



The 65th ASH Annual Meeting Abstracts

ONLINE PUBLICATION ONLY

732.ALLOGENEIC TRANSPLANTATION: DISEASE RESPONSE AND COMPARATIVE TREATMENT STUDIES**Haematopoietic Stem Cell Transplantation for Hepatitis-Associated Aplastic Anaemia: Clinical Characteristics, Outcomes and Risk Factors**Li Jia, PhD¹, Yilin Liu², Erjie Jiang, PhD², Sizhou Feng²

¹ Haematopoietic Stem Cell Transplantation Center, State Key Laboratory of Experimental Hematology, National Clinical Research Center for Blood Diseases, Haihe Laboratory of Cell Ecosystem, Institute of Hematology & Blood Diseases Hospital, Chinese Academy, Tianjin, China

² State Key Laboratory of Experimental Hematology, National Clinical Research Center for Blood Diseases, Haihe Laboratory of Cell Ecosystem, Institute of Hematology & Blood Diseases Hospital, Chinese Academy of Medical Sciences & Peking Union Medical College, Tianjin, China

Introduction:

Hepatitis-associated aplastic anaemia (HAAA) is a severe and life-threatening condition that requires effective treatment approaches. Haematopoietic stem cell transplantation (HSCT) has emerged as a promising therapeutic option for HAAA patients. In this retrospective study, we aimed to investigate the clinical characteristics, outcomes, and prognostic factors associated with HSCT in HAAA patients at a prominent Chinese blood disease hospital between 2008 and 2022.

Methods:

A comprehensive review of 35 HAAA patients who underwent HSCT was conducted, and clinical data were analyzed to determine the treatment's efficacy and safety. The patients were primarily categorized into those with severe AA (28.6%) and very severe AA (65.7%), with a predominant male representation (68.6%) and a median onset age of 23 years (range, 9-44). Both haploidentical and matched sibling donors were utilized in comparable proportions.

Results:

The study revealed a promising 5-year overall survival (OS) rate of 74.0% after HSCT, underscoring the effectiveness of this treatment approach in HAAA patients. However, certain factors significantly impacted survival rates and treatment outcomes. Prolonged intervals from diagnosis to HSCT (≥ 75 days), acute graft-versus-host disease (GVHD), and post-HSCT liver events, such as hepatic GVHD and a three-fold increase in aminotransferase or bilirubin, were found to worsen 5-year OS. Interestingly, higher doses of mononuclear cell count, CD34+, CD3+, CD4+, and CD8+ cells in allografts were associated with poorer OS or graft failure-free survival (GFFS), suggesting that pursuit of higher cell counts in certain cases may be counterproductive. Furthermore, a close examination of the multivariate models revealed that recipients who received sex-matched grafts experienced better OS, while those with younger male donors had a lower incidence of grade II-IV acute GVHD. Moreover, a higher HLA matching degree (HLA $\geq 7/10$) between the recipient and donor emerged as an independent prognostic factor associated with better OS and GFFS.

Conclusion:

This study highlights the safety and efficacy of HSCT as a treatment modality for HAAA patients. Early transplantation, careful donor selection, and efforts to improve post-transplant liver events were identified as crucial factors to optimize treatment outcomes. While the use of certain cells in higher doses may have a negative impact on survival, the importance of achieving a high HLA matching degree cannot be overstated. Moving forward, these findings can serve as valuable insights in guiding the management and treatment decisions for HAAA patients undergoing HSCT. Further research and continuous improvement in transplantation protocols will undoubtedly advance the success rates and long-term prognosis for individuals afflicted with this challenging condition.

Disclosures No relevant conflicts of interest to declare.

<https://doi.org/10.1182/blood-2023-188427>