

Therapeutic Methods, Treatment of Genetic Diseases

AB023. Evaluation the outcome of β -thalassemia intermedia patients on hydroxyurea combined with erythropoietin at National Children's Hospital

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Background: Inherited hemoglobin disorders primarily exist in the low- or middle-income countries of the tropical belt stretching from Southeast Asia like Vietnam. β -thalassemia intermedia is caused by one of the more severe combined with a milder thalassemic gene. Children with thalassemia intermedia start to develop symptoms later in life than those with thalassemia major, usually becoming pale and developing symptoms around 2 years of age. Although β -thalassemia intermedia is in non-transfusion dependent, but some patients may require more frequent transfusions because of poor growth and development. The aim of this study was to evaluate the effective treatment of hydroxyurea (HU) and erythropoietin in β -thalassemia intermedia patients.

Methods: The patients with β -thalassemia intermedia were prospectively treated with HU plus erythropoietin, during October 2011–September 2014 at NCH. Oral dosage of HU was 15–20 mg/kg/24h and IV dosage of erythropoietin was 300 UI/kg/48h. Characteristics of moderate

β -thalassemia base on Mahidol score for hemoglobin E/ β -thalassemia severity classification: severity score 4–7. Full blood count, reticulocyte, HbF level, renal and liver function tests were done before and after treatment a week and monthly. Blood transfusion when Hb <70 g/L. Statistical analysis was performed on SPSS program.

Results: Seventeen (56.7%) patients were well responsive, transfusion-independent with Hb level 85 g/L after a year of treatment. Hb levels reached the highest point at 6 months (1.9 ± 0.6), similar to highest HbF percentage after 6 months from beginning (22.7 ± 14.3). A 43.3% of patients were partly responsive with slightly increased Hb levels but still required blood transfusion from 5.2 ± 1.2 times/year to 3.4 ± 1.3 times/year ($P > 0.05$). The most common side effects were headache (23.3%), alopecia (20%), abdominal pain (10%) and nausea and vomiting (3.3%). One patient had liver dysfunction (3.3%) with slightly elevate AST, ALT which spontaneously resolved after 2 months.

Conclusions: There are 56.7% β -thalassemia intermedia patients who were well responsive to the combined treatment of HU and erythropoietin. Side effects occurred but most of patients are well tolerated.

Keywords: β -thalassemia intermedia; treatment; hydroxyurea (HU); erythropoietin

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