Optimizing Outcomes for Matched Unrelated Allogeneic HCT by Improving Donor Selection Among Younger Donor Options

Blood and Marrow Transplant Clinical Trials Network (BMT CTN) 1507

Study Details:

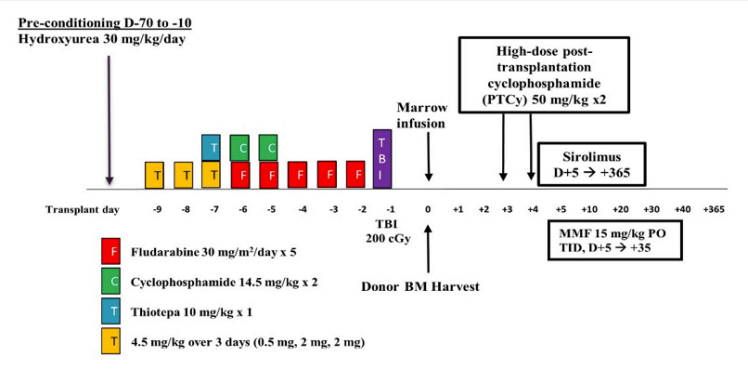
This multi-center, single-arm, phase-II, prospective clinical trial focused on evaluating the efficacy of reduced intensity haploidentical bone marrow transplant (haploBMT) with post-transplant cyclophosphamide (PTCy) for curative treatment in adults with severe sickle cell disease (SCD). The study aimed to explore an alternative curative therapy for SCD patients lacking a matched sibling donor and who are ineligible for myeloablative conditioning due to associated toxicity.

Out of 54 participants enrolled, 42 proceeded to transplant. The majority were male and Black.

Results at a Glance:

- Estimated 2-year event-free survival (EFS) post-transplant was 88%, and 2-year overall survival (OS) was 95%.
- Most qualifying events for EFS occurred within the first 12 months post-transplant.
- The cumulative incidence of acute graft-versus-host disease (GVHD) by day 100 was 26.2% for grades II-IV and only 4.8% for grades III-IV.
- A significant number of participants required re-admission post-BMT, mostly due to infections; however, this trial was conducted during the COVID-19 pandemic.

Figure: Common Conditioning Regimen for HaploBMT



Clinical Impact:

The findings from this study indicate that haploBMT with PTCy is a viable and relatively safe curative option for adults with severe SCD, showing promising event-free and overall survival. This approach not only expands the donor pool beyond matched sibling donors but also offers a less toxic alternative to traditional myeloablative conditioning. The success of haploBMT in this setting could shift the treatment landscape for SCD. As such, it is imperative for healthcare providers to be informed about this therapeutic option, and for continued research and funding to support access to and improvement of this potentially life-saving treatment.

Scan to review more recent clinical research

Read the American Society of Hematology (ASH) Annual Meeting and Exposition abstract published in *Blood* (DOI: 10.1182/blood-2023-192022).

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