

## **The Role of Cytotoxic Therapy with Hematopoietic Stem Cell Transplantation in the Treatment of Multiple Myeloma**

Among the primary objectives of the American Society for Blood and Marrow Transplantation are to:

- define commonly accepted medical practice
- develop standards of medical care for autologous and allogeneic transplants
- provide recommendations and guidelines about the role of transplantation as a therapeutic approach for reimbursement by third-party payers.

To this end, the Society in 1999 began the development of evidence-based reviews of the scientific and medical literature to document when blood and marrow transplantation is indicated in the treatment of selected diseases.

### **Goals**

The goals of the evidence-based reviews are to:

- assemble and critically evaluate all of the evidence regarding the role of cytotoxic therapy with hematopoietic stem cell transplantation in the treatment of each disease selected for review
- make treatment recommendations based on the available evidence
- identify discrepancies in study design or methodology among published studies that may impact the quality of the evidence
- identify needed areas of additional study

### **Guidelines**

The following guidelines are offered for the role of stem cell transplantation as therapy for multiple myeloma, and are based on consensus reached by an expert panel\* following an evidence-based review of the literature\*\*:

1. Stem cell transplantation is more effective than conventional chemotherapy as *de novo* therapy and is the recommended treatment for patients requiring treatment for multiple myeloma. Survival after stem cell transplantation is equivalent for salvage and *de novo* therapy. Stem cell transplantation as *de novo* therapy is preferred, however, because it may avoid the inconvenience, cost and risk of myelodysplasia from conventional alkylating agent therapy.
2. Autologous stem cell transplantation is currently the standard of care for multiple myeloma and is preferred over allogeneic stem cell transplantation based on available evidence. Studies are ongoing, however, to further evaluate the role of allogeneic transplant.

3. Autologous peripheral blood stem cell transplantation (PBSCT) is preferred over bone marrow transplantation.
4. Autologous purged bone marrow transplantation is not recommended as therapy for multiple myeloma.
5. Melphalan is preferred to melphalan plus total body irradiation as the conditioning regimen for autologous stem cell transplantation.
6. PBSCT using CD34 selected or CD34 unselected stem cells are recommended as equivalent in efficacy.
7. No recommendations are made for transplantation techniques that have not been adequately studied, including:
  - stem cell transplantation versus standard chemotherapy as salvage therapy
  - tandem autologous stem cell transplantation
  - autologous or allogeneic stem cell transplantation as a high-dose sequential regimen
  - allogeneic bone marrow transplantation versus PBSCT
  - a preferred allogeneic myeloablative or non-myeloablative conditioning regimen
  - maintenance therapy post-autologous stem cell transplantation.

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