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Quality of life, safety, and efficacy of iron chelation therapy in β thalassemic patients who switched from deferasirox dispersible tablets to deferasirox film-coated tablets.

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Background.

Iron overload (IOL) due to repetitive blood transfusions has a serious impact on morbidity and mortality in β thalassemia patients. Deferasirox (EXJADE®, Novartis, UK) is an oral iron-chelating agent which requires once a day oral administration. However, some patients show a low adherence to therapy with deferasirox dispersible tablets (DFX-DT) due to this administration mode. Thus, the plasmatic levels of ferritin could be higher than what is expected based on the recommended dose.

We report the results of 1-year observational study that aimed at describing the quality of life, safety and efficacy of iron chelation therapy in β thalassemic patients who switched from DFX-DT to deferasirox film-coated tablets (DFX-FCT), or from other iron chelators to DFX-FCT.

Patients and Methods

The patients were recruited from “Thalassemia and Hemoglobinopathies” Center of University Hospital of Messina (Sicily). Twenty-eight patients with β thalassemia major (BTM) (M=15, F=13), and 2 with β thalassemia intermedia (BTI) (M=1, F=1) were included in the study. Median age was 37.1 years ±7.8 in BTM patients. The 2 BTI subjects were 45 and 54 years old, respectively. BTM patients were diagnosed at a median age of 8.3±2.3 months. The 2 BTI patients were diagnosed at the age of 21 and 19 years old, respectively. All patients were blood transfusion-dependent. The standard of care involved regular follow-up, blood transfusion, and iron chelation therapy.

Twenty-seven patients were subjected to therapy with DFX-DT and 3 had chosen to continue deferoxamine therapy, although DFX-DT was repeatedly recommended. At the end of the study, all patients accepted the switching from DFX-DT to DFX-FCT, included those previously treated with deferoxamine. A Short Form-36 (SF36) questionnaire was used to investigate the quality life (QoL) of the patients before and after the switching from DFX-DT to DFX-FCT, or from deferoxamine to DFX-FCT. Safety and tolerability of the DFX-FCT were evaluated by monitoring of adverse drug reactions (ADRs). Efficacy of DFX-FCT was evaluated by analyzing changes in serum ferritin levels.

Results

At the end of 1 year of therapy with DFX-FCT, all patients had scores of QoL significantly higher than when they were subjected to DFX-DT ($p<0.001$) or deferoxamine ($p<0.001$). A significant decrease in the ferritin levels ($p<0.01$) were found in 23/27 (85.1%) of patients previously treated with DFX-DT as well as in the 3 patients previously treated with deferoxamine ($p<0.01$). Moreover, we reduced the dose of DFX-FCT in 7/28 (25%) BTM patients due to hyper chelation. No significant ADRs were found.

Conclusions

In our preliminary study, DFX-FCT shown higher safety and efficacy compared to the DFX-DT as well as to the deferoxamine. The easy mode of administration implies a better adherence to the therapy and, thus, a reduction of IOL.