Reduced-Intensity Stem Cell Allografting for PNH Patients in the Eculizumab Era: The Mexican Experience

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Background: Paroxysmal nocturnal haemoglobinuria (PNH) presents as two major entities: the classical form, predominantly hemolytic, and a secondary type with marrow failure and resultant aplastic anaemia (AA-PNH). Currently, the treatment of choice of the hemolytic variant is eculizumab; however, the most frequent form of PNH in Mexico is AA-PNH.

Patients and Methods: Six consecutive AA-PNH patients with HLA-identical siblings were allografted in two institutions in Mexico, employing a reduced-intensity conditioning regimen for stem cell transplantation (RIST) conducted on an outpatient basis.

Results: Median age of the patients was 37 years (range 25–48). The patients were given a median of \(5.4 \times 10^6 \) kg allogeneic CD34(+) cells, using 1–3 apheresis procedures. Median time to achieve above \(0.5 \times 10^9\) granulocytes was 21 days, whereas median time to achieve above \(20 \times 10^9\) platelets was 17 days. Five patients are alive for 330–3150 days (median 1437) after the allograft. The 3150-day overall survival is 83.3%, whereas median survival has not been reached, being above 3150 days.

Conclusion: We have shown that hypoplastic PNH patients can be allografted safely using RIST and that the long-term results are adequate, the cost–benefit ratio of this treatment being reasonable. Additional studies are needed to confirm the usefulness of RIST in the treatment of AA-PNH.

Ibrutinib Treatment of Relapsed CLL Following Allogeneic Transplantation: Sustained Disease Response and Promising Donor Immune Modulation

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Figure 1. (A) Percent reduction in LN size, reported as sum of the product of LN diameters (SPD), for 4 patients following ibrutinib initiation. (B) CLL MRD (as percent of WBCs) and blood donor CD3 chimerism for SPN3975 and (C) SPN3431. (D) B cells (excluding the CLL clone) as percent of total PBMC for SPN3975. (E) Total IgH molecules and unique IgH clone counts for SPN3975 at time points (D–day) post allo-HCT.