Haploidentical Transplant as Initial Therapy to Treat Severe Aplastic Anemia

WHAT?

A clinical trial using haploidentical (half-matched) bone marrow transplant (BMT) as a first treatment for patients with severe aplastic anemia (SAA).

WHY?

- SAA is a life-threatening condition with a high mortality rate. While immunosuppressive therapy (IST) is traditionally the first treatment, its long-term success rate is suboptimal.
- BMT offers a potential cure for SAA yet it's often reserved as a secondary option after IST stops working, which may complicate or limit its success.
- This study challenges the traditional treatment order, using haploidentical BMT as a primary therapy for SAA.

WHEN?

- August 2016 – July 2020

WHO?

- 27 patients with SAA who received BMT - 20 from the trial and 7 treated per internal standards.
- Median age of the patients at the time of joining the trial was 25 years, ranging between 3 to 63.
- Donors were haploidentical family members.

RESULTS

- 1-year engraftment rate: 89%..
- Overall survival for the 27 patients was 92% at 1, 2 and 3 years.
- Better outcomes were seen after increasing the total body irradiation (TBI) dose.
- 67% of patients experienced post-BMT infections.
- Low rates of both acute and chronic graft-versus-host disease (GVHD).

Read the First line alternative donor alloHCT for SAA study results in Blood: doi: 10.1182/blood.2023020435

IMPACT

- BMT with a haploidentical donor as initial treatment for SAA is an effective treatment option with promising outcomes and broadened access.
- Potential shift in treatment practices and opportunities for further research.
- This research is pivotal for enhancing outcomes for diverse patient groups, suggesting a need for more early BMT referrals.
- The upcoming BMT CTN 2207 CUREAA clinical trial will explore alternative donors for BMT for patients with SAA.

FROM THE EXPERTS

The therapeutic decision-making for a new diagnosis of severe aplastic anemia is multifaceted— dependent upon patient preferences, clinical situation, physician input and donor availability.

It is critical that we have the option to offer any and all patients the best available therapy from diagnosis forward.

Our study illustrates a contemporary BMT approach such that any fit aplastic patient, regardless of age and donor status, can now be considered a BMT candidate and offered this potential path to cure.
This protocol with low rates of GVHD and transplant-related morbidity is the focus of the upcoming BMT CTN 2207 CUREAA clinical trial.

The therapeutic paradigm for upfront management of aplastic anemia is truly shifting.

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