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Original Research Article

Study on the role of iron chelators in the management of iron overload among transfusion-dependent thalassemia (TDT) pediatric patients

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ABSTRACT

Aims and Objectives: This study aims to investigate the efficacy and safety of Deferasirox, an oral iron chelator, in reducing iron burden in pediatric patients with transfusion-dependent beta-thalassemia. Thalassemia syndromes, particularly beta-thalassemia, are inherited hemoglobin disorders requiring regular blood transfusions, leading to iron overload and subsequent complications. Effective management of iron overload is crucial to prevent morbidity and mortality.

Materials and Methods: It was a descriptive observational study on Children between the ages of 2 years and 12 years who present with transfusion-dependent thalassemia and are on blood transfusion and develop iron overload, which is evaluated by serum ferritin levels of more than 2000mcg/l are administered iron chelator Deferasirox (14 mg/kg/d) and patients are evaluated for myocardial, hepatic, pancreatic iron burden and conditions of iron toxicity with the help of Cardiac MRI T2, LIC (Liver Iron Concentration), MRI T2 Pancreas, LVEF (Left Ventricular Ejection Fraction).

Results: A total of 22 patients enrolled in the study; significant reductions were observed in mean serum ferritin levels (2,388 mcg/dl to 2,054 mcg/dl, $p=0.0009$), transferrin saturation (70.45% to 64.32%, $p=0.00005$), and serum transaminases (44.55 U/L to 40.27 U/L, $p=0.003$) at 6 months. Cardiac MRI T2* increased from 19.55 ms to 22.95 ms ($p=0.045$) at the end of 6 months and at the end of 12 months from 19.55 to 28.23 ($p=0.0016$), and LIC reduced from 20.73 mg Fe/g dw to 11.59 mg Fe/g dw ($p=0.00005$). Pancreatic T2 improved from 15.96 ms to 20.23 ms at 12 months ($p=0.007$). A transient increase in serum creatinine was observed at 6 months from 0.64 \pm 0.14 mg/dL to 0.7 \pm 0.13mg/dL($p=0.009$), which returned to normal at the end of 12 months to 0.63 mg/dL, no additional therapy-related adverse events were reported.

Conclusion: Deferasirox has demonstrated significant efficacy in reducing iron overload in pediatric patients with transfusion-dependent beta thalassemia over a 12-month period. The substantial improvements in serum ferritin, cardiac MRI T2*, LIC, transferrin saturation, and pancreatic T2, coupled with its excellent safety profile, support the use of DFX as a cornerstone in the management of iron overload in this vulnerable population.

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1. Introduction

Thalassemia syndromes are characterized by the inability to produce normal hemoglobin. It is a group of

inherited hemoglobin disorders, including α -thalassemia, β -thalassemia, and E/ β -thalassemia. β -thalassemia is the most prevalent, with approximately 60,000 symptomatic individuals born annually worldwide.¹ The prevalence of beta thalassemia in India is around 3.74% of total population, according to recent 2023 estimate.² Recent

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classifications have distinguished thalassemia disorders into transfusion-dependent thalassemia (TDT), which requires regular lifelong blood transfusions starting from the age of 2 years, and non-transfusion-dependent thalassemia.^{3,4}

TDT (Transfusion-Dependent Thalassemia) patients require regular blood transfusions, Due to frequent transfusions, iron overload occurs rapidly, leading to clinical complications like pituitary dysfunction which leads to endocrine issues such as hypothyroidism and hypoparathyroidism, cardiac siderosis due to Iron deposition in the heart, causing arrhythmias and heart failure, Liver Iron overload can lead to liver fibrosis, cirrhosis, and hepatocellular carcinoma (HCC), Diabetes can occur from pancreatic iron overload, Hypogonadism due to iron deposition in the gonads leading to Impaired sexual development, Bone Disease Associated with poor bone health and development as seen in Figure 1.⁵

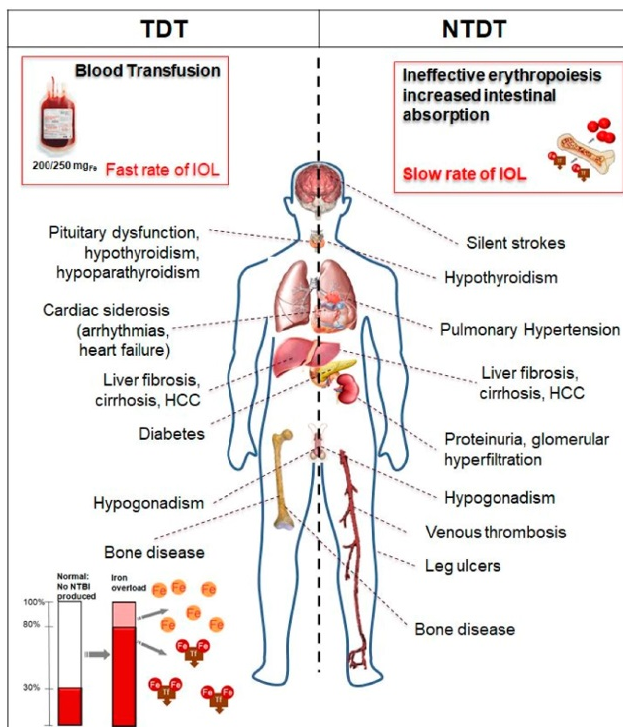


Figure 1: Comparison between transfusion-dependent thalassemia (TDT) and non-transfusion-dependent thalassemia (NTDT)

Transfusion therapy can correct the anemia, which is the cause of death in TDT patients during the first decade of life, and significantly prolong survival.⁶ However, repeated blood transfusions lead to iron overload, as iron that is not saturated by transferrin becomes a toxic agent. The inability to remove excess iron exposes TDT patients to its toxic effects, leading to death from iron-induced cardiomyopathy in the second decade of life.⁷

Deferasirox is an oral iron chelator used primarily to manage chronic iron overload, which is common in patients

with conditions requiring frequent blood transfusions, such as beta-thalassemia major. The drug operates by binding to excess iron in the bloodstream and promoting its excretion, thus mitigating the risk of iron-induced organ damage.⁸ Deferasirox has a high affinity for ferric iron (Fe³⁺) and forms a stable complex that is predominantly excreted through the feces.⁹ This selective binding helps to reduce the iron burden effectively and prevent complications associated with iron overload, such as cardiomyopathy, liver cirrhosis, and endocrine abnormalities.¹⁰

Pharmacokinetically, deferasirox is well absorbed when administered orally, reaching peak plasma concentrations within 1.5 to 4 hours post-dose. The drug exhibits a high degree of protein binding (about 99%), primarily to serum albumin, and is metabolized mainly in the liver via the uridine diphosphate-glucuronosyltransferase (UGT) pathway, with UGT1A1 playing a significant role.¹¹ The majority of the drug-iron complex is excreted via the feces, with a smaller fraction eliminated through urine.¹²

The availability of non-invasive methods to monitor iron loading and unloading in the liver, heart, and pancreas has significantly increased the survival of patients with thalassemia.¹³ Magnetic resonance imaging (MRI), which can accurately monitor multi-organ iron overload (IOL), has enabled a better understanding of the effects of iron chelation therapy. The aim of chelation is to consistently neutralize the toxic effects of iron and prevent or eradicate IOL. This case-based review studies the role of iron chelation therapy (ICT) in the management of iron overload in transfusion-dependent thalassemia patients with currently available iron chelators.¹⁴

2. Aim and Objective

To study the role of iron chelators in management of iron over load in blood transfusion dependent thalassemia pediatric patients

3. Materials and Methods

3.1. Place of study

Study will be conducted in pediatrics department government medical college and government general hospital Ongole for a duration of 12 months.

3.2. Study design

It was a descriptive observational study on children below the age of 12 years who present with hemoglobin levels less than 10 mg/dl are further evaluated using hemoglobin variant analysis by Hemoglobin Electrophoresis High-Performance Liquid Chromatography (HPLC) and patients of transfusion dependent thalassemia are identified. They are given blood transfusion and some develop iron overload, which is evaluated by serum ferritin levels which are high.

Those with elevated serum ferritin levels of more than 2000 mcg/l are administered iron chelators like Deferasirox (14 mg/kg/day). and patients are evaluated for myocardial, hepatic, pancreatic iron burden and conditions of iron toxicity.

3.3. Ethical clearance

Study got clearance from institutional ethics committee, government medical college Ongole. Informed consent from the parents who are willing to participate in the study was obtained.

3.4. Pre-treatment assessment

Detailed history and examination of all the systems was done and patients were evaluated for Hemoglobin, Serum ferritin, Transferrin saturation, Transaminases, Serum total bilirubin, Direct bilirubin, Creatinine, Blood Urea, Cardiac MRI T2, LIC (Liver Iron Concentration), MRI T2 Pancreas, LVEF (Left Ventricular Ejection Fraction)

3.5. Inclusion criteria

1. Children below the age of 12 years.
2. Children whose hemoglobin levels are less than 10 g/dl.
3. Transfusion dependent thalassemia patients on iron chelators for management of iron overload.
4. Those with elevated serum ferritin levels of more than 2000 mcg/l.

3.6. Exclusion criteria

1. Children below age of 2 years.
2. Children with causes of anemia other than thalassemia.
3. Children who do not require blood transfusion (non-transfusion dependent thalassemia patients).

3.7. Follow-up

Patients were evaluated with following investigations at baseline and on each visit at the end of 6th month and 12th month: Hemoglobin, Serum ferritin, Transferrin saturation, Transaminases, Serum total bilirubin, Direct bilirubin, Creatinine, Blood Urea, Cardiac MRI T2, LIC (Liver Iron Concentration), MRI T2 Pancreas, LVEF (Left Ventricular Ejection Fraction).

4. Statistical Analysis

1. Collected data will be expressed as mean +/- standard deviation.
2. Data so collected will be entered into a case record form for study, statistical analysis will be done using Student's t- test.

5. Results

22 children satisfying inclusion criteria were enrolled in study baseline Characteristics of patient with transfusion-dependent β -thalassemia major with severe multiorgan iron overload is given in Table 1.

Table 1: Shows baseline characters of transfusion dependent beta thalassemia patients on Deferasirox (iron chelation therapy)

Baseline characteristics	
Parameters	Mean \pm Standard deviation
Mean age	6.9 \pm 4
Gender	
Male	15
Female	7
Hemoglobin	8.36 \pm 0.85
Hemoglobin-A (%)	2.86 \pm 0.28
Hemoglobin-A2 (%)	4.64 \pm 0.11
Hemoglobin-F (%)	92.49 \pm 0.29
HbA1c (%)	5.25 \pm 0.11
Serum ferritin	2388 \pm 447.9
Transferrin saturation	70.45% \pm 4.95%
Transaminases	44.55 \pm 7.84
Serum total bilirubin	1.05 \pm 0.33
Direct bilirubin	0.46 \pm 0.16
Creatinine	0.64 \pm 0.14
Blood urea	22.14 \pm 3.33
Cardiac MRI T2	19.55 22.95 \pm 5.42
LIC (Liver Iron Concentration)	20.73 \pm 5.77
MRI T2 Pancreas	15.96 \pm 4.4
LVEF (Left Ventricular Ejection Fraction)	54.36 \pm 4.11

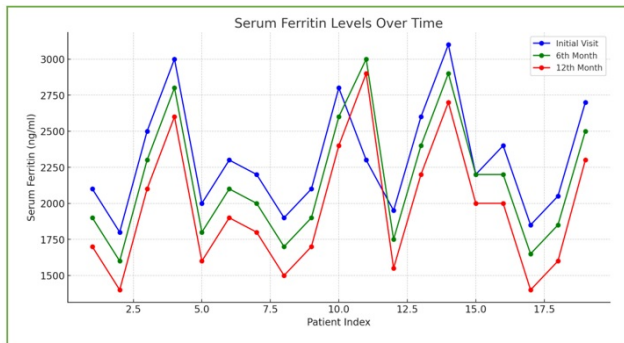
Mean serum ferritin in 0 visit was 2388 mcg/dl. Mean Cardiac MRI T2* was 19.55 ms, indicating severe IOL with normal left ventricular ejection fraction. Mean liver iron concentration (LIC) was 20.73 mgFe/g dry weight (dw) tissue, and mean pancreatic T2 54.32 ms, Iron chelation was prescribed with oral Deferasirox (DFX) at 14 mg/kg During the 12 months of chelation therapy.

5.1. Serum ferritin levels

It was observed there is a significant reduction in mean serum ferritin levels from 2388 mcg/dl to 2054 mcg/dl at the end of 6 months ($p=0.0009$) as shown in Figures 1 and 2. The mean serum ferritin levels, a primary marker of iron overload, significantly decreased from 2388 mcg/dl at the initial visit to 2054 mcg/dl at the end of 6 months ($p=0.0009$). This reduction highlights the effectiveness of DFX in mobilizing and reducing iron stores. Lower ferritin levels are associated with reduced risk of complications such as hepatic and cardiac dysfunction, and improved overall prognosis.

Table 2: Shows mean values of each parameter at each visit

Mean values of parameters in transfusion dependent beta thalassemia patients on Deferasirox (iron chelation therapy)	T0 (Mean±SD)	6 months (Mean±SD)	12 months (Mean±SD)	p-value
Pre-transfusion haemoglobin (g/dL)	8.36±0.85	9.8±0.75	9.95±0.82	0.196
serum ferritin (µg/L)	2388±447.9	2054±371.9	2026±428.3	0.0009
Transferrin saturation	70.45±4.95%	64.32±4.36%	60.23±5.11	0.00005
Transaminases (U/L)	44.55±7.84	40.27±5.99	37.08±6.33	0.003
Total bilirubin (mg/dl)	1.05±0.33	1.16±0.25	0.54±0.21	0.000019
Direct bilirubin (mg/dl)	0.46±0.16	0.49±0.10	0.45±0.19	0.029
Creatinine (mg/dL)	0.64±0.14	0.7±0.13	0.63±0.17	0.009 (initial vs 6 months)
Blood urea (mg/dl)	22.14±3.33	21.23±2.78	21.81±1.74	0.27
cardiac MRI T2* (ms)	19.55±5.42	22.95±5.39	28.23±12.38	0.045 (6 months) 0.0016 (12 months)
LIC (mg/g dw)	20.73±5.77	11.59±4.08	10.23±3.04	<0.00005
LVEF (%)	54.36±4.11	54.77±1.85	56.19±13.14	0.005
MRI-T2* Pancreas (ms)	15.96±4.4	17.54±4.7	20.23±4.7	0.000748
ICT (Iron Chelation Therapy)	DFX film-coated tablet (14 mg/kg)	DFX film-coated tablet (14 mg/kg)	DFX film-coated tablet (14 mg/kg)	

**Figure 2:** Serum Ferritin Levels in transfusion dependent beta thalassemia patients on Deferasirox (iron chelation therapy) at different time interval (n=22)

5.2. Transferrin saturation and serum transaminases

5.2.1. Transferrin saturation

A significant reduction in transferrin saturation at the end of 6 months from 70.45% saturation to 64.32% saturation ($p=0.00005$).

5.2.2. Serum transaminases

A significant reduction in serum transaminases from 44.55 (mg/dl) to 40.27 (mg/dl) at the end of 6 months ($p=0.003$).

The significant reduction in transferrin saturation from 70.45% to 64.32% ($p=0.00005$) and serum transaminases

from 44.55 U/L to 40.27 U/L ($p=0.003$) at 6 months indicates improved iron metabolism and reduced hepatic inflammation, respectively. Transferrin saturation reflects the extent of iron available for tissue uptake, and its reduction suggests decreased iron toxicity. Similarly, lower transaminase levels indicate improved liver function and reduced hepatic stress.

5.2.3. Total bilirubin

Although the mean serum total bilirubin showed a slight increase from 1.05 mg/dl to 1.16 mg/dl, this change was not clinically significant and did not suggest worsening liver function. It is crucial to monitor bilirubin levels to ensure no adverse impact on hepatic function, particularly when using chelation therapy. A significant reduction in serum total bilirubin from 1.05 mg/dl to 0.54 at the end of 12 months ($p=0.000019$). A significant reduction in mean direct bilirubin was seen from 0.46 to 0.45 ($p=0.029$) indicate improvement in hepatic function.

5.2.4. Cardiac T2 MRI

A significant increase in mean cardiac T2* from 19.55 ms to 22.95 ms was seen at the end of 6 months ($p=0.0016$) and at the end of 12 months from 19.55 to 28.23 ($p=0.045$). The mean cardiac MRI T2* increased from 19.55 ms to 22.95 ms ($p=0.045$) over the first 6 months as seen in Figures 3 and 4, indicating an improvement in myocardial iron content. Since a low T2* value is indicative of significant cardiac

iron deposition, the observed increase suggests a reversal of iron-related cardiac risk, which is particularly vital given the high incidence of cardiac complications in this patient group. The maintenance of normal left ventricular ejection fraction throughout the study further supports the cardioprotective effect of DFX.

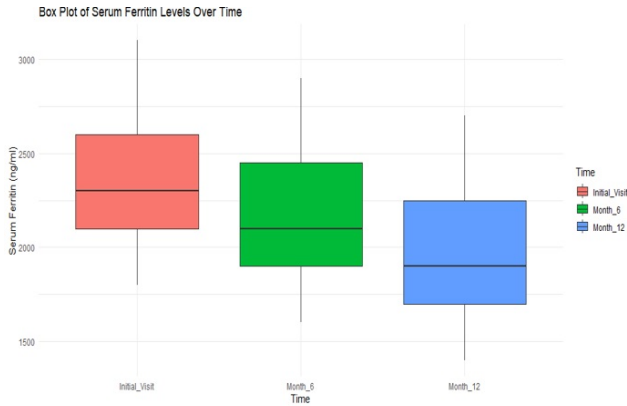


Figure 3: Serum Ferritin Levels in transfusion dependent beta thalassemia patients on Deferasirox (iron chelation therapy) at different time interval (n=22). p< 00009 as compared to baseline, (Paired Student’s t-test)

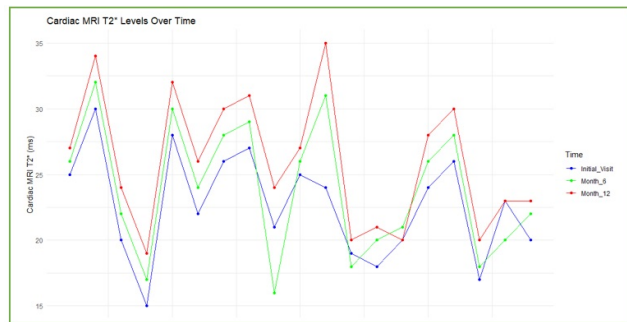


Figure 4: Cardiac MRI T2 in transfusion dependent beta thalassemia patients on Deferasirox (iron chelation therapy) at different time interval (n=22)

5.2.5. LIC (Liver Iron Concentration)

A significant reduction in LIC (Liver Iron Concentration) from 20.73 mg Fe/g dw to 11.59 mg Fe/g dw was seen at the end of 6 months (p=0.00005) as seen in Figures 5 and 6. High LIC is a major concern as it can lead to liver fibrosis and cirrhosis. The marked decrease in LIC demonstrates the potency of DFX in reducing hepatic iron burden, thereby potentially preventing liver-related morbidity.

5.2.6. Pancreatic T2

The significant increase in pancreatic T2 from 15.96 ms to 20.23 ms (p=0.007) at the end of 12 months as seen

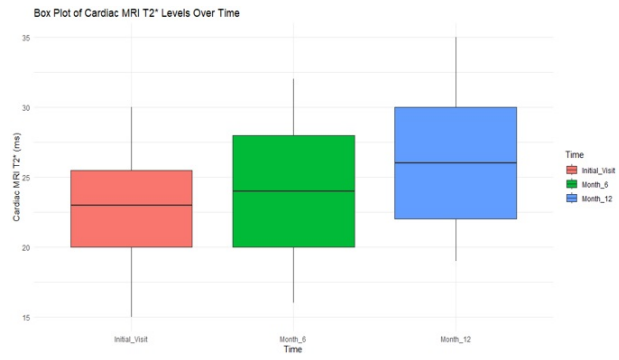


Figure 5: Cardiac MRI T2 in transfusion dependent beta thalassemia patients on Deferasirox (iron chelation therapy) at different time interval (n=22). p < 0016 as compared to baseline, (Paired Student’s t-test)

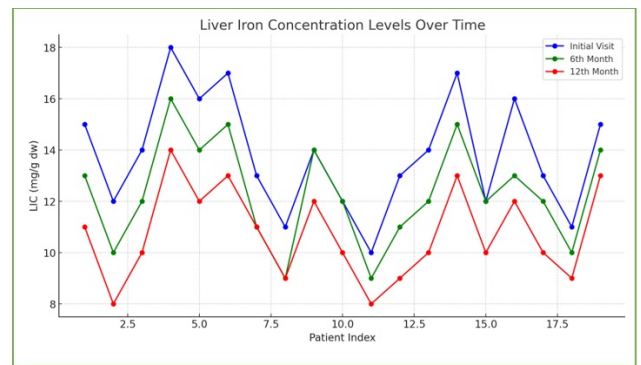


Figure 6: Liver Iron Concentration (LIC) in transfusion dependent beta thalassemia patients on Deferasirox (iron chelation therapy) at different time interval (n=22)

in Figures 7 and 8 is noteworthy. Pancreatic iron loading can lead to diabetes mellitus, a common complication in thalassemia patients. The improvement in pancreatic T2 suggests a beneficial effect of DFX in reducing pancreatic iron deposition, thereby potentially mitigating the risk of diabetes.

5.2.7. Safety and tolerability

There was an increase in mean serum creatinine from 0.64+/-0.14 mg/dL to 0.7+/-0.13 mg/dL was seen at the end of 6 months of treatment with Deferasirox (14 mg/kg) which was statistically significant (p=0.009), but the values of serum creatinine returned to normal at the end of 12 months to 0.63 mg/dL. The patients had not experienced any additional therapy-related adverse events throughout the study period, indicating that DFX was well-tolerated by the paediatric patients. This safety profile is crucial for long-term adherence and effectiveness of the therapy.

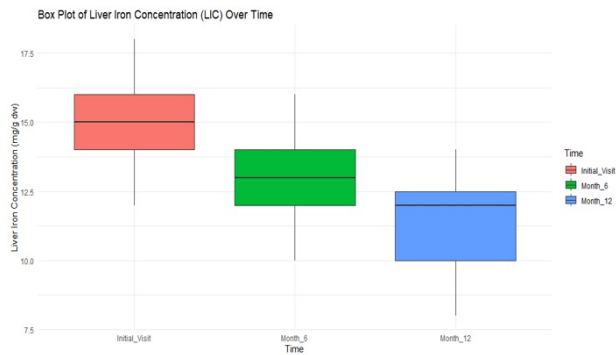


Figure 7: Liver Iron Concentration (LIC) in transfusion dependent beta thalassemia patients on Deferasirox (iron chelation therapy) at different time interval (n=22). $p < 0.00005$ as compared to baseline, (Paired Student's t-test)

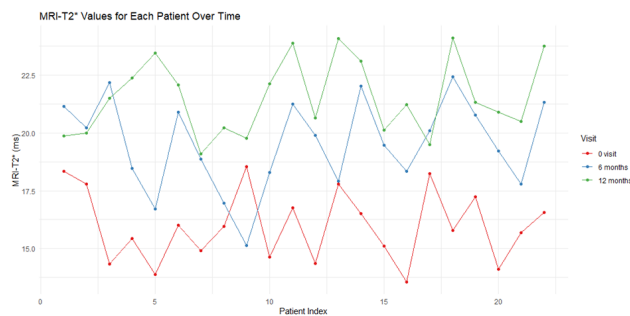


Figure 8: PancreaticT2: in transfusion dependent beta thalassemia patients on Deferasirox (iron-chelation therapy) at different time interval (n=22)

6. Discussion

The results of this study underscore the efficacy of Deferasirox (DFX) in managing iron overload in pediatric patients with transfusion-dependent beta thalassemia. Over a 12-month period, significant improvements were observed in several critical parameters indicative of iron burden and organ function, suggesting that DFX is a potent therapeutic agent in this context. Our findings are consistent with and extend the body of literature on the management of iron overload in beta thalassemia.

6.1. Serum ferritin levels

The mean serum ferritin levels in our study significantly decreased from 2388 mcg/dl at the initial visit to 2054 mcg/dl at the end of 6 months ($p=0.0009$). This reduction aligns with the findings of Taher et al where serum ferritin reduced significantly from 4,139 ng/mL at baseline to 3,176 ng/mL at 12 months.⁽¹⁶⁾, who reported similar decreases in serum ferritin with Deferasirox therapy in a multicenter study.¹⁵ Another study by Cappellini et al demonstrated that DFX effectively lowers serum ferritin levels in both

pediatric and adult populations where mean serum ferritin levels reduced from 3148 mcg/dl to 1626 mcg/dl Over the course of the 5-year.¹⁶ This reduction is crucial as elevated ferritin levels are associated with increased morbidity and mortality due to iron overload-related complications.

6.2. Transferrin saturation and serum transaminases

The significant reduction in transferrin saturation from 70.45% to 64.32% ($p=0.00005$) and serum transaminases from 44.55 U/L to 40.27 U/L ($p=0.003$) at 6 months indicates improved iron metabolism and reduced hepatic inflammation, respectively. This aligns with studies by Porter et al. and Galanello et al., which reported that DFX therapy effectively lowers transferrin saturation and improves liver function, reducing hepatic stress and potential damage.^{17,18}

6.3. Cardiac MRI T2*

The mean cardiac MRI T2* increased from 19.55 ms to 22.95 ms ($p=0.045$) over the first 6 months, indicating a reduction in myocardial iron content. This improvement is particularly significant as cardiac complications are a leading cause of mortality in thalassemia patients. Our findings are similar to the study conducted by Pennell et al., where after 1 year of treatment with Deferasirox, 17.6% of patients normalized their myocardial T2*, and 35.5% of patients improved myocardial T2* from a baseline of 6 to <10 milliseconds to 10 to ≤ 20 milliseconds.¹⁹ The maintenance of normal left ventricular ejection fraction throughout our study further supports the cardioprotective effect of DFX, as seen in other studies.

6.4. Liver iron concentration (LIC)

Our study showed a significant reduction in LIC from 20.73 mg Fe/g dw to 11.59 mg Fe/g dw at the end of 6 months ($p=0.00005$). Comparable reductions in LIC have been reported by Voskaridou et al., where the mean LIC reduced significantly from 31.2 mg Fe/g dw to 24.2 mg Fe/g dw after 12 months of deferasirox therapy and El-Beshlawy et al., where significant reduction in LIC from 15.4 mg Fe/g dw to 11.5 mg Fe/g dw over 12 months with deferasirox treatment was observed.²⁰ These reductions in LIC are essential for preventing long-term hepatic complications in beta thalassemia patients.

6.5. Total bilirubin

Although the mean serum total bilirubin showed a slight increase from 1.05 mg/dl to 1.16 mg/dl, this change was not clinically significant and did not suggest worsening liver function. Monitoring bilirubin levels is essential to ensure no adverse impact on hepatic function, particularly when using chelation therapy. Studies have shown that

fluctuations in bilirubin levels can occur but do not necessarily indicate hepatic dysfunction¹⁸

6.6. Pancreatic T2

The significant increase in pancreatic T2 from 15.96 ms to 20.23 ms ($p=0.007$) at the end of 12 months is significant. Pancreatic iron loading can lead to diabetes mellitus, a common complication in thalassemia patients. The improvement in pancreatic T2 in our study suggests a beneficial effect of DFX in reducing pancreatic iron deposition, thereby potentially mitigating the risk of diabetes. This finding is supported by research conducted by Noetzli et al., where a significantly higher frequency of glucose dysregulation was seen among patients having pancreatic iron overload compared to patients without pancreatic iron overload (71.8% vs. 15.0%; $p < 0.0001$).²¹ In a multicentric observational study done by Alessia Pepe et al. on the influence of pancreatic iron with glucose metabolism and with cardiac complications in thalassemia major, patients with normal glucose metabolism had significantly higher global pancreas T2* values (14.31 ± 11.31 ms) compared to those with impaired fasting glucose (IFG) (8.82 ± 6.23 ms), impaired glucose tolerance (IGT) (8.25 ± 5.03 ms), and diabetes (7.87 ± 4.45 ms).²² This indicates a correlation between lower pancreatic T2* values and higher risk of glucose metabolism disorders and highlights the importance of reducing pancreatic iron to prevent endocrine complications.

6.7. Safety and tolerability

The safety and tolerability of Deferasirox (DFX) in managing iron overload in transfusion-dependent beta-thalassemia pediatric patients are crucial for long-term therapy adherence and overall effectiveness. Our study observed a statistically significant increase in mean serum creatinine levels from 0.64 ± 0.14 mg/dL to 0.7 ± 0.13 mg/dL at the end of 6 months of treatment with Deferasirox (14 mg/kg), which returned to normal levels (0.63 mg/dL) at the end of 12 months ($p=0.009$). Despite this temporary increase in serum creatinine, patients did not experience any additional therapy-related adverse events throughout the study period, indicating that DFX was generally well-tolerated by the pediatric population.

The transient rise in serum creatinine observed in our study aligns with findings from several other studies, which have reported similar trends. For instance, a study by Cappellini et al. noted that while there was an initial increase in serum creatinine levels in patients treated with DFX, these levels normalized over time, suggesting that the renal effects might be reversible and manageable with proper monitoring.¹⁶ Similarly, Taher et al. reported a comparable safety profile, where temporary increases in serum creatinine were observed without long-term renal

impairment.¹⁵

The lack of additional therapy-related adverse events in our study is consistent with the findings of Porter et al., who also highlighted the general tolerability of DFX in pediatric patients. Their study emphasized the importance of regular monitoring to manage any potential side effects effectively, ensuring the continuation of therapy without significant interruptions. Additionally, Galanello et al. noted that DFX therapy was associated with fewer adverse events compared to other iron chelators, making it a preferable option for long-term management of iron overload in thalassemia patients.¹⁰

The favorable safety profile of DFX is crucial for ensuring patient adherence, especially in a pediatric population where long-term treatment is necessary. Adherence to iron chelation therapy is a significant challenge in managing thalassemia, and the well-tolerated nature of DFX facilitates better compliance. As reported by Pennell et al., effective iron chelation with DFX, combined with its tolerability, contributes to improved patient outcomes by reducing myocardial iron content and maintaining normal cardiac function.²³

Overall, our study supports the use of Deferasirox as a safe and effective iron chelator in pediatric patients with transfusion-dependent beta-thalassemia. The temporary increase in serum creatinine should be closely monitored, but it does not pose a significant risk for long-term renal impairment. The absence of additional adverse events further highlights the tolerability of DFX, making it a viable option for the ongoing management of iron overload in this vulnerable population.

Comparison with Other Chelation Therapies: Our findings on the efficacy of DFX are consistent with studies comparing DFX to other chelation therapies such as Deferoxamine (DFO) and Deferiprone (DFP). While DFO remains a standard therapy, its requirement for parenteral administration limits its use, particularly in paediatric populations. DFP, although effective, has been associated with agranulocytosis and other adverse effects.²⁴ DFX, administered orally, offers a more convenient and well-tolerated option with comparable efficacy, as demonstrated in our study and corroborated by comparative studies.

7. Conclusion

Deferasirox has demonstrated significant efficacy in reducing iron overload in paediatric patients with transfusion-dependent beta thalassemia over a 12-month period. The notable improvements in key indicators such as serum ferritin levels, cardiac MRI T2*, liver iron concentration (LIC), transferrin saturation, and pancreatic T2 underscore the drug's effectiveness. These improvements, along with Deferasirox's favorable safety profile, support its role as a cornerstone in managing iron overload in this vulnerable population. Our findings align

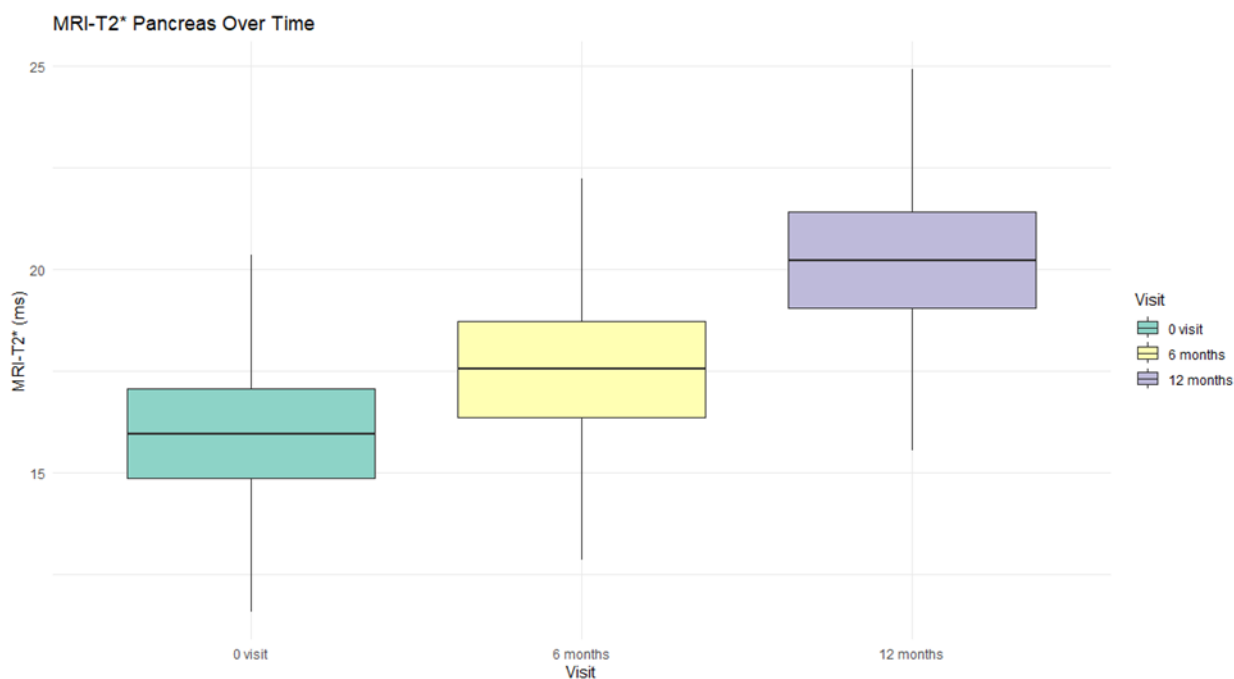


Figure 9: Pancreatic T2: in transfusion dependent betathalassemia patients on Deferasirox (iron chelation therapy) at different time interval (n=22). $p < 0.0007$ as compared to baseline, (Paired student's 't'test)

with and extend existing literature, reinforcing the utility of Deferasirox in enhancing clinical outcomes and quality of life for thalassemia patients. Continued monitoring is essential to ensure the long-term safety and efficacy of Deferasirox therapy.

8. Limitations of Study

1. A large sample size is required to allow a more accurate assessment of this study.
2. Main limitation of this study is the absence of a control arm.
3. Longer-term studies are recommended to further establish the safety of deferasirox and optimize the dosing strategies.

9. Source of Funding

None.

10. Conflict of Interest

None.

11. Acknowledgment


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