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Response of iron overload to deferasirox in rare transfusion-dependent anaemias: equivalent effects on serum ferritin and labile plasma iron for haemolytic or production anaemias.

Abstract:
It is widely assumed that, at matched transfusional iron-loading rates, responses to chelation therapy are similar, irrespective of the underlying condition. However, data are limited for rare transfusion-dependent anaemias, and it remains to be elucidated if response differs, depending on whether the anaemia has a primary haemolytic or production mechanism. The efficacy and safety of deferasirox (Exjade®) in rare transfusion-dependent anaemias were evaluated over 1 yr, with change in serum ferritin as the primary efficacy endpoint. Initial deferasirox doses were 10-30 mg/kg/d, depending on transfusion requirements; 34 patients had production anaemias, and 23 had haemolytic anaemias. Patients with production anaemias or haemolytic anaemias had comparable transfusional iron-loading rates (0.31 vs. 0.30 mL red blood cells/kg/d), mean deferasirox dosing (19.3 vs. 19.0 mg/kg/d) and baseline median serum ferritin (2926 vs. 2682 ng/mL). Baseline labile plasma iron (LPI) levels correlated significantly with the transfusional iron-loading rates and with serum ferritin levels in both cohorts. Reductions in median serum ferritin levels were initially faster in the production than the haemolytic anaemias, but at 1 yr, similar significant reductions of 940 and 617 ng/mL were attained, respectively (-26.0% overall). Mean LPI decreased significantly in patients with production (P< 0.0001) and haemolytic (P = 0.037) anaemias after the first dose and was maintained at normal mean levels (< 0.4 ?m) subsequently. The most common drug-related, investigator-assessed adverse events were diarrhoea (n = 16) and nausea (n = 12). At matched transfusional iron-loading rates, the responses of rare transfusion-dependent anaemias to deferasirox are similar at 1 yr, irrespective of the underlying pathogenic mechanism.