

**The 49th American Society of Hematology (ASH) Meeting and Exposition
Atlanta, Georgia, USA, 8–11 December 2007**

Summary of key presentations

The 49th Annual American Society of Hematology (ASH) congress was one of the largest ever (~30000 attendees from around the world), reflecting recent developments in basic science and the substantial progress being made in the hematologic diseases. In total, 3700 abstracts were accepted for poster or oral presentation and a further 1400 accepted for publication only. The abstracts can be viewed online at <http://www.abstracts2view.com/hem07> or in *Blood* 2007;10(11). Here we provide an overview of the latest data that were presented at the congress related to the myelodysplastic syndromes (MDS) and stem cell transplantation (SCT).

POSTER PRESENTATION

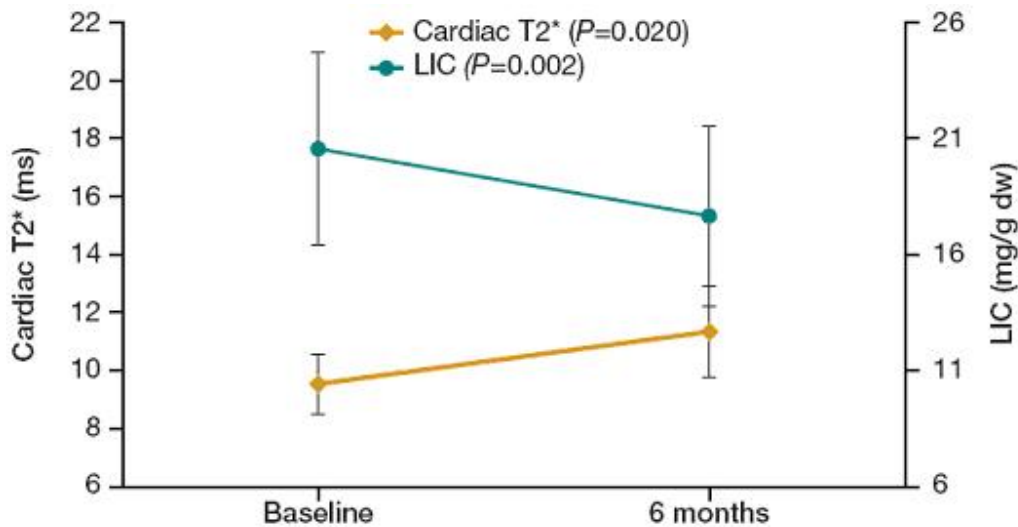
Exjade reduces cardiac iron burden in chronically transfused β -thalassemia patients: An MRI T2* study. *J Wood (abstract 2781)*

Despite the availability of effective iron chelation therapy, cardiac iron overload leading to cardiomyopathy and congestive heart failure remains the principal cause of death in iron-overloaded patients with β -thalassemia major who are transfusion dependent. This ongoing study is a prospective, single-arm multicenter trial evaluating the effect of deferasirox on cardiac iron in patients with thalassemia major. Preliminary results from the first 18 patients (three male, 15 female; aged 10–45 years) who have completed 6 months of treatment on deferasirox 30 mg/kg/day are reported here.

Entry criteria include magnetic resonance imaging (MRI) evidence of cardiac iron overload (T2* <20 ms) and normal left ventricular ejection fraction (LVEF; $\geq 56\%$). Serum ferritin is being assessed monthly and MRI assessments for liver iron concentration (LIC), cardiac T2* and LVEF every 6 months. LPI, serum creatinine, biochemical and hematological status are also being monitored.

At baseline, mean serum ferritin was 4927 ± 987 ng/mL (395–10751; n=12), mean cardiac T2* was 9.8 ± 1.13 ms (5.0–16.1), mean LIC was 20.5 ± 4.2 mg Fe/g dw (3.6–62.3), mean LVEF was $61.7 \pm 1.0\%$ and mean LPI was 0.73 ± 0.28 $\mu\text{mol/L}$ (n=11); five patients had abnormal LPI levels (>0.5 $\mu\text{mol/L}$). After 6 months of deferasirox treatment, the mean decrease in serum ferritin was 516 ng/mL (23.7%; $P=0.403$). Mean reductions in cardiac and hepatic iron were 14.2% ($P=0.020$; Figure 1) and 22.9% ($P=0.002$; Figure 1), respectively. There was no significant change in LVEF. All patients had normal LPI at 6 months, reflecting a decrease of 0.13 ± 0.06 $\mu\text{mol/L}$ ($P=0.027$); mean decrease in patients with abnormal baseline LPI was 0.26 ± 0.11 ($P=0.003$).

Figure 1. Mean change (\pm SEM) in LIC and cardiac T2* from baseline to 6 months (n=18)



No serious drug-related AEs have been observed. No patients have developed serum creatinine levels above normal limits. Four patients (of 15 evaluable) had abnormal liver transaminases on ≥ 2 occasions, but all already had abnormal levels at baseline.

In this study, monotherapy with deferasirox 30 mg/kg/day over an initial 6-month period led to statistically significant improvements in cardiac and liver iron balance and LPI, while LVEF remained stable in this heavily iron overloaded population. These initial data are encouraging and confirm the findings of previous studies regarding positive treatment effects of deferasirox on liver and cardiac iron, as well as normalization of LPI. Further assessments at 12 and 18 months will elucidate whether deferasirox continues to improve cardiac iron burden and maintain/improve cardiac function in these severely iron-overloaded patients.