



EBMT 2018 | A new endpoint that accurately recognizes the long-term outcomes of allo-HCT in refractory GvHD

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Graft-versus-host disease-free survival (GRFS) has been a key measure to estimate transplant outcomes, hence it characterizes disease-free survival as well as survival without major morbidity related to GvHD. GRFS defines GvHD as a permanent therapy failure event, despite of the fact that GvHD might response to treatment. As a result, GRFS may overestimates the effect of GvHD on the outcome of allo-HCT.

This study aimed to identify a new endpoint that accurately recognizes the long-term outcomes of allo-HCT including life expectancy, disease remission, and quality of life (QOL).

Methods:

- QOL was compared after allo-HCT according to the status of chronic GvHD using a nationwide cross-sectional questionnaire study
- Two new composite endpoints were implemented, current GRFS (cGRFS) and refractory GRFS (rGRFS) with the use of a single-center cohort (n = 315)
- cGRFS was defined as survival without disease relapse/progression or active chronic GVHD at a given time after allo-HCT

Key findings:

- Only active cGVHD had an inferior impact on QOL after allo-HCT
- The two cGRFS curves obtained using two altered methods were overlaid and both showed better outcome to conventional GRFS
- The curves of rGRFS and cGRFS were superimposed

This study showed that cGRFS and rGRFS more accurately reflect long-term transplant outcomes than standard GRFS. "Particularly, rGRFS can be easily calculated and analyzed with widely-used statistical approaches including proportional hazard modeling. Therefore, we propose rGRFS as a more effective and simple endpoint for assessing the long-term transplant success."

References

1. Kawamura K. *et al.* Refractory graft-versus-host disease-free, relapse-free survival: proposal of a simple and accurate endpoint to evaluate the long-term success of allogeneic hematopoietic stem cell transplantation. *os12-6*. 2018 March 21. 44th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT).

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