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ORIGINAL ARTICLE

Decision analysis of allogeneic bone marrow transplantation versus immunosuppressive therapy for young adult patients with aplastic anemia

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Abstract

Background Allogeneic bone marrow transplantation (BMT) from an HLA-matched sibling donor is recommended as an initial treatment for young patients. However, immunosuppressive therapy (IST) with cyclosporine and anti-thymocyte globulin may be a viable option even when an HLA-identical sibling donor is available.

Methods We constructed a Markov model to simulate the 10-year clinical course of patients aged 21–40 years with newly diagnosed severe aplastic anemia. Immediate BMT and IST were compared as an initial treatment assuming the availability of an HLA-identical sibling donor. Transition probabilities after treatment were determined based on a registry data analysis for BMT and a long-term prospective study for IST.

Results Quality-adjusted life years (QALYs) after treatment selection were 6.77 for BMT and 6.74 for IST. One-way sensitivity analysis revealed that the utility for being alive without GVHD after BMT, that for being alive with partial response after IST, and the response rate after initial IST strongly affected the results.

Conclusions BMT and IST produced similar QALY for young patients with severe aplastic anemia. An estimation of the response rate to the initial IST may enable an individualized comparison between BMT and IST.

Keywords Severe aplastic anemia · Bone marrow transplantation · Immunosuppressive therapy · Decision analysis

Introduction

Allogeneic bone marrow transplantation (BMT) from an HLA-matched sibling donor is an established treatment for severe aplastic anemia and is recommended as an initial treatment for young patients aged less than 30 or 40 years in several guidelines [1–3]. The incidence of transplant-related mortality is lower than that in allogeneic BMT for leukemia, partly due to the use of non-myeloablative or reduced-intensity conditioning regimens in young patients. However, in addition to the non-negligible incidence of transplant-related mortality, the long-term quality of life (QOL) may

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