

General AML

## Treosulfan-based conditioning regimen improves the long-term survival of AML patients receiving allogeneic HCT

 Sara Valente | Jul 27, 2017

Allogeneic Hematopoietic Cell Transplantation (allo-HCT) is a successful treatment used for patients with Acute Myeloid Leukemia (AML) but high relapse rates and toxicity have hindered long-term survival. Conditioning regimens are a method of improving allo-HCT outcomes for AML patients by providing immunosuppressant and antileukemic effects whilst maintaining a low toxicity profile.

In an article published in *Cancer* on 15<sup>th</sup> July 2017, [Arnon Nagler](#) from the [Chaim Sheba Medical Center](#), Israel, and colleagues reported results from their study which assessed the safety and efficacy of treosulfan-based conditioning regimen in Acute Myeloid Leukemia (AML) patients who had undergone allo-HCT. Treosulfan is an alkylating agent related to busulfan.

In this retrospective study, 520 AML (median age = 57 years) patients receiving allo-HCT and treosulfan-based conditioning between 2000–2012 reported to the [European Society for Blood and Marrow Transplantation \(EBMT\)](#) Acute Leukemia Working Party registry were analyzed.

### The key results of the analysis were:

- At the median follow-up of 61 months
  - 5-year Overall Survival (OS) = 38%
  - 5- year Leukemia Free Survival (LFS) = 33%
  - 5-year Relapse incidence = 42%
  - 5-year Non Relapse Mortality (NRM) rates = 25%
- 11 patients (2%) developed veno-occlusive disease with death occurring in 2

The registry analysis showed that long-term survival was improved for AML patients treated with treosulfan-based conditioning regimens. Additionally, this regimen also demonstrated a “favourable NRM and very low risk of veno-occlusive disease” in AML.

The authors suggested that prospective studies are required in order to optimize treosulfan-based conditioning regimens in AML patients.

### Abstract

**BACKGROUND:** Allogeneic hematopoietic cell transplantation (HCT) is a curative therapy for patients with acute myeloid leukemia (AML). However, post-HCT relapse and regimen-related toxicity remain significant barriers to long-term survival. In recent years, new conditioning regimens have been explored to improve transplantation outcomes in patients with AML. Treosulfan combines a potent immunosuppressive and antileukemic effect with a low toxicity profile. **METHODS:** To investigate the role of treosulfan-based conditioning, the European Society for Blood and Marrow Transplantation Acute Leukemia Working Party performed a registry analysis of 520 adult patients with AML who received treosulfan-based conditioning and underwent HCT between 2000 and 2012, including 225 patients in first complete remission, 107 in second or later complete remission, and 188 with active/advanced disease 188 (88 with primary refractory disease). The median patient age was 57 years (range, 20-73 years). Donors were human leukocyte antigen-identical siblings (n 5 187), unrelated donors (n 5 235), or mismatched related donors (n 5 98). Conditioning regimens included treosulfan (42 g/m<sup>2</sup> [n 5 396], 36 g/m<sup>2</sup> [n 5 109], or 30 g/ m<sup>2</sup> [n 5 15]) with fludarabine or alkylating agents followed by infusion of hematopoietic stem cells (bone marrow, n 5 52; peripheral blood, n 5 468). **RESULTS:** At a median follow-up of 61 months, the 5-year overall survival, leukemia-free survival, relapse incidence, and nonrelapse mortality rates were 38%, 33%, 42%, and 25%, respectively. The incidence of grade II-IV acute and chronic graft-versus-host disease was 24% (grade III-V, 11%) and 38%, respectively. Only 11 patients (2%) developed veno-occlusive disease, with two deaths (0.4%) from veno-occlusive disease. **CONCLUSIONS:** Treosulfan-based conditioning regimens provide an acceptable long-term survival with favorable nonrelapse mortality and a very low risk of veno-occlusive disease. Further studies are needed to optimize the treosulfan-based conditioning regimen for patients with AML.

## References

1. [Nagler A. et al.](#) Long-Term Outcome After a Treosulfan-Based Conditioning Regimen for Patients With Acute Myeloid Leukemia: a Report From the Acute Leukemia Working Party of the European Society for Blood and Marrow Transplantation. *Cancer*. 2017 Jul 15; 123(14): 2671–2679. DOI: [10.1002/cncr.30646](https://doi.org/10.1002/cncr.30646).

---

© 2018 Scientific Education Support Ltd. This PDF is provided for personal use only. For wider or commercial use, please seek permission from [secretariat@scientificeducationsupport.com](mailto:secretariat@scientificeducationsupport.com) and attribute the source as: <<http://www.amlglobalportal.com/medical-information/treosulfan-based-conditioning-regimen-improves-the-long-term-survival-of-aml-patients-receiving-allogeneic-hct>>