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Stem Cell Transplantation for Treatment of Primary Immunodeficiency Disorders

Susanna M. Müller Wilhelm Friedrich

Abstract:

Primary Immunodeficiencies constitute a group of highly complex congenital disorders most of which are characterized by a very poor prognosis. Allogeneic hematopoietic stem cell transplantation (HSCT) has become an established curative treatment approach in many of these disorders, which may be permanently corrected. In this presentation basic and practical aspects of HSCT are presented, with an emphasis on its application in lymphocyte disorders such as severe combined immunodeficiency (SCID). Optimal results and outcome of HSCT are highly dependant on early and correct diagnosis of these rare disorders, and HSCT should usually be applied early in the course of the disease in order to prevent irreversible complications from infections. Clinical results will be summarized based on recent analysis performed in large patient cohorts, which have shown steady improvements and have led to a marked change in the prognosis of patients with primary immunodeficiencies.

Keywords:

Primary Immunodeficiency Disorders . Severe Combined Immunodeficiency . Stem Cell Transplantation

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