Alternative Donor Bone Marrow Transplantation with Post-Transplant Cyclophosphamide as Initial Therapy for Acquired Severe Aplastic Anemia

A Sidney Kimmel Cancer Center study from Johns Hopkins University

Study Details:

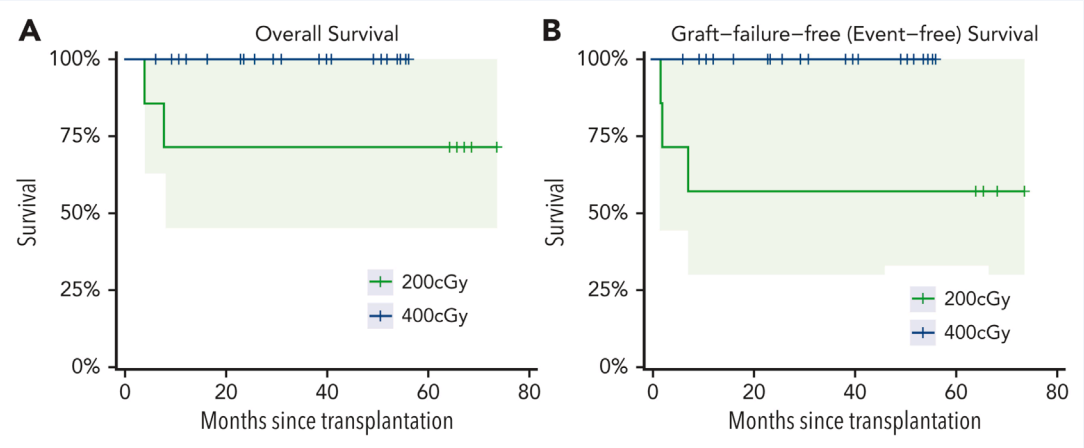
This research supports the use of bone marrow transplantation (BMT) using a haploidentical (haplo) donor and post-transplant cyclophosphamide (PTCy) for graft-versus-host disease (GVHD) prevention as a primary treatment for severe aplastic anemia (SAA). Instead of relying on it only after the failure of immunosuppressive therapy (IST), this method is positioned to enhance treatment access and outcomes.

Conducted from August 2016 to July 2020, the study primarily focused on related haplo donors, with 27 patients (20 initial and 7 post-trial) monitored for approximately 41 months.

Results at a Glance:

- 92% of the 27 patients (52% male, median age 25) demonstrated overall survival at 1, 2 and 3 years, surpassing earlier data.
- Modification in the total body irradiation dosage led to an 88.9% sustained engraftment rate.
- Acute and chronic GVHD occurrence was notably under 10%.
- A significant 96% achieved neutrophil recovery by day 28, and commendable platelet and red blood cell recoveries were observed.
- Two patients grappled with viral infections causing graft losses, yet all remaining patients achieved complete remission by the final follow-up.

Figure: Overall Survival and Graft-Failure-Free Survival for Haplo HCT Patients with SAA



Clinical Impact:

Highlighting the potential of haplo BMT with PTCy GVHD prevention, this study underscores a pivotal shift in SAA therapeutic strategies. Prioritizing HCT over the traditional IST, especially given its limited long-term efficacy, can revolutionize treatment for diverse patient groups lacking a matched sibling donor. This work emphasizes the need for timely HCT consultations and referrals, paving the way for broader treatment access and improved outcomes for SAA.

Read the publication in *Transplantation and Cellular Therapy* (DOI: 10.1182/blood.2023020435).

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