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abstract

A Prospective, Observational Study Examining the Safety and Effectiveness of Thalidomide and Hydroxyurea in Patients with Transfusion-Dependent β-Thalassemia

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A Prospective, Observational Study Examining the Safety and Effectiveness of Thalidomide and Hydroxyurea in Patients with Transfusion-Dependent β-Thalassemia

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Introduction: β-thalassemia (BTM) is one of the most prevalent inherited disorders globally, with approximately 65% of thalassemia patients relying on blood transfusions. Treatment options for BTM include hematopoietic stem cell transplantation (HSCT), iron chelation therapy, emerging gene therapies, and methods to promote erythroid maturation.

Thalidomide, an immunomodulator, has been recognized for its ability to induce fetal hemoglobin (HbF) and is increasingly used in managing BTM, leading to significant reductions in transfusion needs for affected patients. However, thalidomide is associated with various adverse effects affecting gastrointestinal, dermatological, hematological, and immunological systems. There is limited information regarding its toxicity patterns on both hematological and non-hematological profiles.

Methodology: This ongoing single-center prospective study is being conducted at The Children's Hospital,Karachi (CHK), a 50-bed facility specializing in pediatric hematology-oncology care. Participants aged 2 years and older of either sex diagnosed with BTM who have not undergone HSCT due to high costs, lack of a matched donor, or elevated transplant-related mortality risk were included. Exclusion Criteria: Patients with BTM who have underlying comorbidities affecting hepatic, renal, cardiac, or neurological functions was excluded from the study.

Results: The mean age of participants was 8.16 years with a standard deviation of 5.9 years, emphasizing the young demographic under investigation. Gender distribution showed a male predominance, with 21 patients (67.7%) being male and 10 patients (32.3%) female. With regards to efficacy, clinical response to treatment was overwhelmingly positive, with 26 patients (83.9%) categorized as good responders. However, 2 patients (6.5%) showed no response, and an additional 2 patients (6.5%) were classified as partial responders, reflecting a varied spectrum of therapeutic outcomes. Whereas in terms of safety, side effects were minimal, with 29 patients (93.5%) experiencing no adverse reactions.

However, 1 patient (3.2%) reported fever and chest congestion. Another 1 patient (3.2%) displayed a yellowish appearance at the third-month follow-up and later presented with shortness of breath on climbing stairs by the sixth month and the toxicities were measured as per CTCAE 2005 criteria. In this study, we assessed the outcomes of combining hydroxyurea (HU) and thalidomide therapy. Our findings indicated a significant increase in median hemoglobin (Hb) levels and a reduction in transfusion needs among the participants.

Additionally, we observed a notable decrease in liver and spleen size, as well as serum ferritin levels. The response was found to be independent of any genetic mutations. A multicenter study conducted by Yang et al. examined the effects of thalidomide and reported an average increase of 2.9 g/dL in Hb from baseline in non-transfusion-dependent thalassemia patients aged 14 to 65 years. For transfusion-dependent thalassemia patients, there was not only an improvement in Hb levels but also a significant reduction in the frequency of transfusions.

Conclusion: In conclusion, thalidomide and hydroxyurea demonstrates effectiveness as a treatment option
for patients with β-thalassemia, leading to significant increases in hemoglobin levels and a decrease in the need for transfusions.

However, the results of this study may be affected by the relatively small sample size, suggesting that future research should include a larger group of participants.