Allogeneic Blood and Marrow Transplant Improves Outcomes in Myelodysplastic Syndromes for Patients with High-Risk Genetic Mutations

WHAT?

A clinical trial evaluating the impact of allogeneic (cells from a donor) blood or marrow transplant (BMT) or best supportive care for older patients (50-75 years old) with intermediate to high-risk myelodysplastic syndromes (MDS) demonstrated a benefit of BMT but had not considered the impact of genetic mutations on outcomes.

WHY?

- Allogeneic BMT is a potential cure for MDS. However, its effectiveness is uncertain in patients with MDS with specific genetic mutations, including TP53.
- The study aimed to determine if BMT benefits patients with high-risk MDS, even if they have genetic mutations.

WHEN?

- Patients enrolled in the original MDS study from January 2014 to November 2018.

WHO?

- This study included a total of 309 patients from the original study with intermediate to high risk MDS. The donor arm (access to a donor for BMT) included 229 patients while the no donor arm (no access to a donor for BMT) included 80 patients.
- The study focused on patients with MDS with various genetic mutations, with TP53 being a key focus.

RESULTS

- BMT was linked to a 2-fold lower risk of death when compared to non-BMT treatment, regardless of TP53 and other mutations.
- Patients with MDS with TP53 mutations had significantly worse overall survival (OS) compared to patients whose MDS did not have a TP53 mutation.
- Patients with MDS with TP53 mutations who received BMT demonstrated improved OS compared to those with the mutations who did not receive BMT. This highlights the potential benefit of BMT in this subgroup.

Read the BMT CTN 1102 analysis results in the Journal of Clinical Oncology: doi: 10.1200/jco.23.00866

IMPACT

- The study provides crucial evidence that allogeneic BMT is a vital treatment strategy for high-risk MDS patients, including those with MDS with TP53 mutations.
- It emphasizes the need to consider genetic mutations when evaluating treatment options for MDS.
- The findings offer hope and potentially curative options for older adults facing challenging MDS prognoses, marking a significant advancement in MDS treatment.

FROM THE EXPERTS

"Building on the pivotal BMT CTN 1102 study, we conducted a genetic analysis of patients enrolled on the trial using blood samples from the BMT CTN and NMDP biorepositories. Our goal was to determine whether the benefit of allogeneic transplantation extends across the biological spectrum of MDS. We found that transplantation can be a curative treatment even for patients with the highest risk genetic sub-groups, including those with TP53 mutated MDS. Our results indicate that no patients should be excluded from transplant consideration on the basis of MDS genetic characteristics."

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Versluis J, Saber W, Tsai HK, et al. Allogeneic Hematopoietic Cell Transplantation Improves Outcome in Myelodysplastic Syndrome Across High-Risk Genetic Subgroups: Genetic Analysis of the Blood and Marrow Transplant Clinical Trials Network 1102 Study. J Clin Oncol. 2023;41(28):4497-4510. doi: 10.1200/JCO.23.00866.