Challenge TDT

Transfusion-Dependent Beta-Thalassemia (TDT)

Significantly Impacts Quality of Life of Patients and Caregivers[1,2]

KEY POINTS

Patient and caregiver quality of life are significantly affected by ongoing transfusions, iron chelation therapy, and disease complications.[1,2]

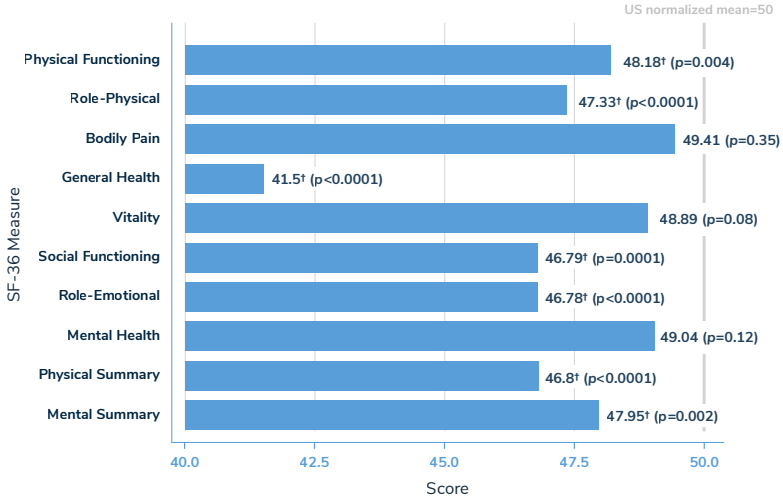
The life-long need for transfusion and chelation places a significant burden on patients' quality of life. The process of receiving a transfusion can take up a patient's entire day, and requires taking time off work or school as well as transport to and from the transfusion center.[3]

Patients who received allogeneic HSCT for thalassemia reported improved quality of life when compared to patients on conventional medical therapy.[4]

30-year overall survival (OS) in conventionally treated patients was similar to transplanted patients; however, transplanted patients reported higher mortality in the first ten years, with nearly 50% of the mortality being driven by acute GvHD.[5]*

*In a study of 516 pediatric and adult patients which looked at survival rates in age/sex-matched patients who received conventional therapy with transfusion and chelation (n=258) vs. those who received allogeneic HSCT (n=258). Two-thirds of the patients who received HSCT had matched related donors and a third had matched unrelated donors.[5]

Health-Related Quality of Life Scores (SF-36*) in the Thalassemia Longitudinal Cohort vs. US Norms[1]



Adapted from Sobota A, Yamashita R, Xu Y, et al. Quality of Life in Thalassemia: A Comparison of SF-36 Results from the Thalassemia Longitudinal Cohort to Reported Literature and the US Norms. *Am J Hematol.* 2011;86(1):92-95.

*These health domains are evaluated as part of the Medical Outcomes Study Short Form 36-Item (SF-36). They are defined as follows: Physical functioning covers limitations in daily life due to health problems. The role-physical scale measures role limitations due to physical health problems. The bodily pain scale assesses the frequency of pain and interference of pain with usual roles. The general health scale measures individual perceptions of general health. The vitality scale assesses energy levels and fatigue. The social functioning scale measures the extent to which ill health interferes with social activities. The role-emotional scale assesses role limitations due to emotional problems.[6]

†one sample T test shows significant difference between population and US norm (p<0.05)

REFERENCES:

1. Sobota A, Yamashita R, Xu Y, et al. Quality of Life in Thalassemia: A Comparison of SF-36 Results from the Thalassemia Longitudinal Cohort to Reported Literature and the US Norms. *Am J Hematol.* 2011;86(1):92-95. **2.** Yengil E, Acipayam C, Kokacya MH, et al. Anxiety, depression and quality of life in patients with beta thalassemia major and their caregivers. *Int J Clin Exp Med.* 2014;7(8):2165-2172. **3.** Baer K. A Guide to Living with Thalassemia. Cioffi G, Butler C, eds. New York, NY: Cooley's Anemia Foundation; 2013: http://www.cooleysanemia.org/updates/pdf/GuideToLivingWithThalassemia.pdf. Accessed August 30, 2018. **4.** La Nasa G, Caocci G, Efficace F, et al. Long-term health-related quality of life evaluated more than 20 years after hematopoietic stem cell transplantation for thalassemia. *Blood.* 2013;122(13):2262-2270. **5.** Caocci G, Orofino MG, Vacca A, et al. Long-term survival of beta thalassemia major patients treated with hematopoietic stem cell transplantation compared with survival with conventional treatment. *Am J Hematol.* 2017;92(12):1303-1310. **6.** Busija L, Pausenberger E, Haines T, et al. Adult Measures of General Health and Health-Related Quality of Life Medical Outcomes Study Short Form 36-Item (SF-36) and Short Form 12-Item (SF-12) Health Surveys, Nottingham Health Profile (NHP), Sickness Impact Profile (SIP), Medical Outcomes Study Short Form 6D (SF-6D), Health Utilities Index Mark 3 (HUI3), Quality of Well-Being Scale (QWB), and Assessment of Quality of Life (AQOL). *Arthritis Care Res.* 2011;63(11):S383-412.

bluebirdbio