Challenge TDT

Allogeneic Hematopoietic Stem Cell Transplant (HSCT)

A potentially curative option that is primarily being used in younger patients with HLA-matched donors[1,2,3]

KEY POINTS

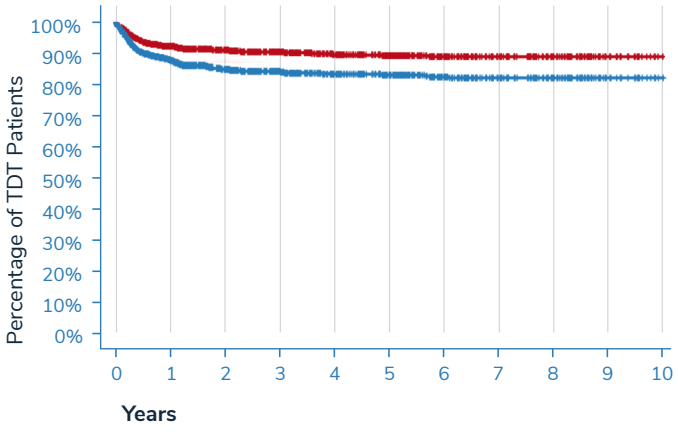
Allogeneic hematopoietic stem cell transplant (HSCT) is an available treatment option that potentially corrects the genetic deficiency in transfusion-dependent beta-thalassemia (TDT).[1,2,3]

In a retrospective non-interventional study of 1493 patients with thalassemia major who underwent allogeneic HSCT (between 2000 and 2010), the estimated 2-year overall survival and thalassemia-free survival were 88% and 81%, respectively.[4]

HSCT is generally performed in younger patients with HLA-matched donors.[1,2,3,4]

Many patients with TDT do not receive allogeneic HSCT due to increased risk of mortality stemming from lack of a suitable donor, existing complications, and/or age.[1,3,4,5]

Survival Rates for 1493 HSCT Recipients With Thalassemia During the Period 2000-2010[4]



	Patients	Events	*Probability**
A. Overall Survival (2 years)	1493	154	0.88±0.01
B. Thalassemia-Free Survival (2 years)	1493	253	0.81±0.01

Survival rates for 1493 transplant recipients in the period 2000-2010.
Overall Survival (A) and Thalassemia-Free Survival (B) are shown.
**Error estimates reflect accuracy of predictions to within 1 percentage point.*

Adapted from Baronciani D, Angelucci E, Potschger U, et al. Hematopoietic stem cell transplantation in thalassemia: a report from the European Society for Blood and Bone Marrow Transplantation Hemoglobinopathy Registry, 2000–2010. *Bone Marrow Transplant.* 2016;51(4):536-541.

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