problems concerning patient groups in second or subsequent remission or with refractory disease (CR2+). The best available evidence for effectiveness of autologous SCT came from RCTs: evidence suggested that autologous SCT was similar to or less effective than chemotherapy. There was a paucity of evidence from published reviews of RCTs for comparisons between different sources of stem cells and between different SCT techniques. Nineteen studies met the inclusion criteria in the cost-effectiveness review, most reporting only cost information, and only one incorporating an

economic model. Despite a wealth of information on costs, and some on cost-effectiveness of allogeneic SCT in adults with AML, very limited evidence concerned relative costs and cost effectiveness for other decision problems covered in this report. Firm conclusions could not be drawn on the cost effectiveness of SCT in the UK NHS.

Recommendations

This report summarizes best available evidence and discusses its implications, but does not make recommendations about policy or about clinical care.

Methods

For clinical effectiveness, a systematic review of published systematic reviews and meta-analyses was carried out, supplemented with searches and mapping of recent RCTs and DvND studies not covered in existing reviews. A systematic review of cost and cost-effective studies was also conducted.

Further research/reviews required

Priorities for further research were offered according to the gaps in the evidence identified in the report.