

# Haemopoietic Stem Cell Transplantation for Acquired Cytopenias

Jill Hows

*University of Bristol, Bristol, United Kingdom*

---

## Abstract

In this talk the transplant management of acquired aplastic anaemia (ASAA), paroxysmal haemoglobinuria (PNH), early myelodysplastic syndrome (MDS) and refractory immune cytopenias will be reviewed.

In patients under 40 years of age with ASAA and an HLA identical sibling there are excellent data to support the continued use of allogeneic stem cell transplantation (SCT) as first line curative therapy. In older patients, those without a HLA identical sibling and those with non severe disease (international aplastic anaemia study group criteria) immunosuppressive therapy is the preferred option. Over the past two decades pilot studies have addressed the problem of transplanting young patients with ASAA who have failed IST from well matched unrelated donors. In contrast to most haematological malignancies the results have remained significantly inferior to those of identical sibling SCT. The major problem has been unacceptably high transplant related mortality especially in patients over the age of 20 years. In the past 5 years, preliminary data has been more promising using non-ablative reduced intensity conditioning protocols containing fludarabine and ATG with or without low dose total body irradiation.

Patients with the PNH/aplasia syndrome without significant haemolysis have traditionally been treated in the same way as patients with ASAA, where therapy is dictated by severity of pancytopenia, patient age and availability of a HLA identical sibling. Management of haemolytic PNH is more controversial. There are no published studies comparing SCT with supportive treatment. In a recent report from the SAA working party of EBMT the result of SCT for 121 patients with PNH were retrospectively compared with a concurrent cohort of 2444 patients transplanted for ASAA. The PNH cohort had a significantly higher median age and were transplanted significantly later after diagnosis. In the 1998-2001 era the probability of 5 year survival was 55% for PNH compared with 85% for ASAA. It was concluded that in PNH both the transplant protocol and patient selection require refinement.

The chronic leukaemia working party of the EBMT has recently analysed results of allo-SCT for MDS. As expected, patients with early MDS defined by less than 10% bone marrow blasts at transplantation fare better than patients with more advanced disease. In early MDS treatment failure is most often due to transplant related mortality rather than recurrent disease. Young patients with favourable disease using the international prognostic scoring system and transplanted without prior chemotherapy have an event free survival in excess of 60% at one year post-transplant. Results of SCT using well matched unrelated donors are similar to identical sibling SCT.

There are very limited data for SCT for refractory immune cytopenias. The autoimmune working party of the EBMT has collected data on 17 patients (10 immune thrombocytopenia, 4 pure red cell aplasia, 2 autoimmune haemolytic anaemia and one Evan's syndrome) treated by auto-SCT. The preliminary data suggest that around 50% of patients attain complete or partial responses, however only the minority, 3/13 evaluable patients, attained a sustained complete response.

---