Title **Therapeutic Use of Hematopoietic Stem Cells From Cord Blood**

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Aim

To evaluate the scientific basis for the therapeutic use of cord blood stem cells.

Conclusions and results

- Autologous transplants of stem cells have yet to be reported or documented. Published results on the therapeutic use of stem cells from cord blood regard allogeneic use.
- More than 2,000 patients (about 1,600 children and 400 adults) have been transplanted with allogeneic stem cells from cord blood. While results from both related and unrelated donors are reported for children, cord blood transplantations in adults have been almost exclusively from unrelated donors.
- The clinical results of allogeneic transplants of cord blood stem cells have been compared only to results of allogeneic stem cells from bone marrow or peripheral blood in retrospective studies. These results suggest that the clinical effect of cord blood transplant, at least in children, may be comparable to transplants with stem cells from bone marrow or peripheral blood.
- The number of stem cells in the cord blood graft is often insufficient to obtain adequate engraftment.
- The risk of graft-versus-host disease is probably less in transplants with cord blood than transplants using allogeneic stem cells from bone marrow or peripheral blood with comparable HLA matching.

Methods

We systematically reviewed all available literature reporting on the clinical outcome of transplantation with the use hematopoietic stem cells from cord blood. Controlled and uncontrolled studies were included for further assessment. Clinical outcomes assessed were overall survival, disease-free/progression-free/event-free survival, engraftment, and complications/adverse effects. Studies were identified by computer-based searches in MEDLINE. Additional studies were identified by hand searching reference lists of relevant primary and secondary publications, and by manual searches using author names and text words. Studies were systematized according to study design and critically assessed for relevance, quality, and validity. Among 435 publications identified, 67 reported results of transplantation of cord blood stem cells. Of these, only 17 studies were found to meet the inclusion and quality criteria, thus, constituting the basis of the systematic review. Fifty publications were not accepted due to overlapping data, double publications, and/or low quality/validity defined as less than 20 patients or less than 10 patients per disease indication. Three cohort studies and 14 case series were included. The cohort studies have a retrospective design with historical controls. Two patient series are registry studies that report results from a large number of patients. None of the studies are population based.

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