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Promising Response to Thalidomide in Symptomatic bThalassemia

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Introduction

Over the past decade, few reports suggested that the drug thalidomide (HbF inducer) may be of value in a subset of transfusiondependent and nontransfusion dependent thalassemia patients. A cohort of 37 patients with symptomatic bthalassemia syndrome [14 transfusions dependent thalassemia (TDT), and 23 Nontransfusion dependent Thalassemia (NTDT)], who were unable to pursue conventional therapy with transfusion and chelation, were recruited over 3 years in a center in Iraqi Kurdistan. After taking informed consent, patients were put on low dose Thalidomide (2–10 mg/kg), with regular follow up after that for a minimum of 8 months for a response. Patients with TDT were considered responders if their yearly transfusion requirement dropped by 25% or more, while NTDT responders were those who had a hemoglobin raise of 1 g m/dL or more. The median age of enrolled patients was 10 years (range 3–43) and included 21 males and 16 females. After a mean of 1.7 months (SD 0.76), responses were documented in 28 patients (75.7%). Among NTDT patients, a significant increase in hemoglobin from a mean of 7.83 (SD 1.07) to 9.96 g/dL (SD 1.11 gm/dL) was documented. While among TDT patients, there was a significant drop in yearly transfusions from 27 (SD 17.7) to 7.79 (SD 7.5) blood unit per year. The response in both categories was sustained after a median follow up of 15 months (8–36 m). Only minimal side effects were documented throughout in the form of constipation and only one patient developed extramedullary hemopoietic abdominal masses. A significant response to thalidomide was documented in the majority of TDT and NTDT patients, a response which was obtained after a mean of 1.7 months, and the response was sustained with limited side effects. The results support a possible role for this medication in a subset of thalassemia patients.

Keywords: Hemopoietic, Thalassemia, Thalidomide

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