



Iron chelation Therapy in β -Thalassemia Major Showing Non-Inferiority of Oral Chelator over Parenteral Chelator

Shahid SHAH ^{1*}, Zunera CHAUDHARY ², Mahmood-ur RAHMAN ³, Malik SAADULLAH ⁴, Ayesha ASLAM ⁵, Ghulam ABBAS ⁶, Ayesha KAZMI ⁷, Muhammad Abid SHAH ⁸, Sana TAHIR ¹ & Komal NAYAB ¹

¹ Department of Pharmacy Practice, ² Department of Pharmacology, Faculty of Pharmaceutical Sciences, Government College University Faisalabad, Pakistan

³ Department of Biotechnology, Government College University Faisalabad, Pakistan

⁴ Department of Pharmaceutical Chemistry, Faculty of Pharmaceutical Sciences, Government College University Faisalabad, Pakistan

⁵ Department of Neurology, King Edward Medical University Lahore, Pakistan

⁶ Department of Pharmaceutics, Faculty of Pharmaceutical Sciences, Government College University Faisalabad, Pakistan

⁷ Children Hospital and the Institute of Child Health Multan, Pakistan

⁸ Pediatric Intensive Care Unit, Agha Khan Hospital Karachi, Pakistan

SUMMARY. In patients with β -thalassemia major (BTM), the incidence of iron overload due to multiple blood transfusions necessitate the use of iron chelators like desferrioxamine (DFO) or deferasirox (DFX). The primary objective of this study was to compare the effectiveness of DFO and DFX in BTM patients and secondary objective was to analyze the relationship of some factors with iron chelation therapy (ICT). A total of 142 BTM children, receiving ICT, were recruited in this study through non-probability consecutive sampling technique. Patients in group A (n = 71) and group B (n = 71) received DFO 50 mg/kg through subcutaneous route by infusion pump and DFX 30 mg/kg orally, respectively. Stratification was done with respect to age (years), gender, height (cm), weight (kg), number of blood transfusion and serum ferritin (SF) levels ($\mu\text{g/L}$). Statistical analysis was done by Independent Sample t-test and ANOVA using SPSS-20 (p-value less than 0.05 considered statistically significant). DFO reduced SF levels to $22.43 \pm 11.38\%$ in group A while DFX reduced to $23.41 \pm 14.5\%$ in group B. Mean differential SF levels in terms of age (2-8 year = 798.73 vs 9-16 year = 1366.71, $p = 0.002$), weight (1-30 kg = 801.33 vs 31-60 kg = 1376.50, $p = 0.002$) and number of blood transfusions (1-15 times = 787.28 vs 16-30 times = 1274.17, $p = 0.002$) were found to be significantly associated with ICT. This study suggests that DFX was as effective as DFO in improving SF levels in BTM patients. Thus, ICT with oral DFX is preferred over subcutaneous DFO due to patient compliance due to patient compliance.

RESUMEN. En pacientes con β -talasemia mayor (BTM) la incidencia de sobrecarga de hierro debido a múltiples transfusiones de sangre requiere el uso de quelantes de hierro como deferoxamina (DFO) o deferasirox (DFX). El objetivo principal de este estudio fue comparar la efectividad de DFO y DFX en pacientes con BTM y el objetivo secundario fue analizar la relación de algunos factores con la terapia de quelación del hierro (TIC). Un total de 142 niños BTM que recibieron TIC fueron reclutados en este estudio mediante la técnica de muestreo consecutivo no probabilístico. Los pacientes del grupo A (n = 71) y del grupo B (n = 71) recibieron 50 mg/kg de DFO por vía subcutánea mediante bomba de infusión y 30 mg/kg de DFX por vía oral, respectivamente. La estratificación se realizó con respecto a la edad (años), sexo, altura (cm), peso (kg), número de transfusiones de sangre y niveles de ferritina sérica (SF) ($\mu\text{g/L}$). El análisis estadístico se realizó mediante la prueba t de muestra independiente y ANOVA utilizando SPSS-20 (valor de p menor que 0,05 considerado estadísticamente significativo). DFO redujo los niveles de SF a $22,43 \pm 11,38\%$ en el grupo A mientras que DFX se redujo a $23,41 \pm 14,5\%$ en el grupo B. Niveles diferenciales medios de SF en términos de edad (2-8 años = 798,73 vs 9-16 años = 1366,71, $p = 0,002$), el peso (1-30 kg = 801,33 vs 31-60 kg = 1376,50, $p = 0,002$) y el número de transfusiones de sangre (1-15 veces = 787,28 vs 16-30 veces = 1274,17, $p = 0,002$) resultaron ser significativamente asociadas con las TIC. Este estudio sugiere que DFX fue tan eficaz como DFO para mejorar los niveles de SF en pacientes con BTM. Por lo tanto, se prefiere la TIC con DFX oral a la DFO subcutánea debido al cumplimiento del paciente debido al cumplimiento del paciente.

KEY WORDS: deferasirox, desferrioxamine, iron chelation therapy, serum ferritin level, β -thalassemia major.

* Author to whom correspondence should be addressed. E-mail: Shahid.waris555@gmail.com