

## SAFETY AND EFFICACY OF THE IRON CHELATOR DEFERIPRONE

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Over the last 20 years, the iron chelator deferiprone has been in clinical use initially in trials and since 1999 it has been licenced for general use in patients with beta thalassaemia major. It is given to patients with other transfusion dependent anaemias in special circumstances and usually with permission from appropriate authorities. It has been shown to be effective in maintaining or reducing hepatic iron provided that it is given in adequate dosage. In almost all comparison studies comparing deferiprone with desferrioxamine the results with both ferritin levels and hepatic iron concentration have been similar. It has been clearly demonstrated to be more effective at removing cardiac iron compared to desferrioxamine, improved Left Ventricular Ejection Fraction and also to be more cardioprotective in longitudinal clinical studies. The combination of desferrioxamine and deferiprone has become the gold standard therapy for patients with beta thalassaemia. It rapidly reduces hepatic iron, cardiac iron and significantly improves LVEF. It has been useful in patients with established cardiac failure and in many cases has allowed the complete resolution of cardiac dysfunction. It has also been shown to improve endocrine function especially abnormal glucose metabolism.

The factors responsible for side effects of the iron chelators depend on their route of administration, their potential for removing iron from iron-dependent enzymes, removal of other divalent cations (Zn, Ca), iron redistribution and direct toxicity.

The most common side effects of deferiprone are gastrointestinal upsets and arthropathy. Some patients may have elevated hepatic enzymes initially, but these usually settle after a few months of therapy. Neutropenia occurs in up to 10% of patients and is more common in non-splenectomised patients. The most serious side effect is the idiosyncratic agranulocytosis which occurs in <1%. Other than for agranulocytosis, it is not usually necessary to permanently stop its administration. It does NOT worsen hepatic fibrosis.

Case presentations demonstrating the efficacy, the adverse effects and how they should be managed will be presented and discussed.

