

SAFETY & EFFICACY OF THALIDOMIDE IN CHILDREN WITH TRANSFUSION DEPENDENT THALASSEMIA: A QUASI RANDOMIZED CONTROLLED TRIAL IN A TERTIARY CARE HOSPITAL IN BANGLADESH

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Background: Children with Transfusion-Dependent Thalassemia (TDT) face a significant health burden. Though regular blood transfusions are life-saving, they carry risks of infection and iron overload. Thalidomide shows promise in increasing fetal hemoglobin (HbF) levels and reducing transfusion needs in adults with TDT. However, its safety and effectiveness in children remain unclear. This study aimed to investigate the safety and efficacy of thalidomide in children with TDT through a quasi-randomized controlled trial. **Methods:** In this single-center, quasi-randomized, single-blind clinical trial, 60 patients of 3-18 years were quasi-randomly assigned to receive thalidomide or placebo for 12 weeks. The primary endpoint was the change in hemoglobin (Hb) levels in the patients. The secondary endpoints included the change in frequency of red blood cell (RBC) transfusion and adverse effects. **Results:** After 12 weeks of treatment Hb concentrations in patients treated with thalidomide significantly increased from a baseline mean of 6.5±0.9 g/dL to 8.2±0.9 g/dL with an average increase of 1.7±0.8 g/dL. HbF significantly increased (7.3%) in thalidomide-treated cases in comparison to placebo group (P<0.001). Within the 12 weeks, the mean RBC transfusion frequency for patients treated with thalidomide and placebo was 0.73 ± 0.9 times and 2.9 ± 0.6 times, respectively (P<0.001). The overall response rate in thalidomide-treated children was 86.7% (P<0.001). Mild adverse events including drowsiness, dizziness, pyrexia, pruritus, abdominal pain, nausea, constipation, and facial edema were more frequently (60%) found in patients treated with thalidomide. No unfavorable effects were observed on kidney and liver functions. **Conclusion:** This study demonstrates that thalidomide is a safe and effective treatment option for children with TDT. It significantly improved Hb levels and reduced the need for blood transfusions. This finding offers a promising alternative option for managing TDT in children, reducing long-term complications associated with frequent transfusions.

Keywords: Thalidomide, Transfusion Dependent Thalassemia, Children.

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